Filed Pursuant to Rule 424(b)(4)
Registration No. 333-253954

12,000,000 Shares

Common Stock

This is the initial public offering of shares of common stock of Design Therapeutics, Inc. We are offering 12,000,000 shares of our common stock.

Prior to this offering, there has been no public market for our common stock. The initial public offering price is $20.00 per share of our common stock.

Our common stock has been approved for listing on the Nasdaq Global Select Market under the symbol “DSGN.”

We are an “emerging growth company” as defined under the federal securities laws and, as such, have elected to comply with certain reduced reporting requirements in this prospectus and may elect to do so in future filings.

See the section titled “Risk Factors” beginning on page 14 to read about factors you should consider before deciding to invest in shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$ 20.00	$240,000,000
Underwriting discounts and commissions(1)	$ 1.40	$ 16,800,000
Proceeds, before expenses, to Design Therapeutics, Inc.	$ 18.60	$223,200,000

(1) See the section titled “Underwriting” for a description of the compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to an additional 1,800,000 shares of our common stock at the initial public offering price, less the underwriting discounts and commissions.

The underwriters expect to deliver the shares against payment in New York, New York on March 30, 2021.

Goldman Sachs & Co. LLC **SVB Leerink** **Piper Sandler**

Prospectus dated March 25, 2021

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Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus is an offer to sell only the shares offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus or in any applicable free writing prospectus is current only as of its date, regardless of its time of delivery or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: Neither we nor the underwriters have done anything that would permit this offering or possession or distribution of this prospectus or any free writing prospectus we may provide to you in connection with this offering in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus and any such free writing prospectus outside the United States.

PROSPECTUS SUMMARY

This summary highlights selected information contained in greater detail elsewhere in this prospectus. This summary is not complete and does not contain all of the information you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus. You should carefully consider, among other things, the sections of this prospectus titled "Risk Factors," "Special Note Regarding Forward-Looking Statements" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and the related notes included elsewhere in this prospectus. Unless the context otherwise requires, the terms "Design Therapeutics," "Design," "we," "us," "our" and similar references in this prospectus refer to Design Therapeutics, Inc.

Overview

We are a preclinical-stage biopharmaceutical company pioneering novel small-molecule therapeutic candidates, called gene targeted chimeras (GeneTACs), that are designed to be disease-modifying and target the underlying cause of inherited nucleotide repeat expansion diseases. Certain nucleotide repeat expansion diseases, such as Friedreich ataxia (FA), can result in reduced expression of specific mRNAs; in other diseases, such as myotonic dystrophy type-1 (DM1), Fuchs endothelial corneal dystrophy (FECD), and Huntington disease, the nucleotide repeat expansions result in the generation of toxic gene products, often associated with pathological nuclear foci. Our GeneTACs are designed to selectively bind to genetic repeat sequences, modulate gene expression either by restoring or blocking transcription, and restore cellular health. As a platform, we believe that GeneTACs have broad potential applicability across monogenic nucleotide repeat expansion diseases.

In preclinical studies for our lead program, we have observed restoration of frataxin (FXN) levels in cells from FA patients using our FA GeneTACs. FA GeneTACs administered to various species, at doses that were observed to be well tolerated, achieved biodistribution to brain and heart, key organs affected by FA, at concentrations that were consistent with those observed to restore FXN levels in FA patient cells. Further, and consistent with this good biodistribution, we observed increased FXN expression in the brain and heart in an animal model of FA after treatment with our FA GeneTACs. We plan to initiate clinical trials with our lead product candidate in FA patients to evaluate its safety, pharmacokinetics (PK) and effect on FXN levels by the first half of 2022, subject to receiving regulatory clearance to proceed into clinical trials.

In our second GeneTAC program in DM1, we observed reduced nuclear foci in DM1 patient muscle cells after administration of our DM1 GeneTACs. We expect to seek regulatory clearance for clinical trials in 2023. We are also advancing our GeneTAC portfolio in preclinical studies to address other serious nucleotide repeat expansion-driven monogenic diseases, and intend to declare an additional product candidate in 2023.

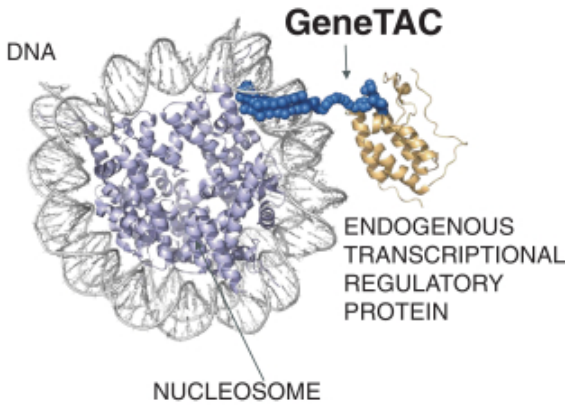
Other genomic therapeutics, including oligonucleotides, mRNA, gene therapy and gene editing, have disease-modifying potential but may have modality-associated limitations related to administration, biodistribution and potential safety concerns. In contrast, we believe the structure and mechanism of action of our GeneTACs may offer the disease-modifying potential of genomic therapeutics, while also offering broad tissue biodistribution, resolution of aberrant gene expression preserving endogenous regulatory control elements, and leveraging established manufacturing, regulatory, and distribution frameworks for small molecules.

Our GeneTAC Platform

We utilize our proprietary GeneTAC platform to design and develop therapeutic candidates for inherited diseases driven by nucleotide repeat expansion. Individuals with nucleotide repeat expansion diseases are born with abnormally expanded stretches of specific nucleotide sequences, often with hundreds to thousands of excess repeats present in the mutant gene. Higher number of excess repeats can lead to more severe, and sometimes a more rapidly progressive form of disease. Nucleotide repeat expansion has been identified as the underlying cause of more than 40 debilitating degenerative diseases impacting millions of people. Currently, there are no approved therapeutic options that address the cause of any nucleotide repeat expansion diseases.

GeneTACs represent a novel class of small molecules designed to act on a diverse array of diseases. We have developed a proprietary framework that combines our understanding of medicinal chemistry and structure-activity relationships that allow us to design targeted DNA-binding moieties that are connected via a linker to ligand moieties that engage and modulate the transcriptional machinery. GeneTAC molecules are heterobifunctional, meaning that they are comprised of two principal moieties that are each designed to have a unique function:

- ***DNA-Binding Moiety:*** One end of the GeneTAC molecule is a DNA minor groove binder that has been designed to recognize and bind to the specific nucleotide repeat sequence of interest (e.g. the repeated GAA sequence seen in the first intron of the FXN gene seen in FA or the CTG repeat in the 3' non-coding region of the dystrophy myotonic protein kinase (DMPK) gene seen in DM1).
- ***Ligand Moiety:*** The other end of the molecule is designed to interact with the endogenous proteins that can regulate transcription.



The structures of the GeneTAC molecules are designed to enable them to act specifically at the site of the disease-causing nucleotide repeat expansion by targeting the mutant allele and modulating the transcriptional machinery in a cell. Consequently, the cell can resume gene expression and production of normal protein isoforms that remain under normal physiological control. The versatility of the GeneTAC platform allows us to design GeneTAC molecules toward a specific nucleotide repeat expansion target, regardless of repeat number, and tailor it to address the underlying disease-specific dysfunction in gene regulation in one of the following ways:

- ***Restoration of Transcription:*** In diseases where the expanded nucleotide repeat structure can cause endogenous transcription machinery to stall, which leads to an insufficient amount of

protein production, GeneTAC molecules can be designed to bind at the desired loci in the genome and engage the endogenous transcriptional machinery with the goal of restoring normal levels of full-length pre-mRNAs. In FA, for example, where the expanded triplet repeat occurs in an intron, a non-coding region of the gene, the abnormally long nucleotide sequence is spliced out of the pre-mRNA thus enabling normal production of natural protein isoforms according to existing physiologic regulatory control.

- ***Reduction of Toxic Gene Product Levels:*** Another type of nucleotide repeat expansion disease occurs when the transcription process results in the accumulation of toxic gene products (e.g. DM1, Huntington disease, FECD), and in some cases the formation of nuclear foci, leading to multiple downstream cellular dysfunctions. In these cases, a single copy of expanded repeat containing allele is sufficient to cause the disease. Our GeneTAC molecules are designed to selectively target the abnormally expanded nucleotide repeat to block the formation of the downstream toxic gene product and restore cellular function without interfering with the gene expression of the normal allele.

Our understanding of the properties of the GeneTAC molecules is based on data-driven assessments of compounds we have designed and synthesized, as well as experience with our most advanced compounds for FA tested *in vitro* and *in vivo*. We continue to develop know-how of permutations of binder, linker, and ligand moieties that drive the drug properties of molecules which are best suited to be developed for treating the underlying cause of each specific disease. This understanding of GeneTAC chemistry has enabled us to generate multiple candidates designed to have optimal potential therapeutic and drug characteristics.

We are using our GeneTAC platform to develop small molecule genomic medicine candidates that are designed to offer precise modulation of gene transcription. We believe that the GeneTAC platform may offer several potential mechanistic and development advantages over other genomic medicine modalities, including:

- GeneTAC small molecules may be more tolerable over complex biologics because GeneTACs are less likely to cause adverse immune reactions;
- GeneTACs may be less likely to be immunogenic and therefore have no limitations with re-dosing;
- GeneTAC treatment is designed to be reversible;
- GeneTACs are designed to act on the transcription machinery of the cell and do not alter the genome;
- GeneTACs modulation of transcription is designed to preserve normal physiological post-transcriptional regulation and protein translation controls;
- GeneTAC structure is designed to enable therapeutically active molecules to be deployed directly at the site of disease-causing mutations, which could enhance specificity and potency, and minimize off-target effects;
- GeneTACs are designed to enable ongoing dose optimization;
- GeneTACs can achieve biodistribution across target organs and into the cell without specialized engineering or delivery technologies;
- GeneTACs are synthetically tractable, offering a potentially readily scalable, cost-effective development path that does not require complex customized manufacturing equipment and processes; and

- GeneTACs have a modular heterobifunctional structure that is intended to allow us to rationally design novel targeting components for specific DNA sequences, creating a potentially highly efficient discovery engine that could enable us to rapidly expand our portfolio into new disease areas.

Our Programs

We are developing a portfolio of GeneTAC product candidates designed to address genetic diseases driven by inherited nucleotide repeat expansions that have urgent medical need and where no approved disease-modifying treatments are currently available.

Our lead candidates and early development programs are summarized in the table below:

PROGRAMS (Targeted nucleotide expansion)	NEXT ANTICIPATED MILESTONE	ANTICIPATED MILESTONE DATE
Friedreich ataxia (GAA)	Obtain regulatory clearance and initiate first-in-human clinical trials	1H 2022
Myotonic dystrophy (CTG)	Obtain regulatory clearance and initiate first-in-human clinical trials	2023

* *To date, we have not submitted an investigational new drug application (IND) with the U.S. Food and Drug Administration (FDA) or any similar submission in any foreign jurisdiction with respect to either of our lead candidate programs.*

FA Program Overview

Our FA program is focused on the development of a potentially disease-modifying treatment. FA is a devastating monogenic, autosomal recessive progressive disease where over 95% of cases are caused by homozygous guanine-adenine-adenine (GAA) triplet repeat expansions in the first intron of the FXN gene, which encodes the mitochondrial protein FXN. The disease is characterized by spinocerebellar ataxia, dysarthria, pyramidal weakness, deep sensory loss, hypertrophic cardiomyopathy, skeletal abnormalities, and diabetes mellitus. Clinical onset occurs most often around puberty, leads to severe disability by early adulthood, with substantial functional loss, wheelchair dependence, and loss of quality of life. Affected individuals have reduced life expectancy, with many premature deaths caused by complications of the cardiomyopathy at about the end of the fourth decade of life.

The estimated prevalence of FA is 1 in 40,000-50,000, affecting more than 5,000 individuals living in the United States and more than 20,000 in Europe. Our FA GeneTAC candidate is designed to address the genetic basis of the disease by restoring functional FXN protein levels and, subject to receiving regulatory clearance to proceed into clinical trials, we anticipate a first-in-human dosing for our first product candidate in the first half of 2022. The primary cause of mortality (approximately 60% of FA patients) is cardiac arrhythmias or heart failure with the mean life expectancy reduced to approximately 35-40 years.

DM1 Program Overview

Our DM1 program is focused on the development of a potentially disease-modifying treatment for DM1. DM1 is a monogenic, autosomal dominant, progressive neuromuscular disease that affects skeletal muscle, heart, brain, and other organs. The cardinal features include muscle weakness, myotonia (slow muscle relaxation), and early cataracts. In addition, affected individuals often experience cardiac arrhythmias and changes in neuropsychological function. DM1 is caused by a mutation in the DMPK gene and is estimated to have a genetic prevalence of 1 in 2,300-8,000 people, affecting more than 70,000 people in the United States and more than 90,000 people in Europe. Our DM1 GeneTAC molecules are designed to address the genetic basis of the disease by preventing the expression of toxic gene product and consequently of nuclear foci. We expect to complete investigational new drug (IND)-enabling studies and seek regulatory clearance for a first-in-human clinical trial in 2023.

Research Program Overview

We are also advancing our GeneTAC product candidate portfolio into development in other serious nucleotide repeat expansion-driven monogenic diseases, such as FECD, Fragile X syndrome, spinocerebellar ataxias, amyotrophic lateral sclerosis, frontotemporal dementia, Huntington disease and spinobulbar muscular atrophy. Many of these monogenic diseases have overlapping triplet repeat expansions, including CTG repeats that cause DM1, allowing for the potential for a single GeneTAC to be used across multiple diseases. Additionally, our experiences with GeneTACs allow us to more rapidly design GeneTACs for additional proposed indications.

Our Strategy

We aim to leverage our GeneTAC platform to design, develop and commercialize a pipeline of disease-modifying therapeutic candidates designed to treat a wide range of inherited nucleotide repeat expansion diseases for which there is urgent unmet medical need. In order to achieve our goal, we intend to:

- Advance our lead program in FA through clinical development to offer meaningful patient benefit;
- Advance our DM1 program through clinical development to offer meaningful patient benefit;
- Leverage our GeneTAC platform to expand our pipeline and address additional nucleotide repeat expansion diseases with significant unmet medical need;
- Selectively enter into strategic collaborations to realize the full potential of our platform;
- Independently commercialize any approved products in indications and geographies where we believe we can maximize value; and
- Establish a leadership position in genetic disease therapeutics by continuing to build and leverage our relationships with the key opinion leaders, clinicians, and patients.

Our History, Team and Investors

Our company was created to design, develop and commercialize a novel class of small molecule therapeutic candidates (GeneTACs) designed to directly address the underlying basis of genetic disease. To achieve this goal, we have assembled a management team with extensive experience in

the design, development and commercialization of drugs for serious diseases, including a seasoned research and development team, comprised of 22 individuals (20 of which are full time employees), 11 of whom have Ph.D.s or M.D.s., as of March 22, 2021.

Our company was started by Pratik Shah, Ph.D. and Aseem Z. Ansari, Ph.D. Dr. Shah, our Co-Founder and Executive Chairperson, has more than 30 years of experience founding and leading biopharmaceutical companies and healthcare investment decisions. Dr. Ansari, our Co-Founder, is an internationally recognized pioneer in transcriptional regulation and DNA minor groove binders and the chair of the Department of Chemical Biology and Therapeutics at St. Jude Children's Research Hospital. João Siffert, M.D., our President and Chief Executive Officer, has more than 20 years of leadership experience in biopharmaceutical companies and clinical medicine. Prior to Design, Dr. Siffert led a publicly traded biotech company developing gene and cell therapies for devastating degenerative diseases, and previously led research and development organizations in the United States and Europe, including programs that received regulatory approvals followed by commercial launches. Sean Jeffries, Ph.D., our Chief Operating Officer, brings over 20 years of experience in business development, portfolio management, and research and development strategy for both emerging and large biopharmaceuticals companies.

Since our inception, we have raised over $170.0 million in gross proceeds, including from a syndicate of leading life sciences investors that include, among others, Logos Capital, SR One Investments, Quan Capital, Cormorant Asset Management, and West River Capital.

Risks Associated with Our Business

Our business is subject to a number of risks that you should be aware of before making a decision to invest in our common stock. These risks are more fully described in the section titled "Risk Factors" immediately following this prospectus summary. These risks include, among others, the following:

- We have incurred significant net losses since inception, and we anticipate that we will continue to incur significant losses for the foreseeable future and may never be able to achieve or sustain revenues or profitability in the future.
- We have a limited operating history and face significant challenges and will incur substantial expenses as we build our capabilities.
- Even if this offering is successful, we will need substantial additional funding.
- We are early in our development efforts and all of our research programs are still in the preclinical or discovery stage. We have no history of conducting clinical trials to test our product candidates in humans.
- Preclinical and clinical development involves a lengthy and expensive process with uncertain timelines and outcomes, and results of earlier studies and trials may not be predictive of future trial results. If development of our development programs is unsuccessful or delayed, we may be unable to obtain required regulatory approvals and be unable to commercialize our product candidates on a timely basis, if at all.
- Our product candidates are based on novel technologies, which make it difficult to predict the timing, results and cost of product candidate development and likelihood of obtaining regulatory approval.

- We contract with third parties for the manufacturing and supply of our product candidates for use in preclinical testing and planned clinical trials, which supply may become limited or interrupted or may not be of satisfactory quality and quantity.
- Our success is highly dependent on our ability to attract and retain highly skilled executive officers and employees.
- The novel coronavirus-2019 (COVID-19) pandemic could adversely impact our business, including our planned clinical trials.
- Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Corporate Information

We were incorporated under the laws of the State of Delaware on December 18, 2017. Our principal executive offices are located at 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011, and our telephone number is (858) 293-4900. Our corporate website address is www.designtx.com. Information contained on, or accessible through, our website shall not be deemed incorporated into and is not a part of this prospectus or the registration statement of which it forms a part. We have included our website in this prospectus solely as an inactive textual reference.

Trademarks and Service Marks

“Design Therapeutics,” “Design,” “GeneTAC,” the Design logo and other trademarks, trade names or service marks of Design Therapeutics, Inc. appearing in this prospectus are the property of Design Therapeutics, Inc. All other trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert their rights thereto.

Implications of Being an Emerging Growth Company and Smaller Reporting Company

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act (JOBS Act), enacted in April 2012, and we may remain an emerging growth company for up to five years following the completion of this offering. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002 (Sarbanes-Oxley Act), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation-related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision

allows an emerging growth company to delay the adoption of some accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards, and therefore we will be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

We would cease to be an “emerging growth company” upon the earliest to occur of: (i) the last day of the fiscal year in which we have $1.07 billion or more in annual revenue; (ii) the date on which we first qualify as a large accelerated filer under the rules of the Securities and Exchange Commission (SEC); (iii) the date on which we have, in any three-year period, issued more than $1.0 billion in non-convertible debt securities; and (iv) the last day of the fiscal year ending after the fifth anniversary of this offering. We may choose to take advantage of some but not all of these reduced reporting burdens.

We are also a “smaller reporting company” as defined in the Securities and Exchange Act of 1934, as amended (Exchange Act). We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

The Offering	
Common stock to be offered	12,000,000 shares.
Option to purchase additional shares	The underwriters have a 30-day option to purchase up to 1,800,000 additional shares of common stock from us.
Common stock to be outstanding immediately after this offering	53,817,322 shares (or 55,617,322 shares if the underwriters exercise their option to purchase additional shares in full).
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $220.3 million (or approximately $253.8 million if the underwriters exercise in full their option to purchase up to 1,800,000 additional shares of common stock), after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, along with our existing cash, cash equivalents and investment securities, (i) to fund development of our Friedreich ataxia program through the completion of IND-enabling studies and a Phase 1 clinical trial; (ii) to fund development of our myotonic dystrophy type-1 program through the completion of IND-enabling studies and a Phase 1 clinical trial; (iii) to fund development of an additional undisclosed program through product candidate identification and IND-enabling studies; and (iv) to fund our other research and development programs and for general corporate purposes. See the section of this prospectus titled “Use of Proceeds.”
Risk factors	You should read the section of this prospectus titled “Risk Factors” for a discussion of factors to consider carefully, together with all the other information included in this prospectus, before deciding to invest in our common stock.
Nasdaq Global Select Market symbol	“DSGN”
Directed share program	At our request, the underwriters have reserved up to 3% of the shares of our common stock offered by this prospectus, excluding the additional shares that the underwriters have a 30-day option to purchase, for sale, at the initial public offering price, to certain of our employees, certain of our directors

and certain other parties. Shares purchased by our directors and officers will be subject to the 180-day lock-up restriction described in the section of this prospectus titled “Underwriting.” The number of shares of common stock available for sale to the general public will be reduced to the extent these individuals purchase such reserved shares. Any reserved shares that are not so purchased will be offered by the underwriters to the general public on the same basis as the other shares offered by this prospectus.

The number of shares of our common stock to be outstanding after this offering is based on 41,817,322 shares of common stock outstanding as of December 31, 2020 (after giving effect to the conversion of 22,012,499 shares of convertible preferred stock outstanding as of December 31, 2020, as well as the issuance and subsequent conversion of 19,083,979 shares of Series B convertible preferred stock issued and sold in January 2021, into an aggregate of 25,212,548 shares of our common stock immediately prior to the completion of this offering; and which includes 646,953 shares outstanding that are subject to forfeiture or our right to repurchase as of such date), and excludes:

- 1,601,214 shares of our common stock issuable upon the exercise of outstanding stock options as of December 31, 2020, with a weighted-average exercise price of $0.91 per share;
- 328,217 shares of our common stock issuable upon the exercise of outstanding stock options granted subsequent to December 31, 2020, with a weighted-average exercise price of $7.71 per share;
- 7,000,000 shares of common stock reserved for future issuance under our 2021 Equity Incentive Plan (2021 Plan), as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the 2021 Plan, which became effective upon the execution and delivery of the underwriting agreement for this offering (including 881,352 shares of common stock reserved for issuance under our 2018 Equity Incentive Plan (2018 Plan), which shares were added to the 2021 Plan upon its effectiveness); and
- 600,000 shares of common stock reserved for future issuance under our 2021 Employee Stock Purchase Plan (ESPP), as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the ESPP, which became effective upon the execution and delivery of the underwriting agreement for this offering.

Unless otherwise indicated, all information contained in this prospectus, including the number of shares of common stock that will be outstanding after this offering, assumes or gives effect to:

- the conversion of all outstanding shares of our convertible preferred stock as of December 31, 2020 and all of our shares of Series B convertible preferred stock issued and sold in January 2021 into an aggregate of 25,212,548 shares of our common stock immediately prior to the closing of this offering;
- no exercise by the underwriters of their option to purchase up to 1,800,000 additional shares of our common stock;
- no exercise of the outstanding options described above;

- the filing and effectiveness of our amended and restated certificate of incorporation immediately prior to the closing of this offering and the adoption of our amended and restated bylaws immediately prior to the closing of this offering; and
- a one-for-1.63 reverse stock split of our common stock, which we effected on March 22, 2021.

Summary Financial Data

The following tables set forth a summary of our financial data as of, and for the periods ended on, the periods indicated. We have derived the summary statements of operations data for the years ended December 31, 2019 and 2020, and balance sheet data as of December 31, 2020, from our audited financial statements included elsewhere in this prospectus. You should read the following summary financial data together with our financial statements and the related notes included elsewhere in this prospectus and in the sections of this prospectus titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of results that should be expected in any future period.

	Year Ended December 31,	
	2019	2020
	(in thousands, except share and per share amounts)	
Statements of Operations Data:		
Revenue:		
Grant revenue	$ 834	$ 226
Operating expenses:		
Research and development	1,654	6,060
General and administrative	1,088	2,496
Total operating expenses	2,742	8,556
Loss from operations	(1,908)	(8,330)
Other (expense) income, net	(139)	50
Net loss	$ (2,047)	$ (8,280)
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (0.13)	$ (0.52)
Weighted-average shares of common stock used in computing net loss per share attributable to common stockholders, basic and diluted(1)	15,475,415	15,796,674
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(2)		$ (0.30)
Pro forma weighted-average shares of common stock used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(2)		27,193,045

(1) See Note 3 to our financial statements included elsewhere in this prospectus for details on the calculation of our basic and diluted net loss per share attributable to common stockholders.

(2) See the subsection titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Unaudited Pro Forma Information" for an explanation of the calculations of our basic and diluted pro forma net loss per share, and the weighted-average number of shares outstanding used in the computation of the per share amounts.

	As of December 31, 2020		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)
		(unaudited) (in thousands)	
Balance Sheet Data:			
Cash, cash equivalents and investment securities	$ 36,091	$ 160,808	$ 381,113
Working capital(3)	33,903	158,634	379,132
Total assets	36,516	161,219	381,326
Total liabilities	2,475	2,641	2,268
Convertible preferred stock	45,356	—	—
Total stockholders' (deficit) equity	(11,315)	158,758	379,058

(1) The pro forma balance sheet data gives effect to (i) the conversion of all outstanding shares of our convertible preferred stock as of December 31, 2020 into an aggregate of 13,504,598 shares of our common stock in connection with the closing of this offering, (ii) the issuance and sale of shares of our Series B convertible preferred stock in January 2021 for aggregate net proceeds of approximately $124.7 million and the subsequent conversion into 11,707,950 shares of our common stock, in connection with the closing of this offering and (iii) the filing and effectiveness of our amended and restated certificate of incorporation immediately prior to the closing of this offering.

(2) The pro forma as adjusted balance sheet data gives effect to (i) the pro forma adjustments set forth in footnote (1) above and (ii) our receipt of net proceeds from the sale of 12,000,000 shares of our common stock at the initial public offering price of $20.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

(3) Working capital is defined as current assets less current liabilities. See our financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

RISK FACTORS

Investing in our common stock is speculative and involves a high degree of risk. You should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes included elsewhere in this prospectus and in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" before deciding whether to invest in our common stock. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment. This prospectus also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of a number of factors, including the risks described below. See the section titled "Special Note Regarding Forward-Looking Statements."

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We have a limited operating history, have incurred net losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future. We may never generate any revenue or become profitable or, if we achieve profitability, may not be able to sustain it.

We are an early-stage biopharmaceutical company with a limited operating history that may make it difficult to evaluate the success of our business to date and to assess our future viability. Our operations to date have been limited to business planning, organizing and staffing our company, raising capital, developing and optimizing our technology platform, identifying potential product candidates, undertaking research and preclinical studies for our lead program, establishing and enhancing our intellectual property portfolio, and providing general and administrative support for these operations. All of our product candidates are in preclinical development, and none have been approved for commercial sale or tested in human subjects. We have never generated any revenue from product sales and have incurred net losses each year since we commenced operations. For the years ended December 31, 2019 and 2020, our net losses were $2.0 million and $8.3 million, respectively. We expect that it will be several years, if ever, before we have a product candidate ready for regulatory approval and commercialization. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future as we advance our product candidates through clinical development. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity (deficit) and working capital.

To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we succeed in commercializing one or more of our product candidates, we may never generate revenue that is significant or large enough to achieve profitability. In addition, as a young business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown challenges. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis and we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Even if this offering is successful, we will need to obtain substantial additional funding to complete the development and commercialization of our product candidates. If we are unable to raise this capital when needed, we may be forced to delay, reduce or eliminate our product development programs or other operations.

Since our inception, we have used substantial amounts of cash to fund our operations and expect our expenses to increase substantially during the next few years. The development of biopharmaceutical product candidates is capital intensive. As our product candidates enter and advance through preclinical studies and clinical trials, we will need substantial additional funds to expand our clinical, regulatory, quality and manufacturing capabilities. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to marketing, sales, manufacturing and distribution. Furthermore, upon the completion of this offering, we expect to incur additional costs associated with operating as a public company.

As of December 31, 2020, we had $36.1 million in cash, cash equivalents and investment securities. In January 2021, we issued 19,083,979 shares of our Series B convertible preferred stock at $6.55 per share for net proceeds of approximately $124.7 million. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investment securities as of the date of this prospectus, together with the estimated net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next 48 months. However, the expected net proceeds from this offering will not be sufficient to fund any of our product candidates through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates.

We have based these estimates on assumptions that may prove to be incorrect or require adjustment as a result of business decisions, and we could utilize our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the scope, rate of progress and costs of our drug discovery, preclinical development activities, laboratory testing and clinical trials for any future product candidates;
- the number and scope of clinical programs we decide to pursue;
- the scope and costs of manufacturing our future product candidates and commercial manufacturing activities; the emergence of competing therapies and other adverse market developments;
- the cost, timing and outcome of seeking Food and Drug Administration (FDA), European Medicines Agency (EMA) and any other regulatory approvals for any future product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the terms and timing of establishing and maintaining strategic collaborations, licenses and other similar arrangements and the financial terms of such agreements;
- our efforts to enhance operational systems and our ability to attract, hire and retain qualified personnel, including personnel to support the development of our product candidates;
- the costs associated with being a public company;
- the timing of any milestone and royalty payments to Wisconsin Alumni Research Foundation, or other future licensors;
- the extent to which we acquire or in-license other product candidates and technologies;
- our need and ability to retain key management and hire scientific, technical, medical and business personnel;

- our implementation of additional internal systems and infrastructure, including operational, financial and management information systems;
- or costs associated with expanding our facilities or building out our laboratory space;
- the effects of the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide from the COVID-19 pandemic; and
- the cost associated with commercialization activities for any our future product candidates, if approved.

Because we do not expect to generate revenue from product sales for many years, if at all, we will need to obtain substantial additional funding in connection with our continuing operations and expected increases in expenses. Until such time as we can generate significant revenue from sales of our product candidates, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potential collaborations, licenses or other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. The impact of the COVID-19 pandemic on capital markets may affect the availability, amount and type of financing available to us in the future. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

Raising additional capital may cause dilution to our stockholders, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial revenue from product sales to support our cost structure, we expect to finance our operations through public or private equity offerings, debt financings or other capital sources, which may include strategic collaborations, licensing arrangements or other similar arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Equity and debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through strategic collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic or otherwise. Because of the numerous risks and uncertainties associated with product development, we cannot predict the timing or amount of increased expenses and cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Risks Related to the Discovery, Development and Regulatory Approval of Our Product Candidates

We are early in our development efforts and all of our research programs are still in the preclinical or discovery stage. We have no history of conducting clinical trials to test our product candidates in humans.

We are early in our development efforts and most of our operations to date have been limited to developing our platform technologies and conducting drug discovery and preclinical studies. We have not yet begun clinical trials for any of our development programs. As a result, we have limited infrastructure, experience conducting clinical trials as a company and regulatory interactions, and cannot be certain that our planned clinical trials will be completed on time, if at all, that our planned development programs would be acceptable to the FDA or other comparable foreign regulatory authorities, or that, if approval is obtained, such product candidates can be successfully commercialized.

Because of the early stage of development of our products candidates, our ability to eventually generate significant revenues from product sales will depend on a number of factors, including:

- completion of preclinical studies and clinical trials with favorable results;
- acceptance of INDs by the FDA or similar regulatory filing by comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates and our proposed design of future clinical trials;
- receipt of marketing approvals from applicable regulatory authorities, including new drug applications (NDAs), from the FDA and maintaining such approvals;
- market acceptance of any of our approved product candidates;
- making arrangements with our third-party manufacturers for, or establishing, commercial manufacturing capabilities;
- maintaining an acceptable safety profile of our products following approval; and
- maintaining and growing an organization of scientists and business people who can develop our products and technology.

The success of our business, including our ability to finance our company and generate any revenue in the future, will primarily depend on the successful development, regulatory approval and commercialization of our FA and DM1 GeneTAC candidates, as well as our other product candidates, which may never occur. In the future, we may also become dependent on other product candidates that we may develop or acquire; however, given our early stage of development, it may be several years, if at all, before we have demonstrated the safety and efficacy of a treatment sufficient to warrant approval for commercialization. If we are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize our product candidates, we may not be able to generate sufficient revenue to continue our business.

Preclinical and clinical development involves a lengthy and expensive process with uncertain timelines and outcomes, and results of earlier studies and trials may not be predictive of future trial results. If development of our development programs is unsuccessful or delayed, we may be unable to obtain required regulatory approvals and be unable to commercialize our product candidates on a timely basis, if at all.

Clinical drug development is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure or delay can occur at any time during the drug development process,

including due to factors outside of our control. Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful. A number of companies in the pharmaceutical industry, including biotechnology companies, have suffered significant setbacks in clinical trials, even after promising results in earlier nonclinical studies or clinical trials. These setbacks have been caused by, among other things, nonclinical findings made while clinical trials were underway and safety or efficacy observations made in clinical trials, including previously unreported adverse events. The results of preclinical, nonclinical and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical and initial clinical trials. Notwithstanding any potential promising results in earlier studies, we cannot be certain that we will not face similar setbacks. Even if our clinical trials are completed, the results may not be sufficient to obtain regulatory approval for our product candidates.

We may experience delays in initiating our future clinical trials for our product candidates and we cannot be certain that the trials or any other future clinical trials for our product candidates will begin on time, need to be redesigned, enroll an adequate number of patients on time or be completed on schedule, if at all. Clinical trials can be delayed or terminated for a variety of reasons, including delay or failure related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our planned clinical trials, or the sufficiency of preclinical data to initiate clinical trials;
- the size of the study population for further analysis of the study's primary endpoints;
- obtaining regulatory approval to commence a trial;
- reaching agreement on acceptable terms with prospective contract research organizations (CROs) and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining institutional review board (IRB), approval at each site;
- recruiting suitable patients to participate in a trial;
- having patients complete a trial or return for post-treatment follow-up;
- clinical sites deviating from trial protocol or dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- addressing any conflicts with new or existing laws or regulations;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board (DSMB) for such trial or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we plan to do for our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure

of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our planned clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of our product candidates.

If we experience delays in the completion, or termination, of any clinical trial of our product candidates, the commercial prospects of our product candidates may be harmed, and our ability to generate product revenue from any of these product candidates will be delayed or not realized at all. In addition, any delays in completing our planned clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may significantly harm our business, financial condition and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Our product candidates are based on novel technologies, which make it difficult to predict the timing, results and cost of product candidate development and likelihood of obtaining regulatory approval.

We have concentrated our research and development efforts on product candidates using our platform technologies, and our future success depends on the successful development of this approach. We have not yet succeeded and may not succeed in demonstrating efficacy and safety for any product candidates based on our platform technologies in clinical trials or in obtaining marketing approval thereafter, and use of our platform technologies may not ever result in marketable products. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners or establishing our own commercial manufacturing capabilities, which may prevent us from completing our planned clinical trials or commercializing any products on a timely or profitable basis, if at all.

The clinical trial requirements of the FDA, EMA and other comparable foreign regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates.

The biotechnology and biopharmaceutical industries are also rapidly developing, and our competitors may introduce new technologies improving the treatments in the field of expansion repeat driven diseases and small molecules that render our technologies obsolete or less attractive. New technology could emerge at any point in the development cycle of our product candidates.

If we encounter difficulties or delays enrolling patients in our planned clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may not be able to initiate or continue our planned clinical trials for our product candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the clinical trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. We may experience difficulties in patient enrollment or retention in our clinical trials for a variety of reasons. The enrollment of patients depends on many factors, including:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

In addition, our planned clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

We plan to conduct clinical trials for our product candidates outside the United States and the FDA may not accept data from such trials.

We plan to conduct additional clinical trials outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of such study data by the FDA is subject to certain conditions. For example, the clinical trial must be conducted in accordance with Good Clinical Practices (GCP) requirements and the FDA must be able to validate the data from the clinical trial through an onsite inspection if it deems such inspection necessary.

Where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will not approve the application on the basis of foreign data

alone unless those data are applicable to the U.S. population and U.S. medical practice, the clinical trials were performed by clinical investigators of recognized competence, and the data are considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. In addition, such clinical trials would be subject to the applicable local laws of the foreign jurisdictions where the clinical trials are conducted.

There can be no assurance the FDA will accept data from clinical trials conducted outside of the United States. If the FDA does not accept any such data, it would likely result in the need for additional clinical trials, which would be costly and time-consuming and delay aspects of our development plan. In addition, the conduct of clinical trials outside the United States could have a significant impact on us. Risks inherent in conducting international clinical trials include:

- foreign regulatory requirements that could burden or limit our ability to conduct our clinical trials;
- difficulties staffing and managing foreign operations;
- compliance with legal requirements applicable to privacy, data protection, information security and other matters;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including value-added tax and withholding of payroll taxes;
- administrative burdens of conducting clinical trials under multiple foreign regulatory schema;
- foreign exchange fluctuations;
- manufacturing, customs, shipment and storage requirements;
- impact of the COVID-19 pandemic on our ability to produce our product candidates and conduct clinical trials in foreign countries;
- potential liability under the Foreign Corrupt Practices Act of 1977, as amended (FCPA), or comparable foreign regulations;
- cultural differences in medical practice and clinical research; and
- diminished protection of intellectual property in some countries.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials as clinical studies conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, cause us to suspend or discontinue planned clinical trials, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt planned clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our planned clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics.

If unacceptable side effects arise in the development of our product candidates, we, the FDA, the IRBs at the institutions in which our studies are conducted, or the DSMB could suspend or terminate our future clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

In addition, if any of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- we may be forced to suspend marketing of that product, or decide to remove the product form the marketplace;
- regulatory authorities may withdraw or change their approvals of that product;
- regulatory authorities may require additional warnings on the label or limit access of that product to selective specialized centers with additional safety reporting and with requirements that patients be geographically close to these centers for all or part of their treatment;
- we may be required to create a medication guide outlining the risks of the product for patients, or to conduct post-marketing studies;
- we may be required to change the way the product is administered;
- we could be subject to fines, injunctions, or the imposition of criminal or civil penalties, or to sued and held liable for harm caused to subjects or patients; and
- the product may become less competitive, and our reputation may suffer.

Any of the foregoing events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and result in the loss of significant revenue to us, which would materially and adversely affect our results of operations and business.

Interim, topline and preliminary data from our preclinical studies or planned clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, preliminary or topline data from our preclinical studies or planned clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Interim, topline and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, such data should be viewed with caution until the final data are available. From time to time, we may also disclose interim data from our clinical trials. Interim, topline, or preliminary data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between preliminary, interim or topline data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, product candidate or our business. If the interim, topline or preliminary data that we report differ from actual results, or if others including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

The regulatory approval process is lengthy, expensive and uncertain, and we may be unable to obtain regulatory approval for our product candidates under applicable regulatory requirements. The denial or delay of any such approval would delay commercialization of our product candidates and adversely impact our ability to generate revenue, our business and our results of operations.

The development, research, testing, manufacturing, labeling, approval, selling, import, export, marketing, promotion and distribution of drug products are subject to extensive and evolving regulation by federal, state and local governmental authorities in the United States, principally the FDA, and by foreign regulatory authorities, which regulations differ from country to country. Neither we nor any future collaborator is permitted to market any of our product candidates in the United States until we receive regulatory approval of an NDA from the FDA.

Obtaining regulatory approval of an NDA can be a lengthy, expensive and uncertain process. Prior to obtaining approval to commercialize a product candidate in the United States or abroad, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to

the satisfaction of the FDA or other foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. The number of nonclinical studies and clinical trials that will be required for FDA approval varies depending on the product candidate, the disease or condition that the product candidate is designed to address, and the regulations applicable to any particular product candidate.

Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. Administering product candidates to humans may produce undesirable side effects, which could interrupt, delay or halt clinical trials and result in the FDA or other regulatory authorities denying approval of a product candidate for any or all indications. The FDA may also require us to conduct additional studies or trials for our product candidates either prior to or post-approval, such as additional drug-drug interaction studies or safety or efficacy studies or trials, or it may object to elements of our clinical development program such as the number of subjects in our future clinical trials from the United States.

The FDA or any foreign regulatory bodies can delay, limit or deny approval of our product candidates or require us to conduct additional nonclinical or clinical testing or abandon a program for many reasons, including:

- the FDA or the applicable foreign regulatory agency's disagreement with the design or implementation of our planned clinical trials;
- negative or ambiguous results from our clinical trials or results that may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- our inability to demonstrate to the satisfaction of the FDA or the applicable foreign regulatory body that our product candidates are safe and effective for the proposed indication;
- the FDA's or the applicable foreign regulatory agency's disagreement with the interpretation of data from nonclinical studies or clinical trials;
- our inability to demonstrate the clinical and other benefits of our product candidates outweigh any safety or other perceived risks;
- the FDA's or the applicable foreign regulatory agency's requirement for additional nonclinical studies or clinical trials;
- the FDA's or the applicable foreign regulatory agency's disagreement regarding the formulation, labeling and/or the specifications of our product candidates;
- the FDA's or the applicable foreign regulatory agency's failure to approve the manufacturing processes or facilities of third-party manufacturers with which we contract; or
- the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or other regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete our planned clinical testing and receive approval of an NDA or foreign marketing application for our product candidates, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or in the case of the FDA, the implementation of a Risk Evaluation and Mitigation Strategy (REMS), which may be required to ensure safe use of the drug after approval. The FDA or the applicable foreign regulatory agency also may approve a product candidate for a more limited indication or a narrower patient population than we originally requested, and the FDA or applicable foreign regulatory agency may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

The COVID-19 pandemic could adversely impact our business and affect our operations, as well as the business or operations of our manufacturers or other third parties with whom we conduct business.

In December 2019, COVID-19 was reported to have surfaced in Wuhan, China. Since then, the virus has spread to most countries across the world, including all 50 states within the United States, resulting in the World Health Organization characterizing COVID-19 as a pandemic. As a result of measures imposed by the governments in affected regions, many commercial activities, businesses and schools have been suspended as part of quarantines and other measures intended to contain this pandemic. As the COVID-19 pandemic continues to spread around the globe, we may experience disruptions that could severely impact our business and planned clinical trials, including:

- delays or difficulties in enrolling and retaining patients in our planned clinical trials, particularly elderly subjects, who are at a higher risk of severe illness or death from COVID-19;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site; investigators and clinical site staff;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;
- difficulties interpreting data from our clinical trials due to the possible effects of COVID-19 on patients;
- changes in local regulations as part of a response to the COVID-19 outbreak which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- interruptions, difficulties or delays arising in our existing operations and company culture as a result of some of our employees working remotely, including those hired during the COVID-19 pandemic;
- diversion of healthcare resources away from the conduct of future clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- limitations in resources that would otherwise be focused on the conduct of our business, our preclinical studies or our clinical trials, including because of sickness or the desire to avoid contact with large groups of people or as a result of government-imposed “shelter in place” or similar working restrictions;

- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- interruption in global freight and shipping that may affect the transport of clinical trial materials, such as investigational drug product to be used in our clinical trials;
- risks relating to potential disruptions of our contracted manufacturing operations as a result of any potential shut downs or other restrictions in operation due to or impact from the COVID-19 pandemic;
- changes in regulations as part of a response to the COVID-19 pandemic which may require us to change the ways in which our clinical trials are to be conducted, or to discontinue the clinical trials altogether, or which may result in unexpected costs;
- delays in necessary interactions with regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government or contractor personnel;
- risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events; and
- refusal of the FDA to accept data from clinical trials in affected geographies outside of their respective jurisdictions.

These and other disruptions in our operations and the global economy could negatively impact our business, operating results and financial condition.

The COVID-19 pandemic and actions taken to reduce its spread continue to rapidly evolve. The extent to which the COVID-19 pandemic may impede the development of our product candidates, reduce the productivity of our employees, disrupt our supply chains, delay our planned preclinical studies and clinical trials, reduce our access to capital or limit our business development activities, will depend on future developments, which are highly uncertain and cannot be predicted with confidence.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

We may seek orphan drug designation for our product candidates from the FDA and/or from the EMA in the future. However, we may be unsuccessful in obtaining or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We may seek orphan drug designation for our product candidates in the future; however, we may be unsuccessful. Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the U.S. Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products may grant orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition, provided that the condition is affecting not more than five in 10,000 persons in the European Union or if, without incentives, it is unlikely that marketing of the drug in the EU would generate

sufficient returns to justify the investment needed to develop the drug, and no satisfactory method of diagnosis, prevention or treatment of the condition exists (or, if such a method exists, the drug must be of significant benefit to patients). There can be no assurance that the FDA or EMA will grant orphan designation for any indication for which we apply, or that we will be able to maintain such designation.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. If a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of orphan drug exclusivity, which precludes the EMA or the FDA from approving another marketing application for the same drug or biologic for that time period, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity. The applicable exclusivity period is ten years in Europe, but such exclusivity period can be reduced to six years if a product no longer meets the criteria for orphan designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same condition if such regulatory authority concludes that the later drug is clinically superior if it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective.

In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active ingredient. In addition, if a subsequent drug is approved for marketing for the same or a similar indication as any of our product candidates that receive marketing approval, we may face increased competition and lose market share regardless of orphan drug exclusivity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates. Our competitors have developed, are developing or may develop products, product candidates and processes competitive with our product candidates. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of repeat expansion driven diseases, including FA and DM1. Our competitors include larger and better funded pharmaceutical, specialty pharmaceutical and biotechnology companies. Moreover, we may also compete with universities, governmental agencies and other research institutions who may be active in the indications we are targeting and could be in direct competition with us. We also compete with these organizations in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We are aware of a number of companies targeting FA including (i) Reata Pharmaceuticals evaluating omaveloxolone, a Nrf2 activator, (ii) PTC Therapeutics evaluating vatiquinone, a 15-lipoxygenase inhibitor, (iii) Retrotope evaluating RT001, a deuterated poly-saturated fatty acid, (iv) Minoryx Therapeutics evaluating leriglitazone, a PPAR-gamma agonist and (vi) Larimar Therapeutics evaluating CTI-1601, a cell penetrating peptide FXN recombinant fusion protein. In addition, several companies are in preclinical development for AAV-based gene therapies including PTC therapeutics, Voyager Therapeutics, Loxeo Therapeutics, Pfizer, StrideBio, and AavantiBio.

With respect to DM1 patients, AMO Pharma is evaluating tideglusib, a GSK3-ß inhibitor. In addition, there are several products currently in preclinical development for the treatment of DM1, including: a histamine 3 receptor inhibitor by Harmony Biosciences for the treatment of excessive daytime sleepiness in DM1; an antibody linked siRNA by Avidity Biosciences; an AAV-antisense candidate by Audentes Therapeutics; an antibody linked oligonucleotide by Dyne Therapeutics; an miR-23b antisense candidate; gene editing treatments by Vertex Pharmaceuticals; an RNA-targeting AAV-based gene therapy by Locana; an AAV-based RNA degrading gene therapy by Enzerna Biosciences; antisense oligonucleotides by NeuBase Therapeutics; antisense oligonucleotides and siRNA candidates by Triplet Therapeutics; small molecules interacting with RNA by Anima Biotech; small molecule modulators of transcription factors by Syros Pharmaceuticals; and small molecules interacting with RNA by Expansion Therapeutics.

We will also compete more generally with other companies developing alternative scientific and technological approaches to modulate individual genes, including other companies working to develop nuclease-based gene editing technologies, such as Beam Therapeutics, CRISPR Therapeutics, Editas Medicine, Intellia Therapeutics, Precision BioSciences and Sangamo Biosciences.

Many of our competitors, either alone or with their collaborators, have significantly greater financial, technical, manufacturing, marketing, sales and supply resources or experience than we do. If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products, the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. competing products could present superior treatment alternatives, including by being more effective, safer, more convenient, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our

business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Separately, in response to the global COVID-19 pandemic, the FDA and regulatory authorities outside the United States have and may adopt restrictions or other policy measures in response to the COVID-19 pandemic that divert resources and delay their attention to any submissions we may make. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we must prioritize our research programs and will need to focus our discovery and development on select product candidates and indications. Correctly prioritizing our research and development activities is particularly important for us due to the breadth of potential product candidates and indications that we believe could be pursued using our platform technologies. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may also relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We may not be successful in our efforts to identify or discover additional product candidates in the future.

Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for a number of reasons, including:

- our inability to design such product candidates with the properties that we desire; or
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance.

Research programs to identify new product candidates require substantial technical, financial and human resources. If we are unable to identify suitable additional candidates for preclinical and clinical development, our opportunities to successfully develop and commercialize therapeutic products will be limited.

Risks Related to Manufacturing, Commercialization and Reliance on Third Parties

We may rely on third parties to conduct, supervise, and monitor our planned clinical trials and perform some of our research and preclinical studies. If these third parties do not satisfactorily carry out their contractual duties or fail to meet expected deadlines, our development programs may be delayed or subject to increased costs, each of which may have an adverse effect on our business and prospects.

We do not have the ability to conduct all aspects of our preclinical testing or clinical trials ourselves. As a result, we are and expect to remain dependent on third parties to conduct our preclinical studies, and any future clinical trials of our product candidates. The timing of the initiation and completion of these studies and trials will therefore be partially controlled by such third parties and may result in delays to our development programs. Nevertheless, we are responsible for ensuring that each of our preclinical studies and planned clinical trials is conducted in accordance with the applicable protocol, legal requirements, and scientific standards, and our reliance on the CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GLP and GCP requirements, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities enforce these GLP and GCP requirements through periodic inspections of preclinical study sites, trial sponsors, clinical trial investigators and clinical trial sites. If we or any of our CROs or clinical trial sites fail to comply with applicable GLP or GCP requirements, the data generated in our preclinical studies and clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional preclinical or clinical trials before approving our marketing applications. In addition, our clinical trials must be conducted with product produced under current Good Manufacturing Practice (cGMP) regulations. Our failure to comply with these regulations may require us to stop and/or repeat clinical trials, which would delay the marketing approval process.

There is no guarantee that any such CROs, clinical trial investigators or other third parties on which we rely will devote adequate time and resources to our development activities or perform as contractually required. These risks are heightened as a result of the efforts of government agencies and the CROs themselves to limit the spread of COVID-19, including quarantines and shelter-in-place orders. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, otherwise performs in a substandard manner, or terminates its engagement with us, the timelines for our development programs may be extended or delayed or our development activities may be suspended or terminated. If any of our clinical trial sites terminates for any reason, we may experience the loss of follow-up information on subjects enrolled in such clinical trials unless we are able to transfer those subjects to another qualified clinical trial site, which may be difficult or impossible. In addition, clinical trial investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA or any comparable foreign regulatory authority concludes that the financial relationship may have affected the interpretation of the trial, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any marketing application we submit by the FDA or any comparable foreign regulatory authority. Any such delay or rejection could prevent us from commercializing our product candidates.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals

for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our products.

We contract with third parties for the manufacturing and supply of our product candidates for use in preclinical testing and planned clinical trials, which supply may become limited or interrupted or may not be of satisfactory quality and quantity.

We do not have any manufacturing facilities. We rely on third parties for the manufacture of our product candidates for preclinical and clinical testing. We will continue to rely on such third parties for commercial product manufacture, if any of our product candidates are approved. We currently have limited manufacturing arrangements and expect that each of our product candidates will only be covered by single source suppliers for the foreseeable future. This reliance increases the risk that we will not have sufficient quantities of our product candidates or products, if approved, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

Furthermore, all entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in clinical trials must be manufactured in accordance with cGMP requirements. These regulations govern manufacturing processes and procedures, including record keeping, and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of an NDA on a timely basis and must adhere to the FDA's cGMP regulations enforced by the FDA through its facilities inspection program. Comparable foreign regulatory authorities may require compliance with similar requirements. The facilities and quality systems of our third-party contract manufacturers must pass a pre-approval inspection for compliance with the applicable regulations as a condition of marketing approval of our product candidates. We do not have direct control of the manufacturing activities of, and are completely dependent on, our contract manufacturers for compliance with cGMP regulations.

In the event that any of our manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, including due to the impact of the COVID-19 pandemic, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third- party, which we may not be able to do on commercially reasonable terms, if at all. In particular, any replacement of our manufacturers could require significant effort and expertise because there may be a limited number of qualified replacements. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty transferring such skills or technology to another third-party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third-party manufacture our product candidates. If we are required to or voluntarily change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines and that the product produced is equivalent to that produced in a prior facility. The delays associated with the verification of a new manufacturer and equivalent product could negatively affect our ability to develop product candidates in a timely manner or within budget.

Our or a third-party's failure to execute on our manufacturing requirements, or to so execute on commercially reasonable terms and timelines in compliance with cGMP requirements, could adversely affect our business in a number of ways, including:

- inability to meet our product specifications and quality requirements consistently;
- an inability to initiate or continue preclinical studies or clinical trials of our product candidates under development;
- delay in submitting regulatory applications, or receiving marketing approvals, for our product candidates, if at all;
- loss of the cooperation of future collaborators;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease development or to recall batches of our product candidates; and
- in the event of approval to market and commercialize our product candidates, an inability to meet commercial demands for our product or any other future product candidates.

Changes in methods of product candidate manufacturing may result in additional costs or delays.

As product candidates progress through preclinical to late-stage clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize yield, manufacturing batch size, change drug product dosage form, minimize costs and achieve consistent quality and results. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commercialize our product candidates and generate revenue.

Any approved products may fail to achieve the degree of market acceptance by physicians, patients, hospitals, healthcare payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. Most of our product candidates target mechanisms for which there are limited or no currently approved products, which may result in slower adoption by physicians, patients and payors. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the availability of coverage and adequate reimbursement from third-party payor programs, and the willingness of patients to pay out of pocket in the absence of such third-party payor coverage and reimbursement;
- the strength of marketing and distribution support; and
- the prevalence and severity of any side effects.

We may not be able to successfully commercialize our product candidates, if approved, due to unfavorable pricing regulations or third-party coverage and reimbursement policies, which could make it difficult for us to sell our product candidates profitably.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time-consuming and costly process, with uncertain results, that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to the payor. There may be significant delays in obtaining such coverage and reimbursement for newly approved products, and coverage may not be available, or may be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacture, sale and distribution expenses. Interim reimbursement levels for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost products and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, by any future laws limiting drug prices and by any future relaxation of laws that presently restrict imports of product from countries where they may be sold at lower prices than in the United States.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, there is no uniform policy among third-party payors for coverage and reimbursement. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting reimbursement policies, but also have their own methods and approval process apart from Medicare coverage and reimbursement determinations. Therefore, one third-party payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product.

Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that reimbursement will be available for any product that we commercialize and, if coverage and reimbursement are available, what the level of reimbursement will be. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with branded therapeutics and therapeutics administered under the supervision of a physician. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Reimbursement may impact the demand for, and the price of, any product for which we obtain marketing approval. Even if we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and

their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with those medications. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement are critical to a new product's acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new products when more established or lower cost therapeutic alternatives are already available or subsequently become available.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself may or may not be available. Instead, the hospital or administering physician may be reimbursed only for providing the treatment or procedure in which our product is used. Further, from time to time, CMS revises the reimbursement systems used to reimburse health care providers, including the Medicare Physician Fee Schedule and Hospital Outpatient Prospective Payment System, which may result in reduced Medicare payments.

We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription medicines, medical devices and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the successful commercialization of new products. Further, the adoption and implementation of any future governmental cost containment or other health reform initiative may result in additional downward pressure on the price that we may receive for any approved product.

Additionally, we or collaborators may develop companion diagnostic tests for use with our product candidates. We, or our collaborators, will be required to obtain coverage and reimbursement for these tests separate and apart from the coverage and reimbursement we may seek for our product candidates. While we have not yet developed any companion diagnostic tests for our product candidates, if we do, there is significant uncertainty regarding our ability to obtain coverage and adequate reimbursement for the same reasons applicable to our product candidates.

Outside of the United States, many countries require approval of the sale price of a product before it can be marketed, and the pricing review period only begins after marketing or product licensing approval is granted. To obtain reimbursement or pricing approval in some of these countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue, if any, we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if such product candidates obtain marketing approval.

If the market opportunities for any of our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.

We are focused initially on the development of treatments for nucleotide expansion repeat diseases. Our projections of addressable patient populations that have the potential to benefit from treatment with our product candidates are based on estimates. If any of our estimates are inaccurate, the market opportunities for any of our product candidates could be significantly diminished and have an adverse material impact on our business.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to research and develop and to manufacture our product candidates, we must share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we may collaborate with will likely expect to be granted rights to publish data arising out of such collaboration and any joint research and development programs may require us to share trade secrets under the terms of our research and development or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

If any of our product candidates are approved for marketing and commercialization and we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we will be unable to successfully commercialize our product candidates if and when they are approved.

We have no sales, marketing or distribution capabilities or experience. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization, which would be expensive and time consuming, or outsource these functions to other third parties. In the future, we may choose to build a focused sales and marketing infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize future products on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product portfolios; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability of these product revenue to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. In entering into third-party marketing or distribution arrangements, any revenue we receive will depend upon the efforts of the third parties and we cannot assure you that such third parties will establish adequate sales and distribution capabilities or devote the necessary resources and attention to sell and market any future products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Even if we obtain FDA approval of any of our product candidates, we may never obtain approval or commercialize such products outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals could result in significant delays, difficulties and costs for us and may require additional preclinical studies or clinical trials which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, our ability to realize the full market potential of our products will be harmed.

Risks Related to Our In-Licenses and Other Strategic Agreements

We may not realize the benefits of any acquisitions, in-license or strategic alliances that we enter into.

We have entered into in-license agreements with multiple licensors and in the future may seek and form strategic alliances, create joint ventures or collaborations, or enter into acquisitions or additional licensing arrangements with third parties that we believe will complement or augment our existing technologies and product candidates.

These transactions can entail numerous operational and financial risks, including exposure to unknown liabilities, disruption of our business and diversion of our management’s time and attention in

order to manage a collaboration or develop acquired products, product candidates or technologies, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition or integration costs, write-downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, impairment of relationships with key suppliers, manufacturers or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. As a result, if we enter into acquisition or in-license agreements or strategic partnerships, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture, or if there are materially adverse impacts on our or the counterparty's operations resulting from COVID-19, which could delay our timelines or otherwise adversely affect our business. We also cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction or such other benefits that led us to enter into the arrangement.

We may wish to form collaborations in the future with respect to our product candidates, but may not be able to do so or to realize the potential benefits of such transactions, which may cause us to alter or delay our development and commercialization plans.

The development and potential commercialization of our product candidates will require substantial additional capital to fund expenses. We may, in the future, decide to collaborate with other biopharmaceutical companies for the development and potential commercialization of those product candidates, including in territories outside the United States or for certain indications. We will face significant competition in seeking appropriate collaborators. We may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If and when we collaborate with a third-party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third-party. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of our technologies, product candidates and market opportunities. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under any license agreements from entering into agreements on certain terms or at all with potential collaborators.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators and changes to the strategies of the combined company. As a result, we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay one or more of our other development programs, delay the potential commercialization or reduce the scope of any planned sales or marketing activities for such product candidate, or increase our expenditures and undertake development, manufacturing or commercialization activities at our own expense. If we elect to increase our expenditures to fund development, manufacturing or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Our product candidates may also require specific technologies to work effectively and efficiently, and rights to those technologies may be held by others. We may be unable to in-license any compositions, methods of use, processes or other third party intellectual property rights from third parties that we identify. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, which would harm our business. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Risks Related to Our Industry and Business Operations

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, diminished profits and future earnings, additional reporting obligations and oversight if subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use of our product candidates harms patients or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;

- withdrawal of clinical trial participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

We may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claims, or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

We face an inherent risk of product liability as a result of the planned studies and clinical trials of our product candidates and will face an even greater risk if we commercialize our product candidates. For example, we may be sued if our product candidates allegedly cause injury or are found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product candidate, negligence, strict liability and a breach of warranties. Claims may be brought against us by clinical trial participants, patients or others using, administering or selling products that may be approved in the future. Claims could also be asserted under state consumer protection acts.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

We conduct substantially all of our operations remotely and at our leased laboratory and office space in La Jolla, California and office space in Carlsbad, California. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options and restricted stock awards that vest over time. The value to employees of stock awards and restricted stock awards that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate

their employment with us on short notice. Although we have employment agreements with certain of our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key person” insurance policies on the lives of these individuals or the lives of any of our employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

We expect to expand our development, regulatory and operational capabilities and, as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of March 22, 2021, we had 22 employees, which represents an increase of 20 employees since January 1, 2020. As we advance our research and development programs, we may be required to further increase the number of our employees and the scope of our operations, particularly in the areas of clinical development, discovery biology, chemistry, product development, general and administrative matters relating to being a public company, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage any future growth, we must:

- identify, recruit integrate, maintain and motivate additional qualified personnel;
- manage our development efforts effectively, including the initiation and conduct of clinical trials for our product candidates; and
- improve our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates, if approved, will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and other resources, and a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. Under legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act (Tax Act), as modified by legislation enacted on March 27, 2020, entitled the Coronavirus Aid, Relief, and Economic Security Act (CARES Act), U.S. federal net operating losses (NOLs), incurred in taxable years beginning after December 31, 2017, can be carried forward indefinitely to offset future taxable income, but the deductibility of such U.S. federal NOLs in taxable years beginning after December 31, 2020, is limited to 80% of taxable income. It is uncertain if and to what extent various states will conform to the Tax Act or the CARES Act.

As of December 31, 2020, we had $10.1 million of U.S. federal NOLs and $10.3 million of state NOLs. U.S. federal NOL carryforwards totaling $0.1 million will begin to expire in 2037 unless previously utilized, and U.S. federal NOL carryforwards of $10.0 million can be carried forward indefinitely under current law. State NOL carryforwards totaling $10.3 million will begin to expire in 2037, unless previously utilized. As of December 31, 2020, we also had aggregate U.S. federal and state research and development (R&D) credits of approximately $0.1 million and $0.2 million, respectively. U.S. federal R&D credits carryforwards begin to expire in 2038 unless previously utilized. The state R&D credit carryforwards do not expire. Our NOL carryforwards and R&D credits are subject to review and possible adjustment by the U.S. and state tax authorities.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the Code), and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards, R&D credits and certain other tax attributes to offset its post-change income or taxes may be limited. This could limit the amount of NOLs, R&D credit carryforwards or other applicable tax attributes that we can utilize annually to offset future taxable income or tax liabilities. Subsequent ownership changes and changes to the U.S. tax rules in respect of the utilization of NOLs, R&D credits and other applicable tax attributes carried forward may further affect the limitation in future years. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, California recently imposed limits on the usability of California state NOL carryforwards to offset taxable income in tax years beginning after 2019 and before 2023. As a result, we may be unable to use all or a material portion of our NOL carryforwards and other tax attributes, which could adversely affect our future cash flows. We have not undertaken a Section 382 study, and it is possible that we have previously undergone one or more ownership changes so that our use of net operating losses is subject to limitation. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Our business operations and current and future relationships with investigators, health care professionals, consultants, third-party payors and customers are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, transparency laws and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Although we do not currently have any products on the market, our operations may be, directly or indirectly through our prescribers, customers and third-party payors, subject to various U.S. federal and state healthcare laws and regulations, including, without limitation, the U.S. federal Anti-Kickback Statute, the U.S. federal civil and criminal false claims laws and the Physician Payments Sunshine Act and regulations. Healthcare providers and others play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. These laws may impact, among other things, our current business operations, including our clinical research activities, and proposed sales, marketing and education programs and constrain the business of financial arrangements and relationships with healthcare providers and other parties through which we may market, sell and distribute our products for which we obtain marketing approval. In addition, we may be subject to additional healthcare, statutory and regulatory requirements and enforcement by foreign regulatory authorities in jurisdictions in which we conduct our business. The laws that may affect our ability to operate include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or paying any remuneration (including any kickback, bribe or certain rebates), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the U.S. federal false claims, including the False Claims Act, which can be enforced through whistleblower actions, and civil monetary penalties laws, which, among other things, impose criminal and civil penalties against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the U.S. federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services; similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, (HITECH), and their respective implementing regulations, impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates and their covered subcontractors that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security, and transmission of such individually identifiable health information;
- the U.S. Federal Food, Drug and Cosmetic Act, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. federal legislation commonly referred to as Physician Payments Sunshine Act, enacted as part of the Affordable Care Act, and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members. Beginning in 2022, such obligations will include payments and other transfers of value provided in the previous year to certain other healthcare professionals, including physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse midwives;
- analogous state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including, but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; and state and local laws requiring the registration of pharmaceutical sales representatives; and

- European and other foreign law equivalents of each of the laws, including reporting requirements detailing interactions with and payments to healthcare providers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government funded healthcare programs, such as Medicare and Medicaid, or similar programs in other countries or jurisdictions, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the delay, reduction, termination or restructuring of our operations. Further, defending against any such actions can be costly and time-consuming, and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may charge for such product candidates.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product for which we obtain marketing approval.

In March 2010, the Affordable Care Act was enacted, which includes measures that have significantly changed the way health care is financed by both governmental and private insurers. There have been executive, judicial and congressional challenges to certain aspects of the Affordable Care Act. On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the “individual mandate” was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. The United States Supreme Court is currently reviewing this case, but it is unknown when a decision will be reached. Although the Supreme Court has not yet ruled on the constitutionality of the Affordable Care Act, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through May 15, 2021 for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. It is also unclear how the Supreme Court ruling, other such litigation and the healthcare reform measures of the Biden administration will impact the Affordable Care Act or our business. We continue to evaluate the effect that the Affordable Care Act and its possible repeal and replacement has on our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, in August 2011, the Budget Control Act of 2011 was signed into law, which, among other things, included aggregate reductions to Medicare payments to providers of, on average, 2% per fiscal year through 2030, with the exception of a temporary suspension from May 1, 2020 through March 31, 2021, unless Congress takes additional action.

Recently, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. congressional inquiries and legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives.

For example, on July 24, 2020 and September 13, 2020, the Trump administration announced several executive orders related to prescription drug pricing that seek to implement several of the administration's proposals. As a result, the FDA released a final rule on September 24, 2020, effective November 30, 2020, providing guidance for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the United States Department of Health and Human Services (HHS) finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed pending review by the Biden administration until March 22, 2021. On November 20, 2020, CMS issued an interim final rule implementing President Trump's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. It is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control costs pharmaceutical and biological products. Moreover, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We expect that the healthcare reform measures that have been adopted, and that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products. Further, it is possible that additional governmental action is taken in response to the COVID-19 pandemic.

Failure to comply with current or future federal, state and foreign laws and regulations and industry standards relating to privacy and data protection laws could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business.

We and our collaborators and third-party providers may be subject to federal, state and foreign data privacy and security laws and regulations. In the United States, numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our collaborators and third-party providers. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. Depending on the facts and circumstances, we could be subject to significant penalties if we violate HIPAA.

In many jurisdictions, enforcement actions and consequences for noncompliance are rising. In the United States, these include enforcement actions in response to rules and regulations promulgated under the authority of federal agencies and state attorneys general and legislatures and consumer protection agencies. In addition, privacy advocates and industry groups have regularly proposed, and may propose in the future, self-regulatory standards that may legally or contractually apply to us. If we fail to follow these security standards, even if no customer information is compromised, we may incur significant fines or experience a significant increase in costs. Many state legislatures have adopted legislation that regulates how businesses operate online, including measures relating to privacy, data security and data breaches. Laws in all 50 states require businesses to provide notice to customers whose personally identifiable information has been disclosed as a result of a data breach. The laws are not consistent, and compliance in the event of a widespread data breach is costly. States are also constantly amending existing laws, requiring attention to frequently changing regulatory requirements. Furthermore, California recently enacted the California Consumer Privacy Act (the CCPA) which became effective on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. At this time, we do not collect personal information relating to residents of California but should we begin to do so, the CCPA will impose new and burdensome privacy compliance obligations on our business and will raise new risks for potential fines and class actions.

Foreign data protection laws, including the EU General Data Protection Regulation (the GDPR), may also apply to health-related and other personal information obtained outside of the United States. The GDPR, which came into effect on May 25, 2018, imposes strict requirements for processing the personal data of individuals within the European Economic Area (EEA) and the United Kingdom, including clinical trial data, as well as potential fines for noncompliant companies of up to the greater of €20 million or 4% of annual global revenue. The GDPR imposes strict requirements for the collection, use and disclosure of personal data, including stringent requirements relating to obtaining consent, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches and taking certain measures when engaging third-party processors. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EU and the United States remains uncertain. For example, in 2016, the EU and United States agreed to a transfer framework for data transferred from the EU to the United States, called the Privacy Shield, but the Privacy Shield was invalidated in July

2020 by the Court of Justice of the European Union. At this time, we do not believe we are subject to the GDPR, but should this change, the GDPR will increase our responsibility and potential liability in relation to personal data that we process, and we may be required to put in place additional mechanisms to ensure compliance with the new EU data protection rules.

Compliance with U.S. and foreign data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Failure by us or our collaborators and third-party providers to comply with U.S. and foreign data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties), private litigation and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects about whom we or our potential collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose such information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend, could result in adverse publicity and could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for our platform technologies and product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our products may be adversely affected.

We rely upon a combination of patents, know-how and confidentiality agreements to protect the intellectual property related to our products and technologies and to prevent third parties from copying and surpassing our achievements, thus eroding our competitive position in our market.

Our success depends in large part on our ability to obtain and maintain patent protection for our platform technologies, product candidates and their uses, as well as our ability to operate without infringing the proprietary rights of others. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel discoveries and technologies that are important to our business. Our pending and future patent applications may not result in patents being issued or that issued patents will afford sufficient protection of our product candidates or their intended uses against competitors, nor can there be any assurance that the patents issued will not be infringed, designed around, invalidated by third parties, or effectively prevent others from commercializing competitive technologies, products or product candidates.

Obtaining and enforcing patents is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications or maintain and/or enforce patents that may issue based on our patent applications, at a reasonable cost or in a timely manner, including delays as a result of the COVID-19 pandemic impacting our or our licensors' operations. It is also possible that we will fail to identify patentable aspects of our research and development results before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract research organizations, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such results before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

Composition of matter patents for biological and pharmaceutical product candidates often provide a strong form of intellectual property protection for those types of products, as such patents provide

protection without regard to any method of use. We cannot be certain that the claims in our pending patent applications directed to composition of matter of our product candidates will be considered patentable by the United States Patent and Trademark Office (USPTO) or by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid and enforceable by courts in the United States or foreign countries. Method of use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products "off-label." Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation, resulting in court decisions, including Supreme Court decisions, which have increased uncertainties as to the ability to enforce patent rights in the future. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our potential future collaborators will be successful in protecting our product candidates by obtaining and defending patents. For example, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates or their intended uses, and as a result the impact of such third-party intellectual property rights upon the patentability of our own patents and patent applications, as well as the impact of such third-party intellectual property upon our freedom to operate, is highly uncertain. Patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, inventorship, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending patent applications may be challenged in patent offices in the United States and abroad. Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. For example, our pending patent applications may be subject to third-party pre-issuance submissions of prior art to the USPTO or our issued patents may be subject to post-grant review (PGR) proceedings, oppositions, derivations, reexaminations, or *inter partes* review (IPR) proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated, or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. The degree of future protection for our proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. Any failure to obtain or maintain patent protection with respect to our product candidates or their uses could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. We may

also rely on trade secret protection as temporary protection for concepts that may be included in a future patent filing. However, trade secret protection will not protect us from innovations that a competitor develops independently of our proprietary know how. If a competitor independently develops a technology that we protect as a trade secret and files a patent application on that technology, then we may not be able to patent that technology in the future, may require a license from the competitor to use our own know-how, and if the license is not available on commercially-viable terms, then we may not be able to launch our product. Although we require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, and this scenario could materially adversely affect our business, financial condition and results of operations.

We cannot ensure that patent rights relating to inventions described and claimed in our pending patent applications will issue or that patents based on our patent applications will not be challenged and rendered invalid and/or unenforceable.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our potential future collaborators will be successful in protecting our product candidates by obtaining and defending patents. We have pending U.S. and foreign patent applications in our portfolio; however, we cannot predict:

- if and when patents may issue based on our patent applications;
- the scope of protection of any patent issuing based on our patent applications;
- whether the claims of any patent issuing based on our patent applications will provide protection against competitors;
- whether or not third parties will find ways to invalidate or circumvent our patent rights;
- whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications;
- whether we will need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose;
- whether the patent applications that we own or in-license will result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries; and/or
- whether, as the COVID-19 pandemic continues to spread around the globe, we may experience patent office interruption or delays to our ability to timely secure patent coverage to our product candidates.

We cannot be certain that the claims in our pending patent applications directed to our product candidates and/or technologies will be considered patentable by the USPTO or by patent offices in foreign countries. There can be no assurance that any such patent applications will issue as granted patents. One aspect of the determination of patentability of our inventions depends on the scope and content of the “prior art,” information that was or is deemed available to a person of skill in the relevant

art prior to the priority date of the claimed invention. There may be prior art of which we are not aware that may affect the patentability of our patent claims or, if issued, affect the validity or enforceability of a patent claim. Even if the patents do issue based on our patent applications, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop and threaten our ability to commercialize our product candidates. In the event of litigation or administrative proceedings, we cannot be certain that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect, and although we currently have an issued patent and pending applications in the United States, filing, prosecuting and defending patents on all of our research programs and product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These competitor products may compete with our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Various companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many countries do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights.

Various countries outside the United States have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. As a result, a patent owner may have limited remedies in certain circumstances, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Further, the standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. As such, we do not know the degree of future protection that we will have on our technologies, products and product candidates. While we will endeavor to try to protect our technologies, products and product candidates with intellectual property rights such as patents, as appropriate, the process of obtaining patents is time consuming, expensive and unpredictable.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make product candidates that are similar to ours but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that noncompliance with the USPTO and foreign governmental patent agencies requirement for a number of procedural, documentary, fee payment and other provisions during the patent process can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be revoked, modified, or held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- we cannot predict the scope of protection of any patent issuing based on our patent applications, including whether the patent applications that we own or in-license will result in issued patents with claims that directed to our product candidates or uses thereof in the United States or in other foreign countries;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates;
- the claims of any patent issuing based on our patent applications may not provide protection against competitors or any competitive advantages, or may be challenged by third parties;
- if enforced, a court may not hold that our patents are valid, enforceable and infringed;
- we may need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose;
- we may choose not to file a patent application in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent application covering such intellectual property;

- we may fail to adequately protect and police our trademarks and trade secrets; and
- the patents of others may have an adverse effect on our business, including if others obtain patents claiming subject matter similar to or improving that covered by our patents and patent applications.

Should any of these or similar events occur, they could significantly harm our business, results of operations and prospects.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

As the biopharmaceutical industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. There can be no assurance that our operations do not, or will not in the future, infringe existing or future third-party patents. Identification of third-party patent rights that may be relevant to our operations is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction.

Numerous U.S. and foreign patents and pending patent applications exist in our market that are owned by third parties. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our products. We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. Patent applications in the United States and elsewhere are typically published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Certain U.S. applications that will not be filed outside the U.S. can remain confidential until patents issue. In addition, patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived. Furthermore, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our products or the use of our products. As such, there may be applications of others now pending or recently revived patents of which we are unaware. These patent applications may later result in issued patents, or the revival of previously abandoned patents, that will prevent, limit or otherwise interfere with our ability to make, use or sell our products.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect. For example, we may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third-party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products.

We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, including our research programs, product candidates, their respective

methods of use, manufacture and formulations thereof, and could result in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

Because our development programs may in the future require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license, or use these third-party proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to our product candidates, there may be times when the filing and prosecution activities for patents and patent applications relating to our product candidates are controlled by our future licensors or collaboration partners. If any of our future licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering our product candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensees, our future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

We may enter into license agreements in the future with others to advance our existing or future research or allow commercialization of our existing or future product candidates. These licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our future licensors fail to prosecute, maintain, enforce, and defend such patents or patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our future product candidates that are subject of such licensed rights could be adversely affected.

Our future licensors may rely on third-party consultants or collaborators or on funds from third parties such that our future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to our future in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- our right to transfer or assign the license;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we license in the future prevent or impair our ability to maintain our licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

In spite of our best efforts, our future licensors might conclude that we materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these

in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

From time to time, we may be required to license technologies relating to our therapeutic research programs from additional third parties to further develop or commercialize our product candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our product candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our product candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

Any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our products or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities, or the ongoing COVID-19 pandemic;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our future product candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable future product candidates;
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator’s sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Our technology licensed from various third parties may be subject to retained rights.

We are currently party to a license agreement with Wisconsin Alumni Research Foundation (WARF) pursuant to which we acquired an exclusive license to certain patents relating to compounds and

methods for modulating gene expression, compounds and methods for modulating FA expression and next generation synthetic transcription factors. WARF retains, and our future licensors may also retain, certain rights under the relevant agreements with us, including the right to use the underlying technology for noncommercial academic and research use, to publish general scientific findings from research related to the technology, and to make customary scientific and scholarly disclosures of information relating to the technology. It is difficult to monitor whether our licensors limit their use of the technology to these uses, and we could incur substantial expenses to enforce our rights to our licensed technology in the event of misuse.

In addition, the United States federal government retains certain rights in inventions produced with its financial assistance under the Patent and Trademark Law Amendments Act, or the Bayh-Dole Act. The federal government retains a “nonexclusive, nontransferable, irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights.” March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself. We sometimes collaborate with academic institutions to accelerate our preclinical research or development. While it is our policy to avoid engaging our university partners in projects in which there is a risk that federal funds may be commingled, we cannot be sure that any co-developed intellectual property will be free from government rights pursuant to the Bayh-Dole Act. If, in the future, we co-own or license in technology which is critical to our business that is developed in whole or in part with federal funds subject to the Bayh-Dole Act, our ability to enforce or otherwise exploit patents covering such technology may be adversely affected.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Third parties may allege that we have infringed or misappropriated their intellectual property. Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and, even if resolved in our favor, is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the market price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates. We cannot be certain that our product candidates and other proprietary technologies we may develop will not infringe existing or future patents owned by third parties. Third parties may assert infringement claims against us based on existing or future intellectual property rights. In the United States, proving

invalidity in court requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing candidate product or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing candidate product or product. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our investigational products or force us to cease some of our business operations, which could materially harm our business.

We may not be aware of patents that have already been issued and that a third party, for example, a competitor in the fields in which we are developing our product candidates, might assert are infringed by our future product candidates, including claims to compositions, formulations, methods of manufacture or methods of use or treatment that cover our product candidates. It is also possible that patents owned by third parties of which we are aware, but which we do not believe are relevant to our product candidates and other proprietary technologies we may develop, could be found to be infringed by our product candidate. In addition, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our product candidates. The pharmaceutical and biotechnology industries have produced a considerable number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity may be difficult. For example, in the United States, proving invalidity in court requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents, and there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on our business and operations. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

We may choose to challenge the enforceability or validity of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third party's patent in patent opposition proceedings in the EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or proprietary technologies.

If we are found to infringe a third-party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third-party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, and could divert the time and attention of our technical personnel and management, cause development delays, and/or require us to develop non-infringing technology, which may not be possible on a cost-effective basis, any of which could materially harm our business. In the event of a successful claim of infringement against us, we may have to pay substantial monetary damages, including treble damages and attorneys' fees for willful infringement, pay royalties and other fees, redesign our infringing drug or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors or other third parties may infringe our patents, trademarks or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention, or decide that the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1). An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, we cannot assure you that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third-party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders, or it may be otherwise impractical or undesirable to enforce our intellectual property against some third parties. Our competitors or other third parties may be able to sustain the costs of complex patent litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology or other product candidates, or enter into development partnerships that would help us bring our product candidates to market.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

As is common in the pharmaceutical industry, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other pharmaceutical companies including our competitors or potential competitors. We could in the future be subject to claims that we or our employees have inadvertently or otherwise used or disclosed alleged trade secrets or other confidential information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know-how or trade secrets of others in their work for us, we may become subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement, or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor.

While we may litigate to defend ourselves against these claims, even if we are successful, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our product candidates, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information

of the former employers. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Changes in patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs, and may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. Patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act (the Leathy-Smith Act), signed into law on September 16, 2011, could increase those uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Further, because of a lower evidentiary standard in these USPTO post-grant proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Thus, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

After March 2013, under the Leahy-Smith Act, the United States transitioned to a first inventor to file system in which, assuming that the other statutory requirements are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third-party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before we file an application covering the same invention, could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates and other proprietary technologies we may develop or (ii) invent any of the inventions claimed in our or our licensor’s patents or patent applications. Even where we have a valid and enforceable patent, we may not be able to exclude others from practicing the claimed invention where the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or

defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the 2013 case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuities fees and various other governmental fees on patents and/or patent applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent and/or patent application. The USPTO and various foreign governmental patent agencies also require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse, including due to the effect of the COVID-19 pandemic on us or our patent maintenance vendors, can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

We may rely on trade secret and proprietary know-how which can be difficult to trace and enforce and, if we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and product candidates, we may also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. Elements of our product candidate, including processes for their preparation and manufacture, may involve proprietary know-how, information, or technology that is not covered by patents, and thus for these aspects we may consider trade secrets and know-how to be our primary intellectual property. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or third-party consultants and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Because we expect to rely on third parties in the development and manufacture of our product candidates, we must, at times, share trade secrets with them. Our reliance

on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Trade secrets and know-how can be difficult to protect. We require our employees to enter into written employment agreements containing provisions of confidentiality and obligations to assign to us any inventions generated in the course of their employment. We and any third parties with whom we share facilities enter into written agreements that include confidentiality and intellectual property obligations to protect each party's property, potential trade secrets, proprietary know-how, and information. We further seek to protect our potential trade secrets, proprietary know-how, and information in part, by entering into non-disclosure and confidentiality agreements with parties who are given access to them, such as our corporate collaborators, outside scientific collaborators, contract research organizations, contract manufacturers, consultants, advisors and other third parties. With our consultants, contractors, and outside scientific collaborators, these agreements typically include invention assignment obligations. We cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes. We cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We may need to share our proprietary information, including trade secrets, with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors, and those affiliated with or controlled by state actors. Further, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third-party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third-party, our competitive position would be harmed.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as the U.S. government, such that our licensors are not the sole and exclusive owners of

the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patent rights are of limited duration. In the United States, if all maintenance fees are paid timely, the natural expiration of a patent is generally 20 years after its first effective filing date. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from biosimilar or generic products. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours. Upon issuance in the United States, the term of a patent can be increased by patent term adjustment, which is based on certain delays caused by the USPTO, but this increase can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. The term of a United States patent may also be shortened if the patent is terminally disclaimed over an earlier-filed patent. A patent term extension (PTE) based on regulatory delay may be available in the United States. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single product. Moreover, the scope of protection during the period of the PTE does not extend to the full scope of the claim, but instead only to the scope of the product as approved. Laws governing analogous PTEs in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent family. Additionally, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. If we are unable to obtain PTE or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration and may take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to launch their product earlier than might otherwise be the case, and our revenue could be reduced, possibly materially.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or descriptive or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest.

During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Although these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names.

Moreover, any name we have proposed to use with our product candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. Similar requirements exist in Europe. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, it may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks Related to Our Common Stock and this Offering

There has been no prior public market for our common stock, the stock price of our common stock may be volatile or may decline regardless of our operating performance and you may not be able to resell your shares at or above the initial public offering price.

There has been no public market for our common stock prior to this offering. The initial public offering price for our common stock was determined through negotiations between the underwriters and us and may vary from the market price of our common stock following this offering. If you purchase shares of our common stock in this offering, you may not be able to resell those shares at or above the initial public offering price. An active or liquid market in our common stock may not develop upon the completion of this offering or, if it does develop, it may not be sustainable. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration. The market price of our common stock may fluctuate significantly in response to numerous factors, many of which are beyond our control, including:

- overall performance of the equity markets;
- our operating performance and the performance of other similar companies;
- the published opinions and third-party valuations by banking and market analysts;

- results from our future clinical trials with our future product candidates or of our competitors;
- adverse results or delays in clinical trials;
- failure to commercialize our product candidates;
- unanticipated serious safety concerns related to the use of our product candidates;
- changes in our projected operating results that we provide to the public, our failure to meet these projections or changes in recommendations by securities analysts that elect to follow our common stock;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- regulatory or legal developments in the United States and other countries;
- changes in the structure of healthcare payment systems;
- the level of expenses related to future product candidates or clinical development programs;
- our failure to achieve product development goals in the timeframe we announce;
- announcements of acquisitions, strategic alliances or significant agreements by us or by our competitors;
- recruitment or departure of key personnel;
- the economy as a whole and market conditions in our industry;
- trading activity by a limited number of stockholders who together beneficially own a majority of our outstanding common stock;
- the expiration of market standoff or contractual lock-up agreements;
- the size of our market float;
- political uncertainty and/or instability in the United States;
- the ongoing and future impact of the COVID-19 pandemic and actions taken to slow its spread; and
- any other factors discussed in this prospectus.

In addition, the stock markets have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many biopharmaceutical companies. Stock prices of many biopharmaceutical companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those companies. The trading prices for common stock of other biopharmaceutical companies have also been highly volatile as a result of the COVID-19 pandemic. In the past, stockholders have filed securities class action litigation following periods of market volatility. If we were to become involved in securities litigation, it could subject us to substantial costs, divert resources and the attention of management from our business and adversely affect our business.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of January 25, 2021, our executive officers, directors, five percent stockholders and their affiliates beneficially owned approximately 80% of our voting stock and, upon closing of this offering,

that same group will beneficially own approximately 62% of our outstanding voting stock, assuming no purchases of any shares of common stock in this offering pursuant to the contemplated directed share program or otherwise. Three of our seven directors were appointed by our significant stockholders pursuant to our amended and restated voting agreement, which will terminate upon the closing of this offering. Therefore, even after this offering, these stockholders will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

As described in more detail under the section titled “Non-Employee Director Compensation,” our board of directors has approved option grants to each of our non-employee directors upon the execution and delivery of the underwriting agreement for this offering, and therefore our non-employee directors may have interests in and arising from this offering that are different from and/or may conflict with the interests of our stockholders.

Substantial amounts of our outstanding shares may be sold into the market when lock-up or market standoff periods end. If there are substantial sales of shares of our common stock, the price of our common stock could decline.

The price of our common stock could decline if there are substantial sales of our common stock, particularly sales by our directors, executive officers and significant stockholders, or if there is a large number of shares of our common stock available for sale and the market perceives that sales will occur. After this offering, we will have 53,817,322 outstanding shares of our common stock, based on the number of shares outstanding as of December 31, 2020 (giving effect to the issuance and subsequent conversion of 19,083,979 shares of Series B convertible preferred stock issued and sold in January 2021). All of the shares of common stock sold in this offering will be available for sale in the public market. Substantially all of our outstanding shares of common stock are currently restricted from resale as a result of market standoff and lock-up agreements, as more fully described in the section of this prospectus titled “Shares Eligible for Future Sale.” These shares will become available to be sold 181 days after the date of this prospectus, in addition to shares issuable pursuant to outstanding options. Shares held by directors, executive officers and other affiliates will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended (the Securities Act) and various vesting agreements.

After the completion of this offering, certain of our stockholders will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or our stockholders, subject to market standoff and lockup agreements. We also intend to register shares of common stock that we have issued and may issue under our employee equity incentive plans. Once we register these shares, they will be able to be sold freely in the public market upon issuance, subject to existing market standoff or lock-up agreements.

Goldman Sachs & Co. LLC, SVB Leerink LLC and Piper Sandler & Co. may, in their discretion, permit our stockholders to sell shares prior to the expiration of the restrictive provisions contained in those lock-up agreements.

The market price of the shares of our common stock could decline as a result of the sale of a substantial number of our shares of common stock in the public market or the perception in the market that the holders of a large number of shares intend to sell their shares.

If you purchase shares of our common stock in this offering, you will experience substantial and immediate dilution.

If you purchase shares of our common stock in this offering, you will experience substantial and immediate dilution in the pro forma net tangible book value per share of $12.96 per share as of December 31, 2020, based on the initial public offering price of $20.00 per share, because the price that you pay will be substantially greater than the pro forma net tangible book value per share of the common stock that you acquire. This dilution is due in large part to the fact that our earlier investors paid substantially less than the initial public offering price when they purchased their shares of our capital stock. You will experience additional dilution upon exercise of options to purchase common stock under our equity incentive plans, upon vesting of options to purchase common stock under our equity incentive plans, if we issue restricted stock to our employees under our equity incentive plans or if we otherwise issue additional shares of our common stock. See the section of this prospectus titled “Dilution” for additional information.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner, we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2021 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares of our common stock reserved for issuance under our 2021 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2022 through January 1, 2031, in an amount equal to the lesser of (i) 5% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of each automatic increase; or (ii) a lesser number of shares determined by our board of directors prior to the applicable January 1st. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

We are an “emerging growth company” and a “smaller reporting company”, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an “emerging growth company” as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

In addition, as an "emerging growth company" the JOBS Act allows us to delay adoption of new or revised accounting pronouncements applicable to public companies until such pronouncements are made applicable to private companies, unless we later irrevocably elect not to avail ourselves of this exemption. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (i) the last day of the fiscal year (1) following the fifth anniversary of the completion of this offering, (2) in which we have total annual gross revenue of at least $1.07 billion or (3) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We are also a smaller reporting company as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect at the completion of this offering could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Following the completion of this offering, our status as a Delaware corporation and the anti-takeover provisions of the Delaware General Corporation Law may discourage, delay or prevent a change in control by prohibiting us from engaging in a business combination with an interested stockholder for a period of three years after the person becomes an interested stockholder, even if a change of control would be beneficial to our existing stockholders. In addition, our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect at the completion of this offering will contain provisions that may make the acquisition of our company more difficult, including the following:

- a classified board of directors with three-year staggered terms, which could delay the ability of stockholders to change the membership of a majority of our board of directors;

- the ability of our board of directors to issue shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquirer;
- the exclusive right of our board of directors to elect a director to fill a vacancy created by the expansion of our board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- the requirement that a special meeting of stockholders may be called only by a majority vote of our entire board of directors, the chairman of our board of directors or our chief executive officer, which could delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors;
- the requirement for the affirmative vote of holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of the voting stock, voting together as a single class, to amend the provisions of our amended and restated certificate of incorporation relating to the management of our business or our amended and restated bylaws, which may inhibit the ability of an acquirer to affect such amendments to facilitate an unsolicited takeover attempt; and
- advance notice procedures with which stockholders must comply to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of us.

In addition, as a Delaware corporation, we are subject to Section 203 of the Delaware General Corporation Law. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

For information regarding these and other provisions, see the section of this prospectus titled "Description of Capital Stock."

Our amended and restated certificate of incorporation, which will become effective upon the closing of this offering designates the state courts the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, and the federal district courts of the United States of America will be the exclusive forums for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees.

Our amended and restated certificate of incorporation, which will become effective upon the closing of this offering, provides that, to the fullest extent permitted by law, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if and only

if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) and any appellate court therefrom shall will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees, governed by the internal affairs doctrine; provided, that, this provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction.

In addition, our amended and restated certificate of incorporation will provide that, to the fullest extent permitted by law, the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, unless we consent in writing to the selection of an alternative forum.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees and may discourage these types of lawsuits, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

Participation in this offering by our existing stockholders and/or their affiliated entities may reduce the public float for our common stock.

To the extent certain of our existing stockholders and their affiliated entities participate in this offering, such purchases would reduce the non-affiliate public float of our shares, meaning the number of shares of our common stock that are not held by officers, directors and controlling stockholders. A reduction in the public float could reduce the number of shares that are available to be traded at any given time, thereby adversely impacting the liquidity of our common stock and depressing the price at which you may be able to sell shares of common stock purchased in this offering.

General Risk Factors

We will incur significantly increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant expenses for director and officer insurance, legal services, accounting services and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently implemented by the SEC, and the

Nasdaq Global Market have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act (the Dodd-Frank Act) was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits smaller "emerging growth companies" to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costlier. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

Failure to build our finance infrastructure and improve our accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.

As a public company, we will operate in an increasingly demanding regulatory environment, which requires us to comply with the Sarbanes-Oxley Act, the regulations of the Nasdaq Global Market, the rules and regulations of the Securities and Exchange Commission, expanded disclosure requirements, accelerated reporting requirements and more complex accounting rules. Company responsibilities required by the Sarbanes-Oxley Act include establishing corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud. Commencing with our fiscal year ending the year after this offering is completed, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. Prior to this offering, we have never been required to test our internal controls within a specified period and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We anticipate that the process of building our accounting and financial functions and infrastructure will require significant additional professional fees, internal costs and management efforts. For example, we expect that we will need to implement new systems to enhance and streamline the management of our financial, accounting, human resources and other functions. However, such system will likely require us to complete many processes and procedures for the effective use of the system, which may result in substantial costs. Any disruptions or difficulties in implementing or using these systems could adversely affect our controls and harm our business. Moreover, such disruption or difficulties could result in unanticipated costs and diversion of management attention. In addition, we may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed, investors could lose confidence in our reported financial information and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon the completion of this offering, we will become subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

Future changes in financial accounting standards or practices may cause adverse and unexpected revenue fluctuations and adversely affect our reported results of operations.

Future changes in financial accounting standards may cause adverse, unexpected revenue fluctuations and affect our reported financial position or results of operations. Financial accounting standards in the United States are constantly under review and new pronouncements and varying interpretations of pronouncements have occurred with frequency in the past and are expected to occur again in the future. As a result, we may be required to make changes in our accounting policies. Those changes could affect our financial condition and results of operations or the way in which such financial condition and results of operations are reported. We intend to invest resources to comply with evolving standards, and this investment may result in increased general and administrative expenses and a diversion of management time and attention from business activities to compliance activities. See the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Recent Accounting Pronouncements.”

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, the Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. In

addition, it is uncertain if and to what extent various states will conform to the Tax Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As a result of the COVID-19 pandemic and actions taken to slow its spread, the global credit and financial markets have recently experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive an economic downturn, which could directly affect our ability to attain our operating goals on schedule and on budget.

We will have broad discretion in the use of the net proceeds of this offering and may not use them effectively or in ways that increase the value of our share price.

We cannot specify with any certainty the particular uses of the net proceeds that we will receive from this offering, but we currently expect such uses will include advancing our FA and DM1 programs into later-stage clinical trials, advancing our earlier stage GeneTAC programs into clinical development, supporting our ongoing drug discovery efforts and supporting our growing infrastructure and needs in operating as a public company. We will have broad discretion in the application of the net proceeds, including working capital and other general corporate purposes, and you and other stockholders may disagree with how we spend or invest these proceeds. The failure by our management to apply these funds effectively could adversely affect our business and financial condition. Pending their use, we may invest the net proceeds from our initial public offering in a manner that does not produce income or that loses value. These investments may not yield a favorable return to our investors.

Our internal information technology systems, or those of our third-party CROs or other contractors or consultants, may fail or suffer security breaches, loss or leakage of data, and other disruptions, which could result in a material disruption of our product candidates' development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. We also have outsourced elements of our operations to third parties, and as a result we manage a number of third-party contractors who have access to our confidential information.

Despite the implementation of security measures, given their size and complexity and the increasing amounts of confidential information that they maintain, our internal information technology systems and those of our third-party CROs and other contractors and consultants are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, natural disasters, terrorism, war and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, contractors, consultants, business partners, and/or other third parties, or from cyber-attacks by malicious third parties (including the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure or lead to data leakage. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and reputational damage and the further development and commercialization of our product candidates could be delayed.

We cannot assure you that our data protection efforts and our investment in information technology will prevent significant breakdowns, data leakages, breaches in our systems or other cyber incidents that could have a material adverse effect upon our reputation, business, operations or financial condition. For example, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs and the development of our product candidates could be delayed. In addition, the loss of clinical trial data for our product candidates could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. Furthermore, significant disruptions of our internal information technology systems or security breaches could result in the loss, misappropriation, and/or unauthorized access, use, or disclosure of, or the prevention of access to, confidential information (including trade secrets or other intellectual property, proprietary business information, and personal information), which could result in financial, legal, business, and reputational harm to us. For example, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our clinical trial subjects or employees, could harm our reputation directly, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business.

We or the third parties upon whom we depend may be adversely affected by earthquakes, fires or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our corporate headquarters and research facility are located in the county of San Diego, California, which in the past has experienced severe earthquakes and fires. If these earthquakes, fires, other natural disasters, terrorism and similar unforeseen events beyond our control prevented us from using all or a significant portion of our headquarters or research facility, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. We do not have a disaster recovery or business continuity plan in place and may incur substantial expenses as a result of the absence or limited nature of our internal or third-party service provider disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business. Furthermore, integral parties in our supply chain are operating from single sites, increasing their vulnerability to natural disasters or other sudden, unforeseen and severe adverse events. If such an event were to affect our supply chain, it could have a material adverse effect on our ability to conduct our clinical trials, our development plans and business.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations (collectively, Trade Laws), prohibit, among other things, companies and their employees, agents, CROs, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase over time. We expect to rely on third parties for research, preclinical studies, and clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other marketing approvals. We can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We, and the third parties with whom we share our facilities, are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Each of our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Each of our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. We could be held liable for any resulting damages in the event of contamination or injury resulting from the use of hazardous materials by us or the third parties with whom we share our facilities, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research and development. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no or only very few securities analysts commence coverage of us, or if industry analysts cease coverage of us, the trading price for our

common stock would be negatively affected. If one or more of the analysts who cover us downgrade our common stock or publish inaccurate or unfavorable research about our business, our common stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our common stock price and trading volume to decline.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements about us and our industry that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this prospectus, including statements regarding our strategy, future financial condition, future operations, research and development, planned clinical trials and preclinical studies, the timing and likelihood of regulatory filings and approvals for our product candidates, our ability to commercialize our product candidates, the potential benefits of collaborations, projected costs, prospects, plans, objectives of management and expected market growth, are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “design,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “positioned,” “potential,” “predict,” “seek,” “should,” “target,” “will,” “would” and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology.

We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our financial condition, results of operations, business strategy and financial needs. These forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions described in the sections of this prospectus titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this prospectus. Other sections of this prospectus may include additional factors that could harm our business and financial performance. In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

Moreover, we operate in a very competitive and rapidly changing environment. New risk factors emerge from time to time, and it is not possible for our management to predict all risk factors nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in, or implied by, any forward-looking statements.

In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur at all. You should refer to the section of this prospectus titled “Risk Factors” for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise. The Private Securities Litigation Reform Act of 1995 and Section 27A of the Securities Act, do not protect any forward-looking statements that we make in connection with this offering.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

MARKET AND INDUSTRY DATA

Certain market, industry and competitive data included in this prospectus were obtained from our own internal estimates and research, as well as from publicly available information, reports of governmental agencies and industry publications and surveys. In some cases, we do not expressly refer to the sources from which this data is derived. All of the market and industry data used in this prospectus is inherently subject to uncertainties and involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such information. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section of this prospectus titled “Risk Factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

USE OF PROCEEDS

We estimate that we will receive net proceeds of approximately $220.3 million (or approximately $253.8 million if the underwriters' option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering, based on the initial public offering price of $20.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

The principal purposes of this offering are to obtain additional capital to support our operations, to create a public market for our common stock and to facilitate our future access to the public equity markets. We anticipate that we will use the net proceeds of this offering, along with our existing cash, cash equivalents and investment securities, as follows:

- approximately $30.0 million to fund development of our FA program through the completion of IND-enabling studies and a Phase 1 clinical trial;
- approximately $35.0 million to fund development of our DM1 program through the completion of IND-enabling studies and a Phase 1 clinical trial;
- approximately $35.0 million to fund development of an additional undisclosed program through product candidate identification and IND-enabling studies; and
- the remaining proceeds to fund our other research and development programs and for general corporate purposes, which we expect will include the hiring of additional personnel, capital expenditures and the costs of operating as a public company.

We may also use a portion of the net proceeds from this offering to in-license, acquire, or invest in complementary businesses, technologies, products or assets. However, we have no current commitments or obligations to do so.

Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investment securities as of the date of this prospectus, together with the estimated net proceeds from this offering, will be sufficient to fund our anticipated operating expenses and capital expenditure requirements for at least the next 48 months following the date of this prospectus.

These are our first development programs and it is difficult to predict with certainty the cost and timing required to complete our development programs due to, among other factors, our lack of experience as a company with initiating and conducting preclinical studies and clinical trials, the rate of subject enrollment in our clinical trials, filing requirements with various regulatory agencies, preclinical and clinical results, and the actual costs of manufacturing our product candidates. The proceeds from this offering will not be sufficient to fund development of our product candidates through regulatory approval and commercialization. To obtain the capital necessary to fund our product candidates through regulatory approval and commercialization we may need to enter into additional public or private equity offerings, debt financings, or other capital sources which may include strategic collaborations, licensing arrangements, or other arrangements with third parties.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering, or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of the net proceeds will vary depending on numerous factors, including the progress, cost and results of our preclinical and clinical development programs, our ability to obtain additional financing, whether we are able to enter into future licensing or collaboration arrangements and other factors described in the section of this prospectus titled "Risk Factors," as well as the amount

of cash used in our operations and any unforeseen cash needs. We may find it necessary or advisable to use the net proceeds for other purposes, and our management will have broad discretion in the application of the net proceeds, and investors will be relying on our judgment regarding the application of the net proceeds from this offering.

Pending their use, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock, and we do not currently intend to pay any cash dividends on our capital stock in the foreseeable future. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business. Any future determination related to dividend policy will be made at the discretion of our board of directors, subject to applicable laws, and will depend upon, among other factors, our results of operations, financial condition, contractual restrictions and capital requirements.

In addition, our ability to pay cash dividends on our capital stock in the future may be limited by the terms of any future debt or preferred securities we issue or any credit facilities we enter into.

CAPITALIZATION

The following table sets forth our cash, cash equivalents and investment securities and our capitalization as of December 31, 2020:

- on an actual basis;
- on a pro forma basis to give effect to (i) the conversion of all outstanding shares of our convertible preferred stock as of December 31, 2020 into an aggregate of 13,504,598 shares of our common stock immediately prior to the closing of this offering, (ii) the issuance and sale of shares of our Series B convertible preferred stock in January 2021 for aggregate net proceeds of approximately $124.7 million and the subsequent conversion into 11,707,950 shares of our common stock, which will occur immediately prior to the closing of the offering, and (iii) the filing and effectiveness of our amended and restated certificate of incorporation immediately prior to the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our sale of 12,000,000 shares of our common stock in this offering at the initial public offering price of $20.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

The following table should be read together with the sections of this prospectus titled “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” and our financial statements and the related notes included elsewhere in this prospectus.

	As of December 31, 2020		
	Actual	Pro Forma	Pro Forma As Adjusted
	(unaudited) (in thousands, except share and per share amounts)		
Cash, cash equivalents and investment securities	$ 36,091	$160,808	$ 381,113
Convertible preferred stock, $0.0001 par value; 22,500,000 shares authorized, 22,012,499 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	$ 45,356	$ —	$ —
Stockholders’ (deficit) equity:			
Preferred stock, $0.0001 par value per share; no shares authorized, issued and outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted			
Common stock, $0.0001 par value; 60,000,000 shares authorized, 16,604,774 shares issued and outstanding(1), actual; 200,000,000 shares authorized, 41,817,322 shares issued and outstanding(1), pro forma; 200,000,000 shares authorized, 53,817,322 shares issued and outstanding(1), pro forma as adjusted	1	4	5
Additional paid-in capital	451	170,521	390,820
Accumulated other comprehensive income	156	156	156
Accumulated deficit	(11,923)	(11,923)	(11,923)
Total stockholders’ (deficit) equity	(11,315)	158,758	379,058
Total capitalization	$ 34,041	$158,758	$ 379,058

(1) The number of shares of common stock issued and outstanding includes 646,953 shares outstanding that are subject to forfeiture or our right to repurchase as of December 31, 2020 and which are therefore not considered outstanding for accounting purposes.

The number of shares of our common stock to be outstanding after this offering is based on 41,817,322 shares of common stock outstanding as of December 31, 2020 after giving effect to the pro forma adjustments described above (after giving effect to the conversion of 22,012,499 shares of convertible preferred stock outstanding as of December 31, 2020, as well as the issuance and subsequent conversion of 19,083,979 shares of Series B convertible preferred stock issued and sold in January 2021, into an aggregate of 25,212,548 shares of our common stock immediately prior to the completion of this offering; and which includes 646,953 shares outstanding that are subject to forfeiture or our right to repurchase as of such date), and excludes:

- 1,601,214 shares of our common stock issuable upon the exercise of outstanding stock options as of December 31, 2020, with a weighted-average exercise price of $0.91 per share;
- 328,217 shares of our common stock issuable upon the exercise of outstanding stock options granted subsequent to December 31, 2020, with a weighted-average exercise price of $7.71 per share;
- 7,000,000 shares of common stock reserved for future issuance under the 2021 Plan, as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the 2021 Plan, which became effective upon the execution and delivery of the underwriting agreement for this offering (including 881,352 shares of common stock reserved for issuance under the 2018 Plan, which shares were added to the 2021 Plan upon its effectiveness); and
- 600,000 shares of common stock reserved for future issuance under the ESPP, as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the ESPP, which became effective upon the execution and delivery of the underwriting agreement for this offering.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book deficit as of December 31, 2020 was $(11.5) million, or $(0.69) per share of our common stock. Our historical net tangible book deficit is the amount of our total tangible assets less deferred offering costs, our total liabilities and our convertible preferred stock, which is not included within stockholders' equity. Historical net tangible book deficit per share represents our historical net tangible book deficit divided by the number of shares of our common stock outstanding as of December 31, 2020 (including 646,953 shares subject to forfeiture or our right to repurchase).

Our pro forma net tangible book value as of December 31, 2020 was $158.6 million, or $3.79 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to (i) the conversion of all outstanding shares of our convertible preferred stock as of December 31, 2020 into an aggregate of 13,504,598 shares of our common stock immediately prior to the closing of this offering and (ii) the issuance and sale of shares of our Series B convertible preferred stock in January 2021 for aggregate net proceeds of approximately $124.7 million and the subsequent conversion into 11,707,950 shares of our common stock immediately prior to the closing of this offering. Pro forma net tangible book value per share represents our pro forma net tangible book value divided by the number of shares of our common stock outstanding as of December 31, 2020, after giving effect to the pro forma adjustments described above.

After giving further effect to our issuance and sale of 12,000,000 shares of our common stock in this offering at the initial public offering price of $20.00 per share and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of December 31, 2020 would have been $379.1 million, or $7.04 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $3.25 to existing stockholders and immediate dilution of $12.96 in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering.

Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Initial public offering price per share		$20.00
Historical net tangible book deficit per share as of December 31, 2020	$(0.69)	
Increase per share attributable to the pro forma effects described above	4.48	
Pro forma net tangible book value per share as of December 31, 2020	3.79	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing shares in this offering	3.25	
Pro forma as adjusted net tangible book value per share after this offering		7.04
Dilution per share to new investors purchasing shares in this offering		$12.96

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be $7.42 per share, representing an immediate increase in pro forma as adjusted net tangible book value per share of $3.63 to existing

stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $12.58 to new investors purchasing common stock in this offering, based on the initial public offering price of $20.00 per share, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. If any shares are issued upon exercise of outstanding options, you will experience further dilution.

The following table summarizes, on the pro forma as adjusted basis described above, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors purchasing shares of common stock in this offering. The calculation below is based on the initial public offering price of $20.00 per share, before deducting the underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Weighted-Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders before this offering	41,817,322	77.7%	$170,146,317	41.5%	$ 4.07
Investors purchasing shares in this offering	12,000,000	22.3	240,000,000	58.5	$ 20.00
Total	53,817,322	100.0%	$410,146,317	100.0%	

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to 75.2% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors participating in the offering would be increased to 24.8% of the total number of shares of our common stock outstanding after this offering.

The foregoing tables and calculations are based on 41,817,322 shares of our common stock outstanding as of December 31, 2020 after giving effect to the pro forma adjustments described above (after giving effect to the conversion of all of our shares of convertible preferred stock outstanding as of December 31, 2020, as well as the issuance and subsequent conversion of all of our shares of Series B convertible preferred stock issued and sold in January 2021 into an aggregate of 11,707,950 shares of our common stock immediately prior to the completion of this offering; and which includes 646,953 shares outstanding that are subject to forfeiture or our right to repurchase as of such date, and which are therefore not considered outstanding for accounting purposes), and excludes:

- 1,601,214 shares of our common stock issuable upon the exercise of outstanding stock options as of December 31, 2020, with a weighted-average exercise price of $0.91 per share;
- 328,217 shares of our common stock issuable upon the exercise of outstanding stock options granted subsequent to December 31, 2020, with a weighted-average exercise price of $7.71 per share;
- 7,000,000 shares of common stock reserved for future issuance under the 2021 Plan, as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the 2021 Plan, which became effective upon the execution and delivery of the underwriting agreement for this offering (including 881,352 shares of common stock reserved for issuance under the 2018 Plan, which shares were added to the 2021 Plan upon its effectiveness); and
- 600,000 shares of common stock reserved for future issuance under the ESPP, as well as any automatic increases in the number of shares of our common stock reserved for future issuance under the ESPP, which became effective upon the execution and delivery of the underwriting agreement for this offering.

To the extent that any outstanding options are exercised, or new options or other equity awards are issued under our equity incentive plans, you will experience further dilution. In addition, to the extent that additional capital is raised through the sale of equity or convertible debt securities in the future, the issuance of these securities may result in further dilution to our stockholders.

SELECTED FINANCIAL DATA

The following tables set forth our selected financial data as of, and for the periods ended on, the dates indicated. We have derived the selected statements of operations data for the years ended December 31, 2019 and 2020 and the selected balance sheet data as of December 31, 2019 and 2020 from our audited financial statements included elsewhere in this prospectus. You should read the following selected financial data together with our financial statements and the related notes included elsewhere in this prospectus and in the section of this prospectus titled "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of results that should be expected in any future period.

	Year Ended December 31,	
	2019	2020
	(in thousands, except share and per share amounts)	
Statements of Operations Data:		
Revenue:		
Grant revenue.	$ 834	$ 226
Operating expenses:		
Research and development	1,654	6,060
General and administrative	1,088	2,496
Total operating expenses	2,742	8,556
Loss from operations	(1,908)	(8,330)
Other (expense) income, net	(139)	50
Net loss	$ (2,047)	$ (8,280)
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (0.13)	$ (0.52)
Weighted-average shares of common stock used in computing net loss per share attributable to common stockholders, basic and diluted(1)	15,475,415	15,796,674
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(2)		$ (0.30)
Pro forma-weighted average shares of common stock used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(2)		27,193,045

(1) See Note 3 to our audited financial statements included elsewhere in this prospectus for details on the calculation of our basic and diluted net loss per share attributable to common stockholders and our basic and diluted pro forma net loss per share attributable to common stockholders, and the weighted-average number of shares used in computing the per share amounts.

(2) See the subsection titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Unaudited Pro Forma Information" for an explanation of the calculations of our basic and diluted pro forma net loss per share, and the weighted-average number of shares outstanding used in the computation of the per share amounts.

	As of December 31,	
	2019	2020
	(in thousands)	
Balance Sheet Data:		
Cash, cash equivalents and investment securities	$ 77	$ 36,091
Working capital(1)	(3,641)	33,903
Total assets	90	36,516
Total liabilities	3,732	2,475
Convertible preferred stock	—	45,356
Total stockholders' deficit	(3,642)	(11,315)

(1) Working capital is defined as current assets less current liabilities. See our financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the section of this prospectus titled "Selected Financial Data" and our financial statements and related notes included elsewhere in this prospectus. Some of the information contained in this discussion and analysis are set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the section of this prospectus titled "Risk Factors", our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. You should carefully read the section of this prospectus titled "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section of this prospectus titled "Special Note Regarding Forward-Looking Statements."

Overview

We are a preclinical-stage biopharmaceutical company pioneering novel small-molecule therapeutic candidates, called GeneTACs, that are designed to be disease-modifying and target the underlying cause of inherited nucleotide repeat expansion diseases. Certain nucleotide repeat expansion diseases, such as FA, can result in reduced expression of specific mRNAs; in other diseases, such as DM1, FECD, and Huntington disease, the nucleotide repeat expansions result in the generation of toxic gene products, often associated with pathological nuclear foci. Our GeneTACs are designed to selectively bind to genetic repeat sequences, modulate gene expression either by restoring or blocking mRNA transcription, and restore cellular health. As a platform, we believe that GeneTACs have broad potential applicability across monogenic nucleotide repeat expansion diseases.

In preclinical studies for our lead program, we have observed restoration of FXN levels in cells from FA patients using our FA GeneTACs. FA GeneTACs administered to various species, at doses that were observed to be well tolerated, achieved biodistribution to brain and heart, the key organs affected by FA, at concentrations that were consistent with those observed to restore FXN levels in FA patient cells. Further, and consistent with this good biodistribution, we observed increased FXN expression in the brain and heart in an animal model of FA after treatment with our FA GeneTACs. We plan to initiate clinical trials with our lead product candidate in FA patients to evaluate its safety, PK and effect on FXN levels by the first half of 2022, subject to receiving regulatory clearance to proceed into clinical trials. In our second GeneTAC program in DM1, we observed reduced nuclear foci in DM1 patient muscle cells after administration of our DM1 GeneTACs. We expect to seek regulatory clearance for clinical trials in 2023. We are also advancing our GeneTAC portfolio in preclinical studies to address other serious nucleotide repeat expansion-driven monogenic diseases, and intend to declare an additional product candidate in 2023.

We were incorporated in December 2017. To date, we have devoted substantially all of our resources to organizing and staffing our company, business planning, raising capital, developing and optimizing our technology platform, identifying potential product candidates, undertaking research and preclinical studies, engaging in manufacturing for our development programs, and providing general and administrative support for these operations. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the sale of our convertible preferred stock, grant revenue and the issuance of convertible notes and debt.

From inception to December 31, 2020, we raised aggregate net proceeds of approximately $45.2 million from the issuance of shares of our convertible preferred stock and convertible notes, including $0.2 million of convertible notes issued for services rendered. Our cash, cash equivalents and investment securities as of December 31, 2020, was $36.1 million. In January 2021, we issued 19,083,979 shares of our Series B convertible preferred stock at $6.55 per share for net proceeds of approximately $124.7 million.

We have incurred net losses and negative cash flows from operations since our inception. Our net loss for the year ended December 31, 2020 was $8.3 million and as of December 31, 2020, we had an accumulated deficit of $11.9 million. Our net losses and cash flows from operations may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of preclinical studies and our expenditures on other research and development activities. We expect our expenses and operating losses will increase substantially for the foreseeable future as we conduct preclinical studies and clinical trials for our product candidates, nominate additional product candidates from our discovery programs, and as we expand our clinical, regulatory, quality and manufacturing capabilities, incur significant commercialization expenses for marketing, sales, manufacturing and distribution, if we obtain marketing approval for any of our product candidates, and incur additional costs associated with operating as a public company.

Moreover, we do not expect to generate any revenues from product sales unless and until we successfully complete development and obtain regulatory approval for one or more product candidates, which will not be for many years, if ever. Accordingly, until such time as we can generate significant revenue from sales of our product candidates, if ever, we expect to finance our cash needs through public and private equity offerings, debt financings, or other capital sources which may include strategic collaborations, licensing arrangements or other arrangements with third parties. However, we may be unable to raise additional funds or enter into such other arrangements when needed, on favorable terms, or at all. Further, if we raise funds through strategic collaborations or other similar arrangements with third parties, we may have to relinquish valuable rights to our platform technology, future revenue streams, research programs or product candidates or may have to grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. Our failure to raise capital or enter into such other arrangements when needed would have a negative impact on our financial condition and could force us to delay, reduce or terminate our research and development programs or other operations, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and disruptions to and volatility in the credit and financial markets in the U.S. and worldwide resulting from the ongoing COVID-19 pandemic or otherwise. Because of the numerous risks and uncertainties associated with drug development, we cannot predict the timing or amount of increased expenses and cannot assure you that we will ever be profitable or generate positive cash flow from operating activities. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investment securities as of the date of this prospectus, together with the estimated net proceeds from this offering, will be sufficient to fund our anticipated operating expenses and capital expenditure requirements for at least the next 48 months following the date of this prospectus.

We do not own or operate manufacturing facilities, nor do we require complex customized manufacturing equipment and processes equipment. We currently rely on third-party manufacturers and suppliers for the polyamides, ligands and linkers used to make our GeneTACs, and we expect to continue to do so to meet our research, preclinical, clinical and commercial activities. Our third-party manufacturers are required to manufacture our product candidates under cGMP requirements and other applicable laws and regulations. We believe there are multiple sources for all of the raw materials required for the manufacture of our product candidates.

The global COVID-19 pandemic continues to rapidly evolve, and we will continue to monitor the COVID-19 situation closely. The extent of the impact of the COVID-19 on our business, operations and clinical development timelines and plans remains uncertain, and will depend on certain developments, including the duration and spread of the outbreak and its impact on our CROs, third-party manufacturers, and other third parties with whom we do business, as well as its impact on regulatory authorities and our key scientific and management personnel. The ultimate impact of the COVID-19 pandemic or a similar health epidemic is highly uncertain and subject to change. To the extent possible, we are conducting business as usual, with necessary or advisable modifications to employee travel and most of our office employees working remotely. We will continue to actively monitor the rapidly evolving situation related to COVID-19 and may take further actions that alter our operations, including those that may be required by federal, state or local authorities, or that we determine are in the best interests of our employees and other third parties with whom we do business. At this point, the extent to which the COVID-19 pandemic may affect our business, operations and clinical development timelines and plans, including the resulting impact on our expenditures and capital needs, remains uncertain and is subject to change.

Components of Our Results of Operations

Grant Revenue

To date, we have not generated any revenues from the commercial sale of any products, and we do not expect to generate revenues from the commercial sale of any products for the foreseeable future, if ever. For the years ended December 31, 2019 and 2020, we derived revenue from grants awarded by the National Institutes of Health, the National Science Foundation and the Friedreich's Ataxia Research Alliance. These grants provide us with funding for certain research and development activities on a best-efforts basis and do not require scientific achievement as a performance obligation for certain allowable costs for funded projects. We recognize revenue from these grant awards in the period during which the related qualifying services are rendered and costs are incurred, relative to the estimated total effort or costs be incurred under the grant. As of December 31, 2020, we have been awarded a total of $1.0 million in research grants, all of which has been recognized as revenue as of December 31, 2020.

Operating Expenses

Our operating expenses consist of (i) research and development expenses and (ii) general and administrative expenses.

Research and Development

To date, our research and development expenses have consisted primarily of direct and indirect costs incurred in connection with our discovery efforts, and the preclinical and formulation development of our product candidates. In the future, we expect a substantial portion of our research and development expenses will relate to the clinical development and manufacturing of our product candidates. Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods or services are received.

Direct costs include:

- external research and development expenses incurred under agreements with contract research organizations, consultants and other vendors that conduct our preclinical and discovery activities;

- expenses related to manufacturing our product candidates for preclinical studies;
- laboratory supplies; and
- license fees.

Indirect costs include:

- personnel-related expenses, consisting of employee salaries, payroll taxes, bonuses, benefits and stock-based compensation charges for those individuals involved in research and development efforts; and
- facilities and other indirect expenses.

A significant portion of our research and development expenses have been direct costs, which we track by stage of development, preclinical or clinical. However, we do not track our internal research and development expenses on a program specific basis, unless specific to research grants, because these costs are deployed across multiple projects and, as such, are not separately classified.

We expect that our research and development expenses will substantially increase for the foreseeable future as we continue the development of our FA program, DM1 program and our other discovery programs, in particular as we advance our product candidates into clinical development. As of the date of this prospectus, we cannot reasonably determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical programs of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Preclinical and clinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments as to each product candidate's commercial potential. We will need to raise substantial additional capital in the future. In addition, we cannot forecast which product candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements.

Our future research and development expenses may vary significantly based on a wide variety of factors such as:

- the number and scope, rate of progress, expense and results of our discovery and preclinical development activities;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the phase of development of the product candidate;

- the efficacy and safety profile of the product candidate;
- the timing, receipt, and terms of any approvals from applicable regulatory authorities including the FDA and non-U.S. regulators;
- maintaining a continued acceptable safety profile of our product candidates following approval, if any, of our product candidates;
- establishing clinical and commercial manufacturing capabilities or making arrangements with third-party manufacturers in order to ensure that we or our third-party manufacturers are able to make product successfully;
- significant and changing government regulation and regulatory guidance;
- the impact of any business interruptions to our operations or to those of the third parties with whom we work, particularly in light of the current COVID-19 pandemic environment; and
- the extent to which we establish additional strategic collaborations or other arrangements.

A change in the outcome of any of these variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate.

The process of conducting the necessary preclinical and clinical research to obtain regulatory approval is costly and time-consuming. The actual probability of success for our product candidates or any future candidates may be affected by a variety of factors. We may never succeed in achieving regulatory approval for any of our product candidates or any future candidates. Further, a number of factors, including those outside of our control, could adversely impact the timing and duration of our product candidates' or any future candidates' development, which could increase our research and development expenses.

General and Administrative

General and administrative expenses consist primarily of personnel-related expenses, including employee salaries, bonuses, benefits, and stock-based compensation charges, for personnel in executive and administrative functions. Other significant general and administrative expenses include legal fees relating to intellectual property and corporate matters, professional fees for accounting, tax and consulting services and insurance costs.

We anticipate that our general and administrative expenses will substantially increase in the foreseeable future as we add general and administrative personnel to support our expanded research and development activities and infrastructure and, if any of our product candidates or any future candidates receive marketing approval, commercialization activities, as well as to support our operations generally, including facility-related expenses and patent-related costs. We also expect to incur increased expenses related to accounting, audit, legal, regulatory and tax-related services, director and officer insurance premiums, board of director fees, investor and public relations, and other costs associated with operating as a public company.

Other Income (Expense), Net

Other income (expense), net consist primarily of interest income from our cash, cash equivalents and investment securities and interest expense incurred on our convertible notes and notes payable. Additionally, it includes the charges we record related to the fair value of the conversion feature on our convertible notes. The fair value of the conversion feature liability was determined based on a pricing model that incorporated the actual conversion price determined upon the initial closing of our Series A

convertible preferred stock financing in February 2020, whereby all our outstanding convertible notes and related accrued interest converted into an aggregate 301,685 shares of our Series A convertible preferred stock.

Results of Operations

Comparison of the Years Ended December 31, 2019 and 2020

The following table summarizes our results of operations for the years ended December 31, 2019 and 2020 (in thousands):

	Year Ended December 31,		
	2019	2020	Change
Revenue:			
Grant revenue	$ 834	$ 226	$ (608)
Operating expenses:			
Research and development	1,654	6,060	4,406
General and administrative	1,088	2,496	1,408
Total operating expenses	2,742	8,556	5,814
Loss from operations	(1,908)	(8,330)	(6,422)
Other (expense) income, net	(139)	50	189
Net loss	$(2,047)	$(8,280)	$(6,233)

Grant Revenue

During 2019 and 2020, we were awarded an aggregate of $1.0 million in research grants from the National Science Foundation, the National Institutes of Health and the Friedreich's Ataxia Research Alliance for research related to FA and for the development of a genomic targeting drug delivery platform. Grant revenue recognized for the years ended December 31, 2019 and 2020, based on efforts expended and costs incurred, was $0.8 million and $0.2 million, respectfully.

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2019 and 2020 (in thousands):

	Year Ended December 31,		
	2019	2020	Change
Direct costs(1)	$1,481	$4,161	$2,680
Indirect costs	173	1,899	1,726
Total research and development expenses	$1,654	$6,060	$4,406

(1) In future periods when clinical trial expenses are incurred, external costs will be broken out between our clinical programs and our preclinical programs.

Research and development expenses were $1.7 million and $6.1 million for the years ended December 21, 2019 and 2020, respectively. The increase of $4.4 million was due primarily to additional spend to support the advancement of our FA program in 2020, including pre-clinical studies, chemistry and manufacturing development costs. Further, we incurred higher personnel-related costs in 2020 as compared to 2019, as we expanded the number of research and development employees to support our programs, including an additional $0.2 million of non-cash stock-based compensation costs.

General and Administrative Expenses

General and administrative expenses was $1.1 million and $2.5 million for the years ended December 31, 2019 and 2020, respectfully. The increase of $1.4 million was due primarily to additional personnel-related costs in 2020 as compared to 2019, as we expanded the number of general and administrative employees to support our organization, including an additional $0.3 million of non-cash stock-based compensation costs. Further, we incurred increased professional fees for legal and accounting services in 2020 as compared to 2019.

Other Income (Expense), Net

Other expense, net, was $0.1 million for the years ended December 31, 2019 as compared to other income, net of $0.1 million for the years ended December 31, 2020, respectively. Other expense in 2019, consisted primarily of interest expense related to borrowings pursuant to our convertible notes and notes payable and the change in fair value of the conversion feature of our convertible notes. Other income in 2020 consisted of interest earned on our investment securities, partially offset by interest expense related to borrowings pursuant to our convertible notes and notes payable and the change in fair value of the conversion feature of our convertible notes.

Unaudited Pro Forma Information

Immediately prior to the completion of this offering, all outstanding shares of our convertible preferred stock will convert into shares of our common stock. The unaudited pro forma basic and diluted net loss per share for the year ended December 31, 2020 was computed using the weighted-average number of shares of common stock outstanding, including the pro forma effect of the conversion of all outstanding shares of convertible preferred stock into shares of common stock, as if such conversion had occurred at the beginning of the period, or their issuance dates if later. There were no shares of convertible preferred stock outstanding at December 31, 2019. Pro forma net loss per share does not include the shares expected to be sold in this offering.

The following table sets forth the computation of the unaudited pro forma basic and diluted net loss per share of common stock for the periods presented (in thousands, except share and per share amounts):

	Year Ended December 31, 2020
Numerator:	
Net loss per share attributable to common stockholders, basic and diluted	$ (0.52)
Denominator:	
Weighted-average common shares outstanding	15,796,674
Weighted-average convertible preferred stock	11,396,371
Pro forma weighted-average shares outstanding, basic and diluted	27,193,045
Pro forma net loss per share, basic and diluted	$ (0.30)

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have funded our operations primarily through the sale of our convertible preferred stock, grant income and the issuance of convertible notes and notes payable. We have

devoted substantially all of our resources organizing and staffing our company, business planning, raising capital, developing and optimizing our technology platform, identifying potential product candidates, undertaking research and preclinical studies, engaging in manufacturing for our development programs, and providing general and administrative support for these operations. From inception to December 31, 2020, we have raised aggregate net proceeds of approximately $45.2 million from the issuance of shares of our convertible preferred stock and convertible notes, including $0.2 million of convertible notes issued for services rendered. We have incurred net losses and negative cash flows from operations since our inception and anticipate we will continue to incur net losses for the foreseeable future. We do not have any products approved for sale and have not generated any revenue from product sales or otherwise. As of December 31, 2020, we had cash, cash equivalents and investment securities of $36.1 million. In January 2021, we issued 19,083,979 shares of our Series B convertible preferred stock at $6.55 per share for net proceeds of approximately $124.7 million.

Cash Flows

The following table sets forth a summary of the net cash flow activities for the years ended December 31, 2019 and 2020 (in thousands):

	Year Ended December 31,		
	2019	2020	Change
Net cash (used in) provided by:			
Operating activities	$ (139)	$ (8,669)	$ (8,530)
Investing activities	—	(33,561)	(33,561)
Financing activities	196	44,532	44,336
Net increase in cash and cash equivalents	$ 57	$ 2,302	$ 2,245

Operating Activities

Net cash used in operating activities was $0.1 million and $8.7 million for the years ended December 31, 2019 and 2020, respectively. The net cash used in operating activities in 2019 was primarily due to our net loss of $2.0 million, which was mostly offset by an increase in accounts payable and accrued liabilities of $1.6 million. In 2020, the net cash used in operating activities was primarily due to our net loss of $8.3 million and changes in our working capital, partially offset by non-cash stock-based compensation of $0.5 million.

Investing Activities

Net cash used in investing activities was $33.6 million for the year ended December 31, 2020, due primarily to the purchase of $55.6 million of available-for-sale investment securities, partially offset by the maturity of $22.1 million of such investments. These funds were invested in available-for-sale investment securities in accordance with our investment policy. We had no such investing activities in 2019.

Financing Activities

Net cash provided by financing activities was $44.5 million for the year ended December 31, 2020, primarily from the $44.7 million of net proceeds we received from the issuance of 21,710,814 shares Series A convertible preferred stock at $2.0727 per share. During 2019, net cash provided by financing activities was $0.2 million from the issuances of notes payable. The notes payable were repaid in full during 2020.

Funding Requirements

Based on our current operating plan, we believe that our existing cash, cash equivalents and investment securities as of the date of this prospectus, without taking into consideration the estimated net proceeds from this offering, will be sufficient to fund our anticipated operating expenses and capital expenditure requirements for at least the next 12 months following the date of this prospectus. However, based upon our current operating plan, we estimate that our existing cash, cash equivalents and investment securities as of the date of this prospectus, together with the estimated net proceeds from this offering, will be sufficient to fund our anticipated operating expenses and capital expenditure requirements for at least the next 48 months following the date of this prospectus. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. We have based this estimate on assumptions that may prove to be wrong, and we could expend our capital resources sooner than we expect. Additionally, the process of testing product candidates in clinical trials is costly, and the timing of progress and expenses in these trials is uncertain.

Our future capital requirements will depend on many factors, including:

- the scope, rate of progress and costs of our drug discovery, preclinical development activities and clinical trials for any future product candidates;
- the number and scope of clinical programs we decide to pursue;
- the scope and costs of manufacturing our future product candidates and commercial manufacturing activities;
- the emergence of competing therapies and other adverse market developments;
- the cost, timing and outcome of seeking FDA, EMA and any other regulatory approvals for any future product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the terms and timing of establishing and maintaining strategic collaborations, licenses and other similar arrangements and the financial terms of such agreements;
- our efforts to enhance operational systems and our ability to attract, hire and retain qualified personnel, including personnel to support the development of our product candidates;
- the costs associated with being a public company;
- the timing of any milestone and royalty payments to Wisconsin Alumni Research Foundation, or other future licensors;
- the extent to which we acquire or in-license other product candidates and technologies;
- our need and ability to retain key management and hire scientific, technical, business, and medical personnel;
- our implementation of additional internal systems and infrastructure, including operational, financial and management information systems;
- or costs associated with expanding our facilities or building out our laboratory space;
- the effects of the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide from the COVID-19 pandemic; and
- the cost associated with commercialization activities for any our future product candidates, if approved.

Until such time, if ever, as we can generate substantial revenues from product sales to support our cost structure, we expect to finance our cash needs through public or private equity offerings, debt financings, or other capital sources which may include strategic collaborations, licensing arrangements or other arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Equity and debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through strategic collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic or otherwise. Because of the numerous risks and uncertainties associated with product development, we cannot predict the timing or amount of increased expenses and cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Contractual Obligations and Commitments

Under our license agreement with the Wisconsin Alumni Research Foundation (WARF), we have payment obligations that are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones, and are required to make development milestone payments and royalty payments in connection with sublicensing revenue and the sale of products developed under that agreement. As of December 31, 2020, we are unable to estimate the timing or likelihood of achieving the milestones or making future product sales. For additional information regarding the WARF license agreement, including our payment obligations thereunder, see the section of this prospectus titled “Business—License Agreement with Wisconsin Alumni Research Foundation” and Note 9 to our financial statements included elsewhere in this prospectus.

In May 2019, we entered into an agreement to lease laboratory and office space in La Jolla, California pursuant to a three-month, automatically renewing lease. As of December 31, 2020, we occupied approximately 700 square feet of space pursuant to the lease and our lease payment amount was $24,000 per month, subject to annual rent increases of 3%. In January 2021, we secured an additional approximately 170 square feet of space pursuant to the lease increasing our current lease payment is $26,000 per month. In March 2021, we further amended the lease to secure an additional 680 square feet of space and, effective April 15, 2021, we will be subject to monthly rent payments of $45,000. We also have access to approximately 2,120 square feet of additional office space on an as-available basis from time to time pursuant to our agreement with Marlinspike Group, LLC (Marlinspike).

In February 2021, we entered into a lease agreement to rent approximately 12,370 square feet of lab and office space. The anticipated delivery date of the space is September 1, 2021, and the lease is expected to commence at that time. The term of the lease is 72 months after commencement with an option to extend the lease term for a period of three years. Annual rent payments will be $0.8 million per year, subject to annual increases of 3%, plus our share of operating expenses and taxes.

Pursuant to our consulting agreement with Marlinspike, if we unilaterally terminate the consulting agreement for any reason other than for cause, we would be subject to a $240,000 termination fee. Currently we cannot determine when, or if, such a termination will occur.

In operating our business, we also enter into contracts and agreements that require capital resources. For example, we enter into contracts in the normal course of business with vendors for preclinical studies, research supplies, manufacturing development activities and other services and products for operating purposes. These contracts generally provide for termination after a notice period. We also enter into unconditional purchase obligations with various vendors and suppliers of goods and services in the normal course of business through purchase orders or other documentation, or that are undocumented except for an invoice. As these obligations are generally outstanding for periods less than a year and are settled by cash payments upon delivery of the goods or services, they are not considered long-term contractual obligations and, therefore, are cancelable contracts.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles (GAAP). The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to grant revenue and research and development expenses. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies and estimates are described in more detail in Note 2 to our financial statements included elsewhere in this prospectus, we believe the following accounting policies and estimates to be most critical to the preparation of our financial statements.

Revenue Recognition

We have been awarded research grants from the National Science Foundation, the National Institutes of Health, and the Friedreich's Ataxia Research Alliance. We recognize grant revenue by measuring the progress of the applicable research and development services provided over time, based on the effort we expend and costs incurred, relative to the estimated total effort and costs to be incurred under the grant. This approach requires us to use judgement and make estimates of future expenditures. If our estimates or judgements change over the course of the term of the grant, it may affect the timing and amount of revenue that it recognizes in the current and future periods.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. We make estimates of our accrued expenses as of each balance sheet date based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses include the costs incurred for services performed by our vendors in connection with research and development activities for which we have not yet been invoiced. Since our inception, we have not experienced any material differences between accrued or prepaid costs and actual costs.

We base our expenses related to research and development activities on our estimates of the services received and efforts expended pursuant to quotes and contracts with vendors that conduct research and development on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid expense accordingly. Advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

Stock-Based Compensation

Stock-based compensation expense represents the grant date fair value of equity awards recognized in the period using the Black-Scholes option pricing model. We recognize the expense for equity awards on a straight-line basis over the requisite service periods of the awards, which is usually the vesting period. Forfeitures are recognized as they occur.

Estimating the fair value of equity awards pursuant to the Black-Scholes option pricing model requires us to make assumptions regarding a number of variables, including the risk-free interest rate, the expected stock price volatility, the expected term of stock options, the expected dividend yield and the fair value of the underlying common stock on the date of grant. Changes in these assumptions can materially affect the fair value and ultimately how much stock-based compensation expense is recognized.

The Black-Scholes option pricing model utilizes inputs which are highly subjective assumptions and generally require significant judgment. We determine these assumptions in the following manner:

- ***Fair Value of Common Stock.*** See the subsection titled "—Common Stock Valuations" below.
- ***Expected Term.*** The expected term of stock options represents the period of time that the awards are expected to be outstanding. Because we do not have sufficient historical exercise behavior, we determine the expected term assumption using the simplified method for our employees and board members, which calculates the expected term as the average time-to-vesting and the contractual life of the award. The expected term for non-employees is generally the contractual term.
- ***Expected Volatility.*** As we are not yet a public company and do not have a trading history for our common stock, the expected volatility assumption was determined by examining the historical volatilities of a group of industry peers whose share prices are publicly available.
- ***Risk-Free Interest Rate.*** The risk-free rate assumption is based on the U.S. Treasury yield in effect at the time of the grant with maturities consistent with the expected term of the awards.
- ***Expected Dividend Yield.*** The expected dividend yield assumption is based on our history and expectation of dividend payouts. The Company has not paid and does not intend to pay dividends and, therefore, used an expected dividend yield of zero.

See Note 2 to our financial statements included elsewhere in this prospectus for more information concerning certain of the specific assumptions we used in applying the Black-Scholes option pricing model to determine the estimated fair value of our stock options. Certain of such assumptions involve inherent uncertainties and the application of significant judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation could be materially different.

We recorded an immaterial amount of stock-based compensation expense for the year ended December 31, 2019, and $0.5 million for the year ended December 31, 2020. As of December 31, 2020, there was $7.3 million of total unrecognized stock-based compensation expense related to unvested stock options which we expect to recognize over a remaining weighted-average period of 3.5 years. We expect to continue to grant stock options and other equity-based awards in the future, and to the extent that we do, our stock-based compensation expense recognized in future periods will likely increase.

The intrinsic value of all outstanding options as of March 22, 2021 was $30.6 million based on the initial public offering price of $20.00 per share, of which approximately $0.2 million was related to vested options and approximately $30.4 million was related to unvested options.

Common Stock Valuations

Historically, for all periods prior to this offering, since there has been no public market of our common stock to date, the fair value of the shares of common stock underlying our share-based awards was estimated on each grant date by our board of directors. To determine the fair value of our common stock underlying option grants, our board of directors considered, among other things, input from management, valuations of our common stock prepared by unrelated third-party valuation firms in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation* (the Practice Aid), and our board of directors' assessment of additional objective and subjective factors that it believed were relevant, and factors that may have changed from the date of the most recent valuation through the date of the grant. These factors include, but are not limited to:

- our results of operations and financial position, including our levels of available capital resources;
- our stage of development and material risks related to our business;
- progress of our research and development activities;
- our business conditions and projections;
- the lack of marketability of our common stock and our preferred stock as a private company;
- the prices at which we sold shares of our redeemable convertible preferred stock to outside investors in arms-length transactions;
- the rights, preferences, and privileges of our redeemable convertible preferred stock relative to those of our common stock;
- the analysis of initial public offerings (IPOs) and the market performance of similar companies in the biopharmaceutical industry;
- the likelihood of achieving a liquidity event for our securityholders, such as an initial public offering or a sale of our company, given prevailing market conditions;
- the hiring of key personnel and the experience of management;
- trends and developments in our industry; and
- external market conditions affecting the life sciences and biotechnology industry sectors.

The Practice Aid prescribes several valuation approaches for setting the value of an enterprise, such as the cost, income and market approaches, and various methodologies for allocating the value

of an enterprise to its common stock. The cost approach establishes the value of an enterprise based on the cost of reproducing or replacing the property less depreciation and functional or economic obsolescence, if present. The income approach establishes the value of an enterprise based on the present value of future cash flows that are reasonably reflective of our future operations, discounting to the present value with an appropriate risk adjusted discount rate or capitalization rate. The market approach is based on the assumption that the value of an asset is equal to the value of a substitute asset with the same characteristics. Each valuation methodology was considered in our valuations.

The various methods for allocating the enterprise value across our classes and series of capital stock to determine the fair value of our common stock in accordance with the Practice Aid include the following:

- ***Current Value Method.*** Under the current value method, once the fair value of the enterprise is established, the value is allocated to the various series of preferred and common stock based on their respective seniority, liquidation preferences or conversion values, whichever is greatest.
- ***Option Pricing Method (OPM).*** Under the OPM, shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The values of the preferred and common stock are inferred by analyzing these options.
- ***Probability-Weighted Expected Return Method (PWERM).*** The PWERM is a scenario-based analysis that estimates the value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class.

For our valuations performed through 2019, we determined the use of the OPM on the underlying assets of the company was the most appropriate method for determining the fair value of our common stock based on our early-stage of development and other relevant factors.

For our valuation performed in January 2020, we used the hybrid method of OPM and PWERM to estimate the fair value of our common stock. We considered various scenarios, including two outcomes for the completion of a Series A preferred stock financing and no financing.

For our valuation performed in March 2020, we estimated the fair value of our common stock using the market approach, specifically a backsolve to the recently completed Series A preferred stock financing.

For our valuation performed as of December 31, 2020, we used the PWERM method to estimate the fair value of our common stock. We considered various scenarios, including an IPO with and without the sale of Series B preferred stock, a stay-private scenario, and a dissolution scenario. Under the stay private scenario, the enterprise value was determined at the valuation date using a combination of the market approach and a backsolve to the Series B preferred stock financing. We used the OPM to allocate value in the stay-private and dissolution scenarios. The relative probabilities between the future exit scenarios were determined by our board of directors based on an analysis of performance and market conditions at the time, including then current IPO valuations of similarly situated companies and expectations as to the timing and likely prospects of future event scenarios. Upon the completion of the Series B preferred stock financing in January 2021, we updated the valuation using the PWERM method to include the Series B preferred stock financing with an IPO scenario, a stay private scenario and a dissolution scenario.

Retrospective Reassessment of Fair Value of Common Stock for Financial Reporting Purposes

As part of the preparation of the financial statements necessary for inclusion in this prospectus, we reassessed for financial reporting purposes, on a retrospective basis, the fair value of our common stock for each stock option granted in 2020. For purposes of this reassessment, we evaluated our original inputs and the methodologies used to determine our enterprise value, the methods we used to allocate enterprise value and the timing of those valuations. In February 2020, we granted common stock options based on the valuation we performed in January 2020. Common stock options granted from March 2020 to July 2020 were based on the valuation we performed in March 2020. In our assessment of these grants, we did not identify any significant internal or external value-generating events between the valuation dates and the grant dates, and concluded that the inputs and methodologies were appropriate and that the fair value per common share at each of the grant dates was equal to the fair value of our common stock.

Additional common stock options were granted from October 2020 through December 2020 based on the valuation we performed in March 2020. Although we did not identify any specific internal or external value-generating events between March 2020 and the dates of these grant dates, on a retrospective basis and in light of our recent IPO organizational meeting and the valuation performed as of December 31, 2020, we concluded that the fair value per share of our common stock for financial reporting purposes was $6.19 per share, equal to the valuation we determined for our common stock as of December 31, 2020. We applied this reassessed value to grants awarded since August 2020 using the Black-Scholes option pricing model to determine the fair value of these grants.

The following table summarizes by grant date the number of shares of common stock options granted from August 1, 2020 through December 31, 2020, the associated per share exercise price and the reassessed per share fair value of our common stock for financial reporting purposes on the applicable grant date:

Grant Date	Number of Common Shares Underlying Options Granted	Exercise Price Per Common Share	Reassessed Fair Value Per Common Share	Intrinsic Value Per Common Share
October 29, 2020	1,027,604	$ 0.95	$ 6.19	$ 5.24
November 13, 2020	30,674	$ 0.95	$ 6.19	$ 5.24
December 7, 2020	306,744	$ 0.95	$ 6.19	$ 5.24

We utilized the above reassessed fair values to determine the stock-based compensation expense, which is recorded in our financial statements.

The assumptions underlying these valuations represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could have been materially different. Following the completion of this offering, the fair value of our common stock will be based on the closing price as reported on the date of grant on the primary stock exchange on which our common stock is traded.

Recent Accounting Pronouncements

See Note 2 to our financial statements included elsewhere in this prospectus for information about recent accounting pronouncements, the timing of their adoption, and our assessment, if any, of their potential impact on our financial condition and results of operations**.**

Off-Balance Sheet Arrangements

During the periods presented we did not have, nor do we currently have, any off-balance sheet arrangements as defined under SEC rules.

Quantitative and Qualitative Disclosures About Market Risk

Interest Rate Risk

Our cash, cash equivalents and investment securities balance as of December 31, 2020 consisted of cash in readily available checking accounts, money market accounts, U.S. Treasury bills and certificates of deposit insured by the Federal Deposit Insurance Corporation (FDIC). Some of the financial instruments that we invest in could be subject to market risk, meaning that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the prevailing interest rate later rises, the value of that security will probably decline. With the goal of minimizing this risk, we intend to maintain a portfolio which may include a variety of securities, all with various maturity dates. Based on our current investment portfolio, we do not believe that a hypothetical 100 basis point increase or decrease in interest rates during any of the periods presented would have had a material impact on our financial statements included elsewhere in this prospectus.

As of December 31, 2020, we had no outstanding debt and therefore were not exposed to related interest rate risk.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and research and development contract costs. We do not believe inflation has had a material effect on our financial statements included elsewhere in this prospectus.

Emerging Growth Company and Smaller Reporting Company Status

We are an emerging growth company, as defined in the JOBS Act, and we may remain an emerging growth company for up to five years following the completion of this offering. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation-related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will remain an emerging growth company until the earliest to occur of: (i) the last day of the fiscal year in which we have at least $1.07 billion in annual revenue; (ii) the last day of the fiscal year in which we are deemed to be a “large accelerated filer,” as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded $700.0 million as of the last business day of the second fiscal quarter of such year; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt securities during the prior three-year period; and (iv) the last day of the fiscal year ending after the fifth anniversary of this offering.

We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the aggregate amount of gross proceeds to us as a result of this offering is less than $700.0 million and our annual revenue was less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250.0 million; or (ii) our annual revenue was less than $100.0 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

BUSINESS

Overview

We are a preclinical-stage biopharmaceutical company pioneering novel small-molecule therapeutic candidates, called gene targeted chimeras (GeneTACs), that are designed to be disease-modifying and target the underlying cause of inherited nucleotide repeat expansion diseases. Certain nucleotide repeat expansion diseases, such as Friedreich ataxia (FA), can result in reduced expression of specific mRNAs; in other diseases, such as myotonic dystrophy type-1 (DM1), Fuchs endothelial corneal dystrophy (FECD), and Huntington disease, the nucleotide repeat expansions result in the generation of toxic gene products, often associated with pathological nuclear foci. Our GeneTACs are designed to selectively bind to genetic repeat sequences, modulate gene expression either by restoring or blocking transcription, and restore cellular health. As a platform, we believe that GeneTACs have broad potential applicability across monogenic nucleotide repeat expansion diseases.

In preclinical studies for our lead program, we have observed restoration of frataxin (FXN) levels in cells from FA patients using our FA GeneTACs. FA GeneTACs administered to various species, at doses that were observed to be well tolerated, achieved biodistribution to brain and heart, key organs affected by FA, at concentrations that were consistent with those observed to restore FXN levels in FA patient cells. Further, and consistent with this good biodistribution, we observed increased FXN expression in the brain and heart in an animal model of FA after treatment with our FA GeneTACs. We plan to initiate clinical trials with our lead product candidate in FA patients to evaluate its safety, pharmacokinetics (PK) and effect on FXN levels by the first half of 2022, subject to receiving regulatory clearance to proceed into clinical trials.

In our second GeneTAC program in DM1, we observed reduced nuclear foci in DM1 patient muscle cells after administration of our DM1 GeneTACs. We expect to seek regulatory clearance for clinical trials in 2023. We are also advancing our GeneTAC portfolio in preclinical studies to address other serious nucleotide repeat expansion-driven monogenic diseases, and intend to declare an additional product candidate in 2023.

Other genomic therapeutics, including oligonucleotides, mRNA, gene therapy and gene editing, have disease-modifying potential but may have modality-associated limitations related to administration, biodistribution and potential safety concerns. In contrast, we believe the structure and mechanism of action of our GeneTACs may offer the disease-modifying potential of genomic therapeutics, while also offering broad tissue biodistribution, resolution of aberrant gene expression preserving endogenous regulatory control elements, and leveraging established manufacturing, regulatory, and distribution frameworks for small molecules.

Our GeneTAC Platform

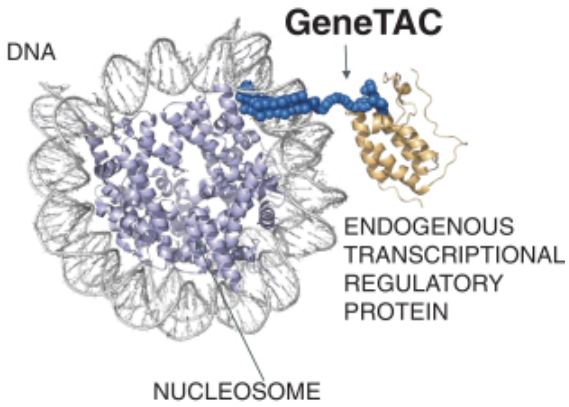
We utilize our proprietary GeneTAC platform to design and develop therapeutic candidates for inherited diseases driven by nucleotide repeat expansion. Individuals with nucleotide repeat expansion diseases are born with abnormally expanded stretches of specific nucleotide sequences, often with hundreds to thousands of excess repeats present in the mutant gene. Higher number of excess repeats can lead to more severe, and sometimes a more rapidly progressive form of disease. Nucleotide repeat expansion has been identified as the underlying cause of more than 40 debilitating degenerative diseases impacting millions of people. Currently, there are no approved therapeutic options that address the cause of any nucleotide repeat expansion diseases.

RNA is generated based on a DNA sequence of a gene through a process called transcription. The RNA is, in turn, used as a template to make the proteins that control cellular functions in a process

called translation. Combined, transcription and translation are responsible for gene expression. Individuals who have a nucleotide repeat expansion in the DNA can experience different alterations of transcription. In some diseases such as FA, the transcription machinery stalls at the abnormally expanded repeat sequence leading to insufficient production of a critical protein called FXN. In other cases, such as in DM1, the abnormal RNA transcript arising from the nucleotide repeat expansion mutation is misprocessed, leading to a cascade of downstream toxicity and cellular dysfunction.

GeneTACs represent a novel class of small molecules designed to act on a diverse array of diseases. We have developed a proprietary framework that combines our understanding of medicinal chemistry and structure-activity relationships that allow us to design targeted DNA-binding moieties that are connected via a linker to ligand moieties that engage and modulate the transcriptional machinery. GeneTAC molecules are heterobifunctional, meaning that they are comprised of two principal moieties that are each designed to have a unique function:

- ***DNA-Binding Moiety:*** one end of the GeneTAC molecule is a DNA minor groove binder that has been designed to recognize and bind to the specific nucleotide repeat sequence of interest (e.g. the repeated GAA sequence seen in the first intron of the FXN gene seen in FA or the CTG repeat in the 3' non-coding region of the dystrophy myotonic protein kinase (DMPK) gene seen in DM1).
- ***Ligand Moiety:*** the other end of the molecule is designed to interact with the endogenous proteins that can regulate transcription.



The structures of the GeneTAC molecules are designed to enable them to act specifically at the site of the disease-causing nucleotide repeat expansion by targeting the mutant allele and modulating the transcriptional machinery in a cell. Consequently, the cell can resume gene expression and production of normal protein isoforms that remain under normal physiological control. The versatility of the GeneTAC platform allows us to design GeneTAC molecules toward a specific nucleotide repeat expansion target, regardless of repeat number, and tailor it to address the underlying disease-specific dysfunction in gene regulation in one of the following ways:

- ***Restoration of Transcription:*** In diseases where the expanded nucleotide repeat structure can cause endogenous transcription machinery to stall, which leads to an insufficient amount of protein production, GeneTAC molecules can be designed to bind at the desired loci in the genome and engage the endogenous transcriptional machinery with the goal of restoring normal levels of full-length pre-mRNAs. In FA, for example, where the expanded triplet repeat occurs in an intron, a non-coding region of the gene, the abnormally long nucleotide sequence is spliced out of the pre-mRNA thus enabling normal production of natural protein isoforms according to existing physiologic regulatory control.

- ***Reduction of Toxic Gene Product Levels:*** Another type of nucleotide repeat expansion disease occurs when the transcription process results in the accumulation of toxic gene products (e.g. DM1, Huntington disease, FECD), and in some cases the formation of nuclear foci, leading to multiple downstream cellular dysfunctions. In these cases, a single copy of expanded repeat containing allele is sufficient to cause the disease. Our GeneTAC molecules are designed to selectively target the abnormally expanded nucleotide repeat to block the formation of the downstream toxic gene product and restore cellular function without interfering with the gene expression of the normal allele.

Our understanding of the properties of the GeneTAC molecules is based on data-driven assessments of compounds we have designed and synthesized, as well as experience with our most advanced compounds for FA tested *in vitro* and *in vivo*. We continue to develop know-how of permutations of binder, linker, and ligand moieties that drive the drug properties of molecules which are best suited to be developed for treating the underlying cause of each specific disease. This understanding of GeneTAC chemistry has enabled us to generate multiple candidates designed to have optimal potential therapeutic and drug characteristics.

Our Programs

We are developing a portfolio of GeneTAC product candidates designed to address genetic diseases driven by inherited nucleotide repeat expansions that have urgent medical need and where no approved disease-modifying treatments are currently available. Because GeneTACs are designed to be a novel class of disease-modifying small molecule therapeutic candidates, we have selected disease programs where we believe the underlying cause is amenable to intervention using our technology and prioritized our development efforts where we believe there is a clear and efficient path to advance these candidates through clinical development, with the goal of providing a disease-modifying therapy for patients.

Our lead candidates and early development programs are summarized in the table below:

Figure 1: GeneTAC Pipeline

PROGRAMS (Targeted nucleotide expansion)	NEXT ANTICIPATED MILESTONE	ANTICIPATED MILESTONE DATE
Friedreich ataxia (GAA)	Obtain regulatory clearance and initiate first-in-human clinical trials	1H 2022
Myotonic dystrophy (CTG)	Obtain regulatory clearance and initiate first-in-human clinical trials	2023

* *To date, we have not submitted an investigational new drug application (IND) with the U.S. Food and Drug Administration (FDA) or any similar submission in any foreign jurisdiction with respect to either of our lead candidate programs.*

FA Program Overview

Our FA program is focused on the development of a potentially disease-modifying treatment. FA is a devastating monogenic, autosomal recessive progressive disease where over 95% of cases are

caused by homozygous guanine-adenine-adenine (GAA) triplet repeat expansions in the first intron of the FXN gene, which encodes the mitochondrial protein FXN. The disease is characterized by spinocerebellar ataxia, dysarthria, pyramidal weakness, deep sensory loss, hypertrophic cardiomyopathy, skeletal abnormalities, and diabetes mellitus. Clinical onset occurs most often around puberty, leads to severe disability by early adulthood, with substantial functional loss, wheelchair dependence, and loss of quality of life. Affected individuals have reduced life expectancy, with many premature deaths caused by complications of the cardiomyopathy at about the end of the fourth decade of life.

The estimated prevalence of FA is 1 in 40,000-50,000, affecting more than 5,000 individuals living in the United States and more than 20,000 in Europe. Our FA GeneTAC candidate is designed to address the genetic basis of the disease by restoring functional FXN protein levels and, subject to receiving regulatory clearance to proceed into clinical trials, we anticipate a first-in-human dosing for our first product candidate in the first half of 2022. The primary cause of mortality (approximately 60% of FA patients) is cardiac arrhythmias or heart failure with the mean life expectancy reduced to approximately 35-40 years.

DM1 Program Overview

Our DM1 program is focused on the development of a potentially disease-modifying treatment for DM1. DM1 is a monogenic, autosomal dominant, progressive neuromuscular disease that affects skeletal muscle, heart, brain, and other organs. The cardinal features include muscle weakness, myotonia (slow muscle relaxation), and early cataracts. In addition, affected individuals often experience cardiac arrhythmias and changes in neuropsychological function. DM1 is caused by a mutation in the DMPK gene and is estimated to have a genetic prevalence of 1 in 2,300-8,000 people, affecting more than 70,000 people in the United States and more than 90,000 people in Europe. Our DM1 GeneTAC molecules are designed to address the genetic basis of the disease by preventing the expression of toxic gene product and consequently of nuclear foci. We expect to complete investigational new drug (IND)-enabling studies and seek regulatory clearance for a first-in-human clinical trial in 2023.

Research Program Overview

We are also advancing our GeneTAC product candidate portfolio into development in other serious nucleotide repeat expansion-driven monogenic diseases, such as FECD, Fragile X syndrome, spinocerebellar ataxias, amyotrophic lateral sclerosis, frontotemporal dementia, Huntington disease and spinobulbar muscular atrophy. Many of these monogenic diseases have overlapping triplet repeat expansions, including CTG repeats that cause DM1, allowing for the potential for a single GeneTAC to be used across multiple diseases. Additionally, our experiences with GeneTACs allow us to more rapidly design GeneTACs for additional proposed indications.

Our Strategy

We aim to leverage our GeneTAC platform to design, develop and commercialize a pipeline of disease-modifying therapeutic candidates designed to treat a wide range of inherited nucleotide repeat expansion diseases for which there is urgent unmet medical need. In order to achieve our goal, we intend to:

- ***Advance our Lead Program in FA Through Clinical Development to Offer Meaningful Patient Benefit.*** FA is a serious monogenic degenerative disease for which there are currently no available treatments. Our FA GeneTACs are specifically designed to restore levels of FXN, the underlying cause of FA. Restoration of FXN has been shown to improve FA-like symptoms in animal models. We believe that demonstrating clinical proof of concept by restoring FXN expression in FA patients may confirm the therapeutic potential of our FA GeneTACs and

underscore the broader potential of our GeneTAC platform. We plan to conduct formal GLP safety studies in both rats and non-human primates and, subject to receiving regulatory clearance to proceed into clinical trials, anticipate beginning clinical trials in the first half of 2022.

- ***Advance our DM1 Program Through Clinical Development to Offer Meaningful Patient Benefit.*** DM1 is a serious monogenic degenerative disease for which there are currently no available treatments. Our DM1 GeneTACs are specifically designed to reduce the formation of CUG repeat hairpin structures that trap splicing factors and form toxic nuclear foci that cause DM1. Blocking the formation of CUG foci has demonstrated phenotypic benefit. We believe that demonstrating clinical proof of concept by reducing the repeat hairpin structures in DM1 patients may confirm the therapeutic potential of this candidate. We expect to complete IND-enabling studies and seek regulatory clearance for first-in-human clinical trial in 2023.
- ***Leverage our GeneTAC Platform to Expand our Pipeline and Address Additional Nucleotide Repeat Expansion Diseases with Significant Unmet Medical Need.*** There remains a significant unmet medical need for all nucleotide repeat expansion diseases. We plan to advance our GeneTAC portfolio to address the underlying cause of other serious nucleotide repeat expansion-driven monogenic diseases, which may include FECD, Fragile X syndrome, spinocerebellar ataxias, familial amyotrophic lateral sclerosis, frontotemporal dementia, Huntington disease and spinobulbar muscular atrophy, the majority of which have no approved disease-modifying treatments.
- ***Selectively Enter Into Strategic Collaborations to Realize the Full Potential of Our Platform.*** Given the broad potential of our GeneTAC platform, we may explore collaborations in select disease areas or geographic regions that are better served by the resources or specific expertise of a strategic partner to accelerate the development and commercialization of our GeneTAC product candidates.
- ***Independently Commercialize any Approved Products in Indications and Geographies Where we Believe we can Maximize Value.*** We intend to commercialize our product candidates in indications and geographies that we believe we can commercialize successfully on our own, if any of our candidates receives regulatory approval.
- ***Establish a Leadership Position in Genetic Diseases by Continuing to Build and Leverage our Relationships with the Key Opinion Leaders, Physicians, and Patients.*** We have an established advisory network of pharmaceutical research and development experts, scientists, clinicians and patient organizations in areas relevant to our programs. We have continued to grow our network as needed to inform our programs with the most up to date data and practices that might enhance our ability to effectively bring potentially life saving treatments to patients in need.

Our History, Team and Investors

Our company was created to design, develop and commercialize a novel class of small molecule therapeutic candidates (GeneTACs) designed to directly address the underlying basis of genetic disease. To achieve this goal, we have assembled a management team with extensive experience in the design, development and commercialization of drugs for serious diseases, including a seasoned research and development team comprised of 22 individuals (20 of which are full time employees), 11 of whom have Ph.D.s or M.D.s., as of March 22, 2021.

Our company was started by Pratik Shah, Ph.D. and Aseem Z. Ansari, Ph.D. Dr. Shah, our Co-Founder and Executive Chairperson, has more than 30 years of experience founding and leading biopharmaceutical companies and healthcare investment decisions. Dr. Ansari, our Co-Founder, is an internationally recognized pioneer in transcriptional regulation and DNA minor groove binders and the

chair of the Department of Chemical Biology and Therapeutics at St. Jude Children's Research Hospital. João Siffert, M.D., our President and Chief Executive Officer, has more than 20 years of leadership experience in biopharmaceutical companies and clinical medicine. Prior to Design, Dr. Siffert led a publicly traded biotech company developing gene and cell therapies for devastating degenerative diseases, and previously led research and development organizations in the United States and Europe, including programs that received regulatory approvals followed by commercial launches. Sean Jeffries, Ph.D., our Chief Operating Officer, brings over 20 years of experience in business development, portfolio management, and research and development strategy for both emerging and large biopharmaceuticals companies.

Since our inception, we have raised over $170.0 million in gross proceeds, including from a syndicate of leading life sciences investors that include, among others, Logos Capital, SR One Investments, Quan Capital, Cormorant Asset Management, and West River Capital.

Background on Genomic Medicines

What is Genetic Disease?

Genetic diseases arise when a change to the DNA, called a mutation, disrupts normal cellular functioning. These mutations can range from alteration of a single nucleotide in an individual's DNA to major abnormalities affecting many genes or even entire chromosomes. When a mutation occurs in a single gene, the disease is referred to as a monogenic disease. More than 10,000 monogenic diseases have been identified and many are serious conditions that collectively affect millions of people globally, most of which have no effective therapeutic options.

What is Genomic Medicine?

Genomic medicines are created based on understanding of genetic causes of disease, targeting specific defects at the genetic level with the potential to address the underlying cause of disease and restore cellular function.

Technical and scientific advances in genomics have identified possible genetic targets for therapeutic interventions. Several approaches have been developed to address diseases caused by genetic mutations, including oligonucleotides, mRNA, gene therapy and gene editing. While these technologies have led to numerous product candidates over the last decade, significant challenges have limited their utility in the clinic as a result of:

- immunogenicity that creates safety concerns and limits activity and re-dosing;
- unregulated gene expression;
- off-target effects;
- limitations of dose adjustments/silencing;
- limitations and heterogeneity of biodistribution; and
- challenges with consistency, quality and scalability of manufacturing.

Advantages of Our Platform

We are using our GeneTAC platform to develop small molecule genomic medicine candidates that are designed to offer precise modulation of gene transcription. We believe that the GeneTAC platform may offer several potential mechanistic and development advantages over other genomic medicine modalities, including:

- GeneTAC small molecules may be more tolerable over complex biologics because GeneTACs are less likely to cause adverse immune reactions;

- GeneTACs may be less likely to be immunogenic and therefore have no limitations with re-dosing;
- GeneTAC treatment is designed to be reversible;
- GeneTACs are designed to act on the transcription machinery of the cell and do not alter the genome;
- GeneTACs modulation of transcription is designed to preserve normal physiological post-transcriptional regulation and protein translation controls;
- GeneTAC structure is designed to enable therapeutically active molecules to be deployed directly at the site of disease-causing mutations, which could enhance specificity and potency, and minimize off-target effects;
- GeneTACs are designed to enable ongoing dose optimization;
- GeneTACs can achieve biodistribution across target organs and into the cell without specialized engineering or delivery technologies;
- GeneTACs are synthetically tractable, offering a potentially readily scalable, cost-effective development path that does not require complex customized manufacturing equipment and processes; and
- GeneTACs have a modular heterobifunctional structure that is intended to allow us to rationally design novel targeting components for specific DNA sequences, creating a potentially highly efficient discovery engine that could enable us to rapidly expand our portfolio into new disease areas.

By combining the disease-modifying potential of genomic medicines with the drug-like properties, manufacturing and logistics advantages of small molecules, we believe GeneTACs could be developed as novel therapeutic options in genetic diseases where disease-modifying treatments have previously been elusive.

Our Portfolio

Friedreich Ataxia

Disease Overview, Prevalence and Current Treatment Landscape

FA is a monogenic, autosomal recessive, progressive multi-system disease that affects organ systems highly dependent on mitochondrial function, eventually leading to neurological, cardiac, and metabolic dysfunction. Clinical manifestations include poor coordination of legs and arms, progressive loss of balance and ability to walk, generalized weakness, loss of sensation, scoliosis, hypertrophic cardiomyopathy and cardiac arrythmia, and glucose intolerance, including diabetes. FA patients also report impaired vision, hearing and speech. FA significantly impairs quality of life with loss of independence, physical limitations and reduced participation in social activities and work.

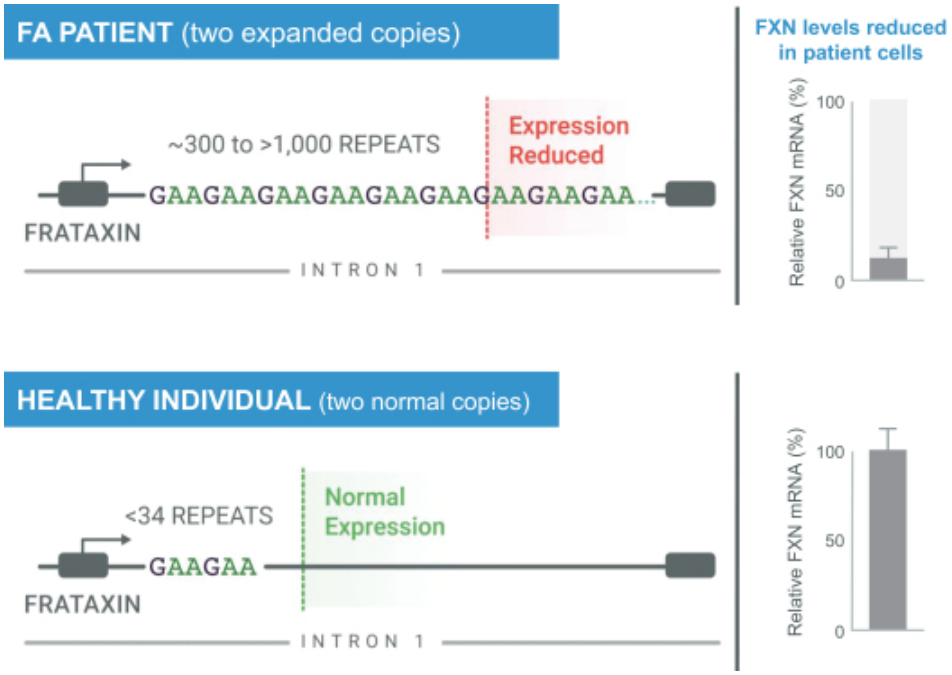
The primary cause of mortality (approximately 60% of FA patients) is cardiac arrhythmias or heart failure with the mean life expectancy reduced to approximately 35-40 years from approximately 78 years.

In over 95% of patients, FA is caused by an inherited homozygous increase in the number of GAA triplet repeats found in the first intron of the FXN gene. The number of repeats ranges from up to approximately 30 GAA repeats in healthy individuals to over several hundred to over 1,000 in FA patients. The expanded triplet repeat results in gene silencing and reduction in capacity to produce the FXN protein, which is required for proper functioning of the mitochondria and ultimately the entire cell. Levels of FXN correlate inversely with disease severity, and when levels are reduced to levels of approximately 25% of normal healthy individuals, iron homeostasis and iron-sulfur cluster synthesis are

impaired, leading to general impairment of normal mitochondrial function. Heterozygote carriers typically have approximately half of the FXN levels of normal individuals, but are asymptomatic and hence on average, the doubling of FXN protein levels in FA patients to achieve carrier levels or higher is expected to restore mitochondrial function and provide therapeutic benefit.

The genetic basis for FA is illustrated in Figure 2 below.

Figure 2: Genetic basis for FA



The clinical course of FA is progressive, with most patients (approximately 75%) presenting in their adolescent years with gait ataxia and scoliosis. About 10 years after disease onset, most patients lose their ability to walk and require a wheelchair because of progressive loss of balance and muscle weakness in the torso and legs. Eventually, muscle weakness in the tongue and throat makes it difficult to swallow and eat, and almost all patients experience some degree of dysarthria (slowing/slurring of speech), which limits communication. Approximately 50% of FA patients develop glucose intolerance and approximately 30% develop diabetes. More than two thirds of FA patients have cardiac abnormalities at baseline including arrhythmia, conduction abnormalities, or hypertrophic cardiomyopathy. Cardiac abnormalities are responsible for approximately 60% of mortality in FA patients. FA significantly reduces life expectancy and impairs quality of life for patients and their families with loss of independence, physical limitations and reduced participation in social activities and work.

The estimated prevalence of FA is 1 in 40,000-50,000, affecting more than 5,000 individuals living in the United States and more than 20,000 in Europe.

There are currently no approved therapies for the treatment of FA, and treatment is focused largely on symptom management. There are several product candidates in clinical development, but none of them have shown the ability to restore the deficiency in FXN protein, which is the underlying cause of the disease. There remains a high unmet medical need for new disease-modifying therapies.

Our Approach

Our FA program is based on GeneTAC small molecules consisting of a DNA-binding moiety designed to bind to the expanded GAA repeat sequence in the first intron of the FXN gene in FA patients, linked to a ligand moiety designed to recruit an endogenous transcriptional elongation complex to unblock the transcriptional machinery, and restore the production of functional natural FXN proteins to therapeutic levels.

The key advantages of our FA program include the following:

- ***Designed to Address the Underlying Cause of the Disease.*** FA is caused by reduced FXN protein levels. Our FA GeneTACs are designed to restore FXN levels, thereby restoring normal physiological activity.
- ***High Potency Compounds.*** Our FA GeneTACs have shown high potency and prolonged restoration of FXN levels in preclinical assays in FA patient cells.
- ***Designed for Efficient Delivery of Drug to the Target Organs.*** We have observed good biodistribution and bioavailability for FA GeneTACs in multiple animal species, where they reached therapeutically relevant concentrations in the most affected organs such as heart, brain, muscle and spinal cord.

Preclinical Data

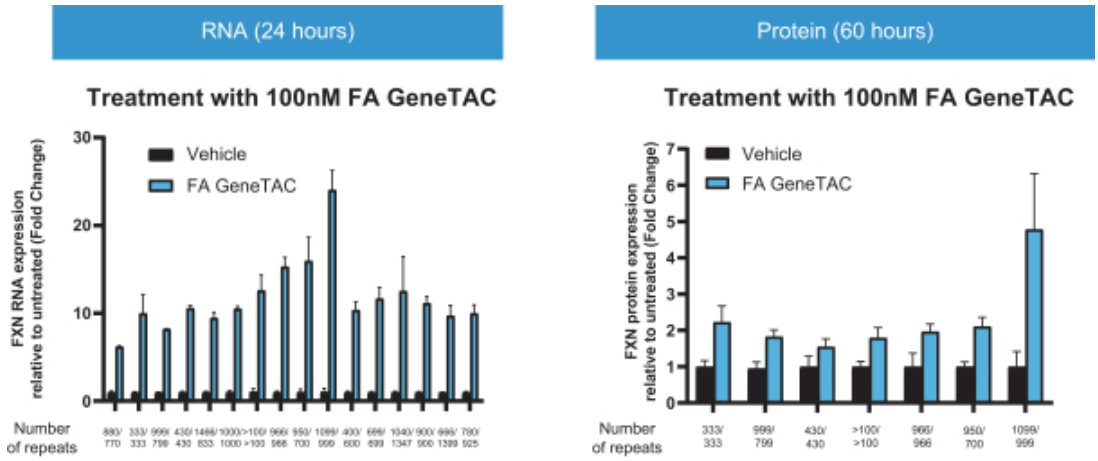
We believe that the results of our preclinical studies to date support the hypotheses that FA GeneTACs may confer a clinical benefit to FA patients. In *in vitro* experiments in primary cells from FA patients and in neurons and cardiomyocytes derived from FA patient stem cells, robust and durable increases in FXN mRNA and protein restoration was observed following exposure to FA GeneTACs, even at low nanomolar (nM) concentrations. In preclinical studies, FA GeneTACs achieved therapeutically relevant concentrations in key organs of disease, including the heart, brain, muscle and spinal cord, at doses that were well tolerated in multiple species.

We conducted preclinical studies in several models of disease. We used multiple types of FA patient cells, including white blood cells and cardiomyocytes and neurons derived from stem cells. We used the Pook800J mouse model, which contains a hemizygous insertion of the human disease allele with approximately 800 GAA repeats, in mice lacking endogenous mouse FXN.

Increase in FXN mRNA and Protein Levels in Patient Blood Cells

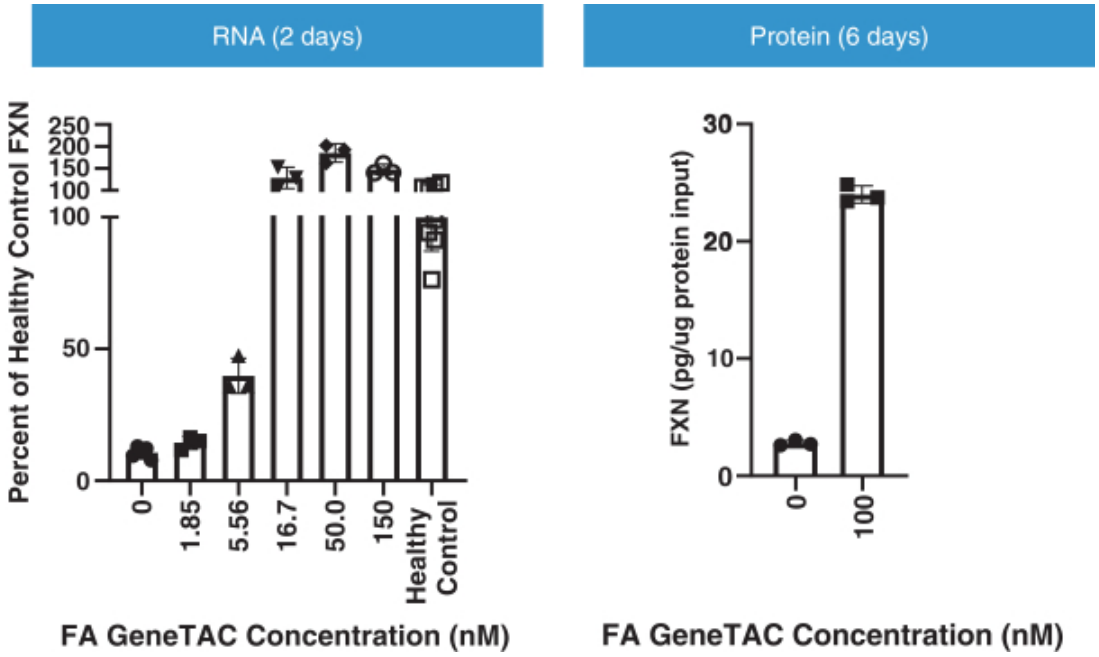
In preclinical studies in FA primary patient blood samples that have reduced FXN levels, we observed an increase in both FXN mRNA and protein levels after the administration of FA GeneTACs. As illustrated below in Figure 3, after a single 100nM administration of our FA GeneTAC, we observed increased FXN mRNA levels in all 10 patient samples with GAA repeat lengths ranging from approximately 100 to over 1,000. We also observed an increase in FXN protein levels in all 7 patient samples tested after a single administration of our FA GeneTAC. Sixty hours post this single administration of our FA GeneTAC, we observed a nearly two-fold increase in FXN protein levels compared to cells with vehicle control. Predictions from studies examining FXN levels in FA patients suggest that a two-fold increase in FXN levels would be highly therapeutic.

Figure 3: FXN mRNA and protein levels in primary patient blood samples



In preclinical studies in an FA patient lymphoblastoid white blood cell line, we observed that treatment with FA GeneTACs resulted in a dose dependent increase in FXN mRNA levels with an EC50 of 4nM and an EC90 of 9nM (Figure 4). Following a single dose of an FA GeneTAC, we observed increased FXN mRNA levels up to the levels observed in a healthy control with two normal FXN alleles. We also observed a multi-fold increase in FXN protein levels after continuous exposure to 100nM FA GeneTAC for six days.

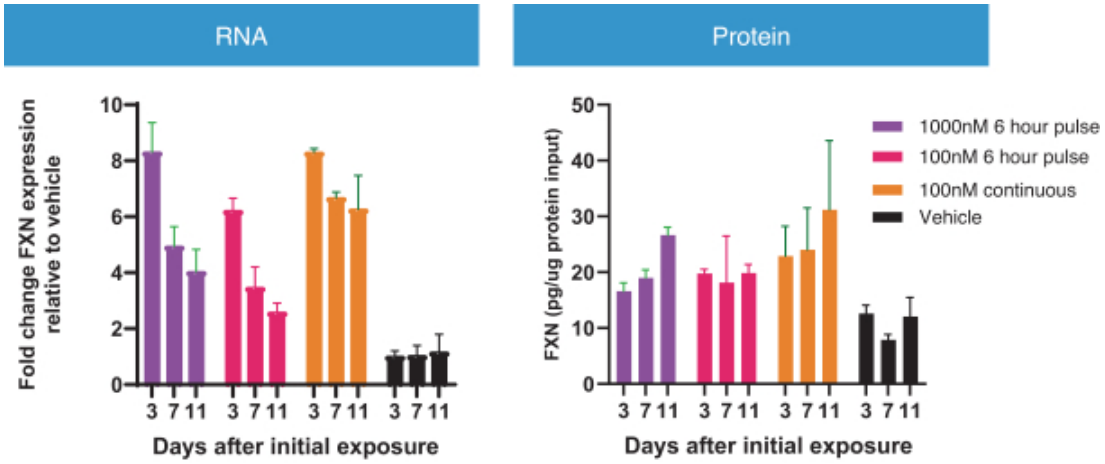
Figure 4: FXN mRNA and protein levels in patient lymphoblastoid cell line



Increase in FXN mRNA and Protein in FA Patient Cardiomyocytes and Neurons

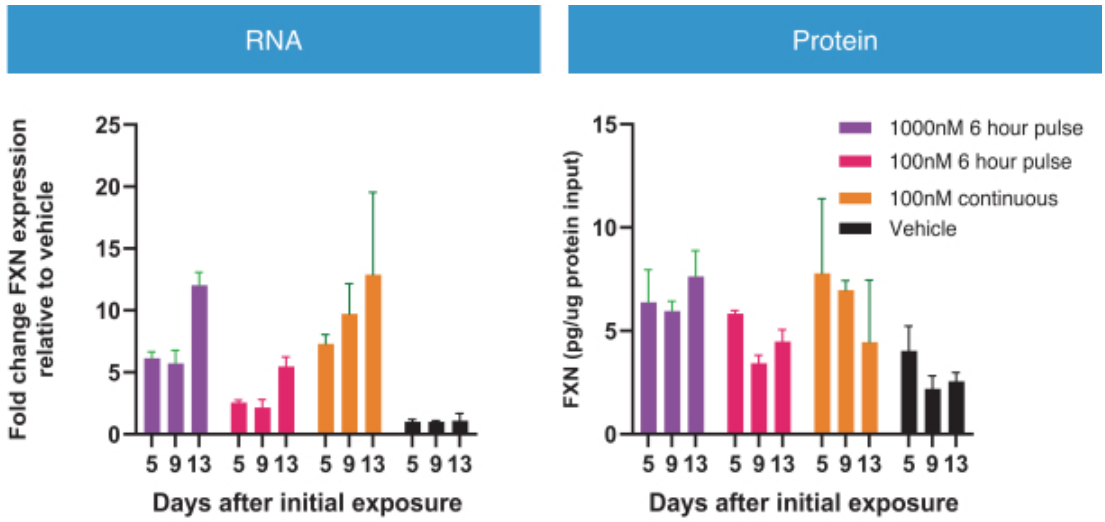
In preclinical studies in both cardiomyocytes and neurons derived from an FA patient stem cell line, we observed an increase in FXN mRNA and protein levels following exposure to our FA GeneTACs. As illustrated in Figure 5, in the study of cardiomyocytes derived from an FA patient stem cell line, increased mRNA and protein levels were observed after both single and continuous exposure to our FA GeneTAC. Importantly, the increase in FXN mRNA and protein levels occurred after both short exposure followed by washout (6-hour pulse) and continuous exposure of cells to our FA GeneTAC. In both cases, the increase in FXN was observed greater than 10 days after the initial exposure to our FA GeneTAC.

Figure 5: FXN mRNA and protein levels in patient cardiomyocytes



As illustrated in Figure 6, in this study in neurons derived from an FA patient stem cell line, increased mRNA and protein levels were shown after both single and continuous exposure to our FA GeneTAC. Importantly, the increase in FXN mRNA and protein levels occurred after both short exposure followed by washout (6-hour pulse) and continuous exposure of cells to our FA GeneTAC. In both cases, the increase in FXN was observed greater than 10 days after the initial exposure to our FA GeneTAC.

Figure 6: FXN mRNA and protein levels in FA patient neurons

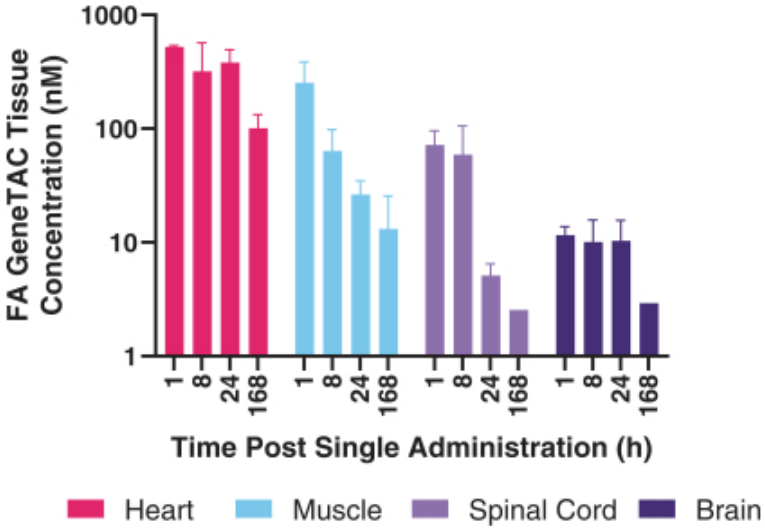


In preclinical studies in Pook800J mice, we observed an increase in FXN protein levels in both heart and brain tissue after treatment with FA GeneTACs.

Distribution and Tolerability of FA GeneTACs

In preclinical studies in Sprague-Dawley rats, after a single intravenous (IV) administration of our FA GeneTAC, we observed intact FA GeneTACs in target tissues (heart, brain, muscle and spinal cord) at or above concentrations shown to increase FXN mRNA and protein levels in FA patient cells. (Figure 7). In addition, FA GeneTACs were observed in target tissues one week after a single IV dose. Repeat administration at this dose level in Sprague-Dawley rats was found to be well tolerated and resulted in no clinically meaningful changes in hematology, serum biochemistry or histology.

Figure 7: FA GeneTAC concentration in target tissues of Sprague-Dawley rats



In our preclinical studies, we have observed a dose dependent increase in both FXN mRNA and protein levels in primary blood cells collected from FA patients, a dose dependent increase in both FXN mRNA and protein levels in neurons and cardiomyocytes and neurons derived from FA patients, durability of FXN mRNA and protein levels greater than 10 days, and sustained FA GeneTAC levels after a single dose in heart, brain, muscle and spinal cord in rats.

Based on these results, we plan to conduct formal GLP safety studies in both rats and non-human primates and anticipate beginning clinical trials in the first half of 2022, subject to receiving regulatory clearance to proceed into clinical trials. We are also developing an additional structurally distinct development candidate that has shown an EC50 of 30nM and an EC90 of 62nM in FA patient lymphoblastoid white blood cell line that we are advancing through IND-enabling studies. After multiple IV administration to Sprague-Dawley rats, this molecule reached tissue concentrations above the EC90 in both heart and brain. Repeat administration at this dose level in Sprague-Dawley rats was found to be well tolerated and resulted in no clinically meaningful changes in hematology, serum biochemistry or histology. We plan to advance this second development candidate into the clinic only if we determine such molecule may have the potential for superior safety or efficacy compared to our first development candidate, and subject to receiving regulatory clearance to proceed with clinical trials.

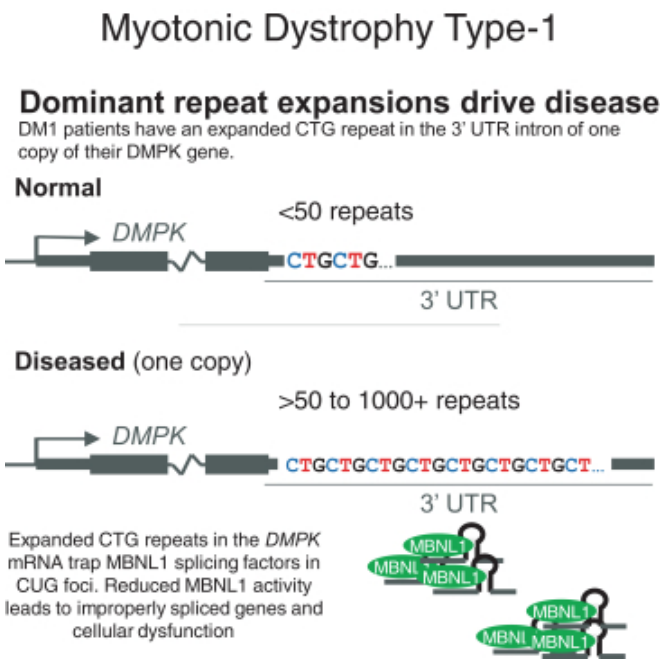
Myotonic Dystrophy Type-1 (DM1)

Disease Overview, Prevalence and Current Treatment Landscape

DM1 is a dominantly-inherited, monogenic progressive neuromuscular disease affecting skeletal muscle, heart, brain, and other organs. Clinical manifestations include muscle weakness, myotonia (slow muscle relaxation), early cataracts, cardiac arrhythmias and changes in neuropsychological function. DM1 is progressive and may become extremely disabling, leading to poor quality of life and early mortality.

DM1 is caused by an increased number of CTG triplet repeats found in the 3’ non-coding region of the DMPK gene. The number of repeats ranges from up to approximately 35 in healthy individuals to many thousands in DM1 patients. When transcribed, the higher-than-normal number of triplet repeats in terminal end of the mutant DMPK allele form pre-mRNAs with large CUG hairpin loops that remain entrapped in the nucleus and form clumps also called foci. Specifically, mutant DMPK pre-mRNA sequesters a critical CUG-binding protein, muscle blind-like protein 1 (MBNL1), which leads to the formation of toxic nuclear foci and inhibits the ability of MBNL1 in processing many pre-mRNAs. As a result, multiple pre-mRNAs that encode key proteins are mis-spliced. This mis-splicing in the nucleus results in the translation of atypical proteins, which ultimately cause the clinical presentation of DM1. When levels of mutant DMPK mRNA containing higher numbers of CUG repeats are reduced, nuclear foci are diminished and MBNL1 proteins are freed to function normally. This disease process is illustrated below:

Figure 8: DM1: Genetic basis and clinical presentation



DM1 is estimated to have a genetic prevalence of 1 in 2,300-8,000 people, affecting more than 70,000 people in the United States and more than 90,000 people in Europe. However, we believe that the patient population is currently underdiagnosed due to lack of available therapies. DM1 is typically categorized into four overlapping phenotypes based on age of onset: late-onset; classical (adult-onset); childhood; and congenital (cDM1).

Overview of DM1 phenotypes

Phenotype	Age of onset	Estimated % of DM1 patients
Late-onset	40+ years	~10%
Classical (Adult-onset)	10 – 40 years	~65% to 75%
Childhood	1 - 10 years	~15%
Congenital (cDM1)	Birth	~5% to 15%

All DM1 phenotypes, except the late-onset form, are associated with high levels of disease burden and premature mortality. The clinical course of DM1 is progressive, and may become extremely disabling, especially when more generalized limb weakness and respiratory muscle involvement develops. Systemic manifestations such as fatigue, GI complications, cataracts and excessive daytime sleepiness greatly impact a patient's quality of life. As a result, DM1 leads to physical impairment, activity limitations, decreased participation in social activities and work and impairs quality of life for patients and their families. Life expectancy in classical DM1 ranges from 48-55 years. Respiratory failure due to muscle weakness (especially diaphragmatic weakness) causes at least 50% of early mortality, and cardiac abnormalities, including sudden death, account for approximately 30% of early mortality.

There are currently no approved therapies for the treatment of DM1, leaving a high unmet medical need and opportunity for new disease-modifying therapies. There are several product candidates currently in preclinical and clinical development, one of which is in later stage clinical development and does not show disease-modifying potential, while the other product candidates are in preclinical stages and have yet to demonstrate clinical proof-of-concept.

Our Approach

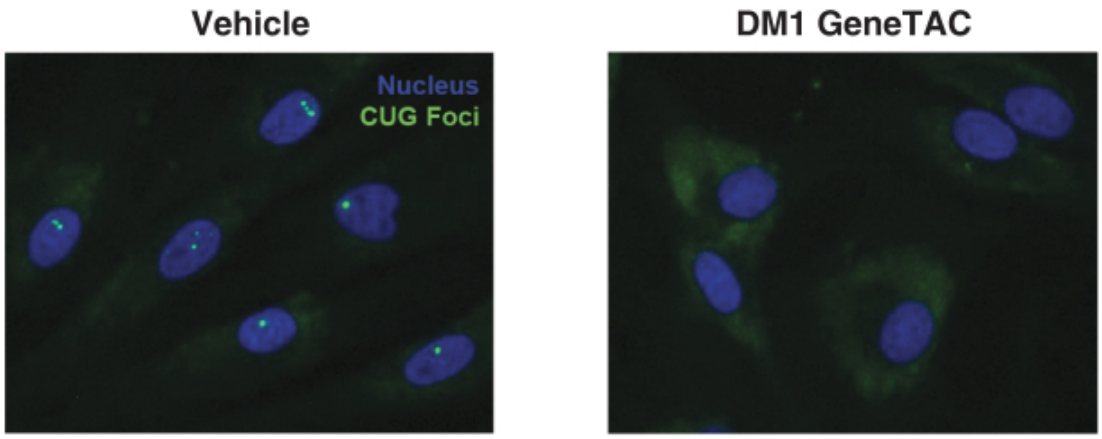
Our DM1 program is based on GeneTAC small molecule candidates consisting of a DNA-binding moiety designed to bind to the CTG repeats in the 3' untranslated region of the DMPK gene, linked to a ligand moiety that is designed to block transcription of the mutant expanded CTG repeat without disrupting the normal DMPK expression. As a result, the DM1 GeneTAC is designed to prevent the formation of the CUG hairpin structures that trap splicing proteins and produce nuclear foci. Like our FA program, the DM1 program is designed to address the underlying cause of the disease and benefit from the favorable development advantages of small molecules.

Preclinical Data

We are currently conducting preclinical studies of our DM1 GeneTACs in DM1 patient cells. We have observed reduced nuclear foci in DM1 cells from multiple patients after administration of our DM1 GeneTACs. We believe these data support the potential for our DM1 GeneTACs to be evaluated in clinical trials as a potential therapy for patients with DM1.

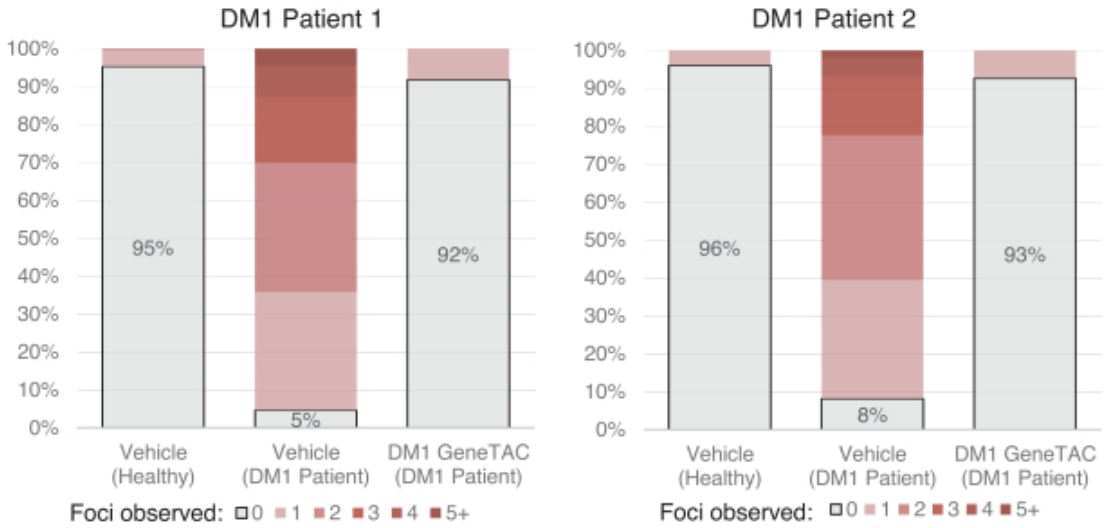
In preclinical studies in DM1 patient cells that contained toxic nuclear DMPK RNA, we observed reduction of nuclear foci following exposure to our DM1 GeneTAC. When toxic nuclear DMPK levels are reduced, the nuclear foci are diminished, releasing splicing proteins, allowing restoration of normal mRNA processing, and potentially stopping or reversing disease progression. As illustrated in Figure 9, we observed a reduction in CUG nuclear foci in DM1 patient cells exposed to our DM1 GeneTAC as determined through a fluorescence in situ hybridization imaging and analysis. This reduction was seen within several days after exposure to our DM1 GeneTAC. The reduced CUG nuclear foci are indicated by the reduction in green punctate staining.

Figure 9: Decrease in CUG nuclear foci in DM1 patient cells exposed to DM1 GeneTACs



In preclinical studies in cells from multiple DM1 patients, we observed reduced the number of observable CUG nuclear foci after exposure to our DM1 GeneTAC. As seen in Figure 10, after exposing DM1 Patient 1 and 2 cells to our DM1 GeneTAC, we observed an increase in the percentage of cells with no observable CUG nuclear foci to over 92%, similar to healthy cells.

Figure 10: CUG nuclear foci in cells from multiple DM1 patients



We plan to continue evaluating the properties of our DM1 GeneTACs in both *in vivo* and *in vitro* preclinical studies. We expect to complete IND-enabling studies and seek regulatory clearance for a first-in-human clinical trial in 2023.

Discovery Programs

We are also advancing our GeneTAC portfolio in other serious nucleotide repeat expansion-driven monogenic diseases, such as FECD, Fragile X syndrome, spinocerebellar ataxias, amyotrophic lateral sclerosis, frontotemporal dementia, Huntington disease and spinobulbar muscular atrophy as outlined in the table below. Many of these monogenic diseases have overlapping triplet repeat expansions, including CTG repeats that cause DM1, allowing for the potential for a single GeneTAC to be used across multiple diseases. Additionally, our experiences with GeneTACs allow us to more rapidly design GeneTACs for additional indications.

Figure 11: Broad applicability of GeneTACs across multiple indications

PROGRAM	ESTIMATED U.S. PREVALENCE
CGG	
• Fragile X	~87,000
• Fragile XE mental retardation	~6,500
CAG/CTG	
• Fuchs endothelial corneal dystrophy (TCF4)	~5 million
• Huntington disease	~40,000
• Various spinocerebellar ataxias	~18,000 total
• Spinobulbar muscular atrophy	~5,400
GGGGCC	
• ALS/FTD (C9orf72)	~7,000
TGGAA	
• SCA31	Not reported

Competition

The biotechnology and biopharmaceutical industries are characterized by rapid technological advancement, significant competition and an emphasis on intellectual property. Any product candidates that we successfully design, develop and commercialize will compete with current therapies and new therapies that may become available in the future. While we believe that our technology, development experience and scientific knowledge in the field of nucleotide repeat expansion diseases and small molecules provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions.

Friedreich Ataxia. There are currently no approved therapies for the treatment of FA. Some of the product candidates currently in development to treat FA include: omaveloxolone, a Nrf2 activator by Reata Pharmaceuticals; vatiquinone, a 15-lipoxygenase inhibitor by PTC Therapeutics; RT001, a deuterated poly-saturated fatty acid by Retrotope; leriglitazone, a PPAR-gamma agonist by Minoryx Therapeutics; and CTI-1601, an HIV-derived cell penetrating peptide FXN recombinant fusion protein by Larimar Therapeutics. In addition, several companies are in preclinical development for AAV-based gene therapies, including PTC therapeutics, Voyager Therapeutics, Loxeo Therapeutics, Pfizer, StrideBio, and AavantiBio. We are not aware of any competing company that has a small molecule program in development that is designed to restore deficient FXN protein levels, the underlying cause of FA.

Myotonic Dystrophy Type-1. There are currently no approved therapies for the treatment of DM1. The only product candidate in clinical-stage development of which we are aware is tideglusib, a GSK3-ß inhibitor by AMO Pharma for the congenital phenotype of DM1. Some of the products

currently in preclinical development to treat DM1 include: a histamine 3 receptor inhibitor by Harmony Biosciences for the treatment of excessive daytime sleepiness in DM1; an antibody linked siRNA by Avidity Biosciences; an AAV-antisense candidate by Audentes Therapeutics, an Astellas company; an antibody linked oligonucleotide by Dyne Therapeutics; an miR-23b antisense candidate; gene editing treatments by Vertex Pharmaceuticals; an RNA-targeting AAV-based gene therapy by Locana; an AAV-based RNA degrading gene therapy by Enzerna Biosciences; antisense oligonucleotides by NeuBase Therapeutics; antisense oligonucleotides and siRNA candidates by Triplet Therapeutics; small molecules interacting with RNA by Anima Biotech; small molecule modulators of transcription factors by Syros Pharmaceuticals; and small molecules interacting with RNA by Expansion Therapeutics.

Other Nucleotide Repeat Expansion Driven Diseases. There are currently no approved therapies targeting the underlying cause of other inherited nucleotide repeat expansion diseases where we believe GeneTACs could have applicability, including FECD, Fragile X syndrome, spinocerebellar ataxias, amyotrophic lateral sclerosis, frontotemporal dementia, Huntington disease and spinobulbar muscular atrophy, among others.

We will also compete more generally with other companies developing product candidates that utilize alternative scientific and technological approaches to modulate individual genes, including other companies working to develop nuclease-based gene editing technologies, such as Beam Therapeutics, CRISPR Therapeutics, Editas Medicine, Intellia Therapeutics, Precision BioSciences and Sangamo Biosciences.

Many of our competitors, either alone or with strategic partners, have substantially greater financial, technical and human resources than we do. Accordingly, our competitors may be more successful than us in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approval for treatments and achieving widespread market acceptance, rendering our treatments obsolete or non-competitive. Merger and acquisition activity in the biotechnology and biopharmaceutical industries may result in even more resources being concentrated within a smaller number of our competitors. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our commercial opportunity could be substantially limited if our competitors develop and commercialize products that are more effective, safer, less toxic, more convenient or less expensive than our comparable products. In geographies that are critical to our commercial success, competitors may also obtain regulatory approvals before us, resulting in our competitors building a strong market position in advance of the entry of our products. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of other drugs. The key competitive factors affecting the success of our programs are likely to be their efficacy, safety profile, biodistribution, manufacturability, effectiveness of commercial activities, intellectual property protection and availability of reimbursement.

License Agreement

License Agreement with Wisconsin Alumni Research Foundation

On February 20, 2019, we and Wisconsin Alumni Research Foundation (WARF) entered into a human therapeutics exclusive license agreement (the WARF License Agreement), pursuant to which we received (i) an exclusive, worldwide, royalty-bearing, sublicensable license under certain of WARF's patents relating to compounds and methods for treating genetic disease by modulating gene expression, including expression of frataxin, through nucleic acid binding moieties that specifically bind to one or more repeats of a target oligonucleotide sequence as well as a synthetic transcription factor having a nucleic acid binding moiety that specifically binds to a target oligonucleotide sequence and (ii) a non-exclusive, worldwide, sublicensable license under certain of WARF's know-how relating to the

foregoing patents, in each case (i) and (ii), to research, develop, make, have made, use, have used, sell, offer for sale, have sold, export and import products developed through the use of such licensed patents and know-how in all fields. We are currently using the licensed patents and know-how in our FA program. The licenses granted pursuant to the WARF License Agreement are subject to certain rights retained by (i) the United States government under the Bayh-Dole Act and (ii) WARF to grant the University of Wisconsin, non-profit research institutions collaborating with the University of Wisconsin and governmental agencies non-exclusive licenses to practice and use the licensed patents for non-commercial research purposes. Such rights retained by the United States government and WARF are typical for a license from a U.S. university or research institution, and we believe such rights do not pose a material risk to our business. We further granted to WARF, the University of Wisconsin, the inventors of the licensed patents, and governmental research organizations a covenant not to sue under certain improvements to the licensed patents for non-commercial research purposes. Under the WARF License Agreement, we are required to use commercially reasonable diligence to develop, seek regulatory approval for, manufacture, market and sell licensed products throughout the term of the agreement, including satisfying certain funding and diligence milestones.

In consideration for the rights granted to us under the WARF License Agreement, we paid WARF an upfront licensing fee of $250,000, which is the only payment we have made under the WARF License Agreement to date. We are also required to pay WARF up to an aggregate of $17.6 million upon the achievement of certain development and commercial sales milestones. Each such milestone payment is payable once for each licensed product for which the milestone is achieved, except for the two milestones relating to IND submission and human proof of concept study, which are payable only for the first licensed product for which such milestones are achieved (and not for subsequent licensed products). In addition, we are required to pay WARF, on a licensed product-by-licensed product and country-by-country basis, upon first commercial product sale, a fixed royalty of a low single digit percentage on sales of licensed products by us and/or by our sublicensees, subject to certain reductions and a minimum total annual royalty payment of $100,000. Our royalty obligation will terminate on the date of expiration of the last-to-expire of the licensed patents in the relevant country. We are also obligated to pay WARF sublicense fees in the mid-single digits percentage for funding or royalties earned from the granting of sublicenses to the WARF patents and know-how. We are required to reimburse WARF for all costs associated with filing, prosecuting and maintaining the licensed patents prior to and after the effective date of the WARF License Agreement.

The WARF License Agreement will continue until the earliest of (i) the date of early termination in accordance with the agreement, (ii) expiration of the licensed patents in all countries, or (iii) our cessation, once begun, of royalty payments for more than two years. The WARF License Agreement may be terminated by us upon 90 days' written notice, provided we include a statement of reasons for termination. WARF may terminate the WARF License Agreement (a) upon written notice if our cumulative earned royalties paid to WARF does not exceed $100,000 on or before December 31, 2031, (b) if we fail to make timely payments, fail to timely provide development reports or provide any false information with respect thereto, fail to actively pursue the development plan, or commit any breach of any other covenant, representation or warranty under the WARF License Agreement, in each case, without curing such failure or breach within 90 days after written notice thereof, (c) if we commit any act of bankruptcy or become insolvent, or (d) immediately if we or our sublicensee(s) offer any rights to the licensed patents to our or our sublicensees' creditors. As of January 31, 2021, the licensed patents include one issued U.S. patent that is projected to expire on or around March 29, 2037, not including any patent term adjustments and extensions. The license also includes three pending patent applications in the United States, Europe, and Canada. Any patents that issue from these patent applications are projected to expire on or around March 29, 2037, not including any patent term adjustments and extensions that may be available. Additionally, the license includes a pending Patent Cooperation Treaty application, with patents that eventually issue from this patent application expected to expire on or around October 22, 2039, not including any patent term adjustments and extensions.

Finally, the license includes another pending U.S. patent application which, if issued, will expire on or around March 29, 2038, not including any patent term adjustments and extensions that may be available.

Manufacturing

GeneTACs are synthetically tractable, offering a readily scalable, cost-effective development path that does not require complex customized equipment and processes. We do not own or operate, and currently have no plans to establish, current Good Manufacturing Practice (cGMP) manufacturing facilities and laboratories. We currently rely on third-party manufacturers and suppliers for the raw materials and starting components used to make our GeneTACs, and we expect to continue to do so to meet our research and development and commercial activities. Our third-party manufacturers are qualified to manufacture our product candidates under cGMP requirements and other applicable laws, guidances and regulations. We believe there are multiple sources for all of the materials and components required for the manufacture of our product candidates.

Intellectual Property

Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates and other discoveries, inventions, trade secrets and know-how that are critical to our business operations. Our success also depends in part on our ability to operate without infringing the proprietary rights of others, and in part, on our ability to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual property is provided under the section of this prospectus titled “Risk Factors—Risks Related to Our Intellectual Property.”

As of January 31, 2021, we own ten pending U.S. patent applications, six pending European patent applications and one pending Patent Cooperation Treaty application, which, if issued (or in case of provisional applications, if issued from future non-provisional applications that we file), are expected to expire between 2039 and 2042, not including any patent term adjustments and extensions that may be available. In addition, we acquired an exclusive license from WARF under one issued U.S. patent, two pending U.S. patent applications, one pending European patent application, one pending Canadian patent application and one pending Patent Cooperation Treaty application, with the issued patent expected to expire in 2039 and the other patents, if issued, are expected to expire between 2037 and 2039, not including any patent term adjustments and extensions that may be available. These patents cover our proprietary GeneTAC Platform technology that is used in our FA program, DM1 program and other therapeutic programs directed to genetic diseases that are further discussed below.

For our FA program, as of January 31, 2021, we own or hold an exclusive, worldwide license from WARF. In particular, we have licensed from WARF an issued U.S. patent, a pending U.S. patent application, and two pending patent applications in Europe and Canada directed to compounds and methods for modulating frataxin expression. The U.S. patent and any patents issued from these pending patent applications are projected to expire on or around March 29, 2037, not including any patent term adjustments and extensions. We also license from WARF a pending Patent Cooperation Treaty application directed to methods and compounds for treatment of FA. Any patents that eventually issue from this patent application are projected to expire on or around October 22, 2039. Additionally, we own four pending U.S. patent applications, and a pending patent application in Europe directed to compositions of matter and methods for the treatment of FA. Any patents that eventually issue from these patent applications are projected to expire in the 2039-2042 timeframe.

For our DM1 program, as of January 31, 2021, two of our pending U.S. patent applications and one pending patent application in Europe are directed to compositions of matter and methods for the

treatment of DM1. Any patents issuing from patent applications in these families are projected to expire in the 2039-2041 timeframe, not including any patent term adjustments and extensions that may be available.

As of January 31, 2021, four of our pending U.S. patent applications, four of our pending patent applications in Europe and one of our pending Patent Cooperation Treaty application are directed to our therapeutic programs related to other genetic diseases (including, but not limited to, Amyotrophic Lateral Sclerosis, Fragile X and Spinocerebellar Ataxia). These therapeutic programs utilize our GeneTAC Platform technology and any patents that issue from these patent applications are projected to expire in the 2039-2041 timeframe not including any patent term adjustments or extensions. We also license from WARF a pending U.S. patent application that, if issued, is projected to expire on or around March 29, 2038, not including any patent term adjustments or extensions. These patent applications cover compositions of matter and methods of treating the related genetic diseases under our therapeutic programs, including the use of transcription modulator molecules that contain a DNA-binding moiety capable of specifically binding to certain nucleotide repeat sequences that are implicated in the applicable genetic disease. In addition to patent protection, we rely on trade secret protection, trademark protection and know-how to expand our proprietary position around our chemistry, technology and other discoveries and inventions that we consider important to our business. We are a party to the WARF License Agreement, described in more detail above, under which we are granted intellectual property rights to know-how that are important to our business.

As of January 31, 2021, six of our pending U.S. patent applications and six of our pending patent applications in Europe are directed to our GeneTAC Platform. Any patents that are expected to issue from these patent applications are projected to expire in 2039, not including any patent term adjustments and extensions that may be available.

We also seek to protect our intellectual property by having confidentiality terms in our agreements with companies with whom we share proprietary and confidential information in the course of business discussions, and by having confidentiality terms in our agreements with our employees, consultants, scientific advisors, clinical investigators and other contractors and also by requiring our employees, commercial contractors, and certain consultants and investigators, to enter into invention assignment agreements that grant us ownership of any discoveries or inventions made by them while in our employ.

Government Regulation and Product Approval

As a pharmaceutical company that operates in the United States, we are subject to extensive regulation. Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug products such as those we are developing. Product candidates that we develop must be approved by the Food and Drug Administration (FDA), before they may be legally marketed in the United States and by the appropriate foreign regulatory agency before they may be legally marketed in foreign countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way, but country-specific regulation remains essential in many respects.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act (FDCA), and implementing regulations. A new drug must be approved by the FDA through the new

drug application (NDA) process before it may be legally marketed in the United States. Drugs are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include, among other actions, refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us. The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests, preclinical animal studies and formulation studies in accordance with applicable regulations, including the FDA's Good Laboratory Practice (GLP) regulations and other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board (IRB) at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable regulations, including the FDA's good clinical practice (GCP) regulations to establish the safety and efficacy of the proposed drug for its proposed indication;
- submission to the FDA of an NDA after completion of all pivotal trials;
- a determination by the FDA within 60 days of its receipt of an NDA to file the NDA for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the drug is produced to assess compliance with the FDA's cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- potential FDA audit of the preclinical and/or clinical trial sites that generated the data in support of the NDA to assess compliance with GCP regulations;
- satisfactory completion of an FDA advisory committee review, if applicable; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Before testing any compounds with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies, to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLP requirements. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human trials. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the proposed clinical trials and places the IND on clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a product candidate at any time before or during clinical trials due to safety concerns or non-compliance.

Clinical trials involve the administration of the product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Further, each clinical trial must be reviewed and approved by an independent IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- ***Phase 1.*** The drug is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion, the side effects associated with increasing doses and if possible, to gain early evidence of effectiveness. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- ***Phase 2.*** The drug is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases or conditions and to determine dosage tolerance, optimal dosage and dosing schedule. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- ***Phase 3.*** The drug is administered to an expanded patient population to further evaluate dosage and clinical efficacy at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall benefit/risk ratio of the product and provide an adequate basis for product approval. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, FDA may mandate the performance of Phase 4 trials. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice, and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the meetings at the end of the Phase 2 trial to discuss Phase 2 clinical results and present plans for the pivotal Phase 3 clinical trials that they believe will support approval of the new drug.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected AEs or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA, the IRB, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access to certain data from the trial.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. Data may come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA. The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances.

In addition, the Pediatric Research Equity Act (PREA) requires a sponsor to conduct pediatric clinical trials for most drugs, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, original NDAs and supplements must contain a pediatric assessment unless the sponsor has received a deferral or waiver. The required assessment must evaluate the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The sponsor or FDA may request a deferral of pediatric clinical trials for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric clinical trials are complete or that additional safety or effectiveness data need to be collected before the pediatric clinical trials begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation. Unless otherwise required by regulation, the Pediatric Research Equity Act does not apply to any drug for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s).

The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the Prescription Drug User Fee Act (PDUFA) guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision after it the application is submitted The FDA does not always meet its PDUFA goal dates for standard and priority NDAs, and the review process is often significantly extended by FDA requests for additional information or clarification.

After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA may refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions and typically follows the advisory committee's recommendations.

Before approving an NDA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical sites to assure compliance with GCP requirements. After the FDA evaluates the application, manufacturing process and manufacturing facilities, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the NDA identified by the FDA. The Complete Response Letter may require additional clinical data and/or (an) additional pivotal Phase 3 clinical trial(s), and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling or may condition the approval of the NDA on other changes to the proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post-market studies or clinical trials. For example, the FDA may require Phase 4 testing, which involves clinical trials designed to further assess a drug safety and effectiveness, and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. The FDA may also determine that a REMS is necessary to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the NDA without an approved REMS, if required. A REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States or, if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making a drug product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA. After the FDA grants orphan designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or inability to manufacture the product in sufficient quantities. The designation of such drug also entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity.

Expedited Development and Review Programs

The FDA has a number of programs intended to expedite the development or review of products that meet certain criteria. For example, the FDA has a fast track designation program that is intended to expedite or facilitate the process for reviewing new drug products that meet certain criteria. Specifically, new drugs are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a fast track product has opportunities for more frequent interactions with the applicable FDA review team during product development and, once an NDA is submitted, the product candidate may be eligible for priority review. With regard to a fast track product, the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

Any product submitted to the FDA for approval, including a product with a fast track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it is designed to treat a serious condition, and if approved, would provide a significant improvement in the treatment, diagnosis or prevention of a serious condition compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review. The FDA endeavors to review applications with priority review designations within six months of the filing date as compared to ten months for review of new molecular entity NDAs under its current PDUFA review goals.

In addition, a product may be eligible for accelerated approval. Drug products intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials to verify the predicted clinical benefit. Products receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to conduct the required clinical trials, or if such trials fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

A sponsor may seek FDA designation of a product candidate as a “breakthrough therapy” if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes intensive FDA interaction and guidance. If a drug is designated as breakthrough therapy, FDA will expedite the development and review of such drug. Breakthrough therapy designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance. The breakthrough therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. If a product is designated as breakthrough therapy, the FDA will work to expedite the development and review of such drug.

Fast track designation, priority review, accelerated approval and breakthrough therapy designation do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. In addition, such designations or shortened review periods may not provide a material commercial advantage.

Post-Approval Requirements

Any drug products manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, and complying with FDA promotion and advertising requirements. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual program fees for any marketed products.

In addition, quality control and manufacturing procedures must continue to conform to applicable manufacturing requirements after approval to ensure the long term stability of the drug product. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to

expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. In addition, changes to the manufacturing process are strictly regulated, and depending on the significance of the change, may require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical studies to assess new safety risks, or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters, or untitled letters;
- clinical holds on clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, warning letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures.

The FDA closely regulates the marketing, labeling, advertising and promotion of drug products. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe, in their independent professional medical judgment, legally available products for uses that are not described in the product's labeling and that differ from those tested and approved by the FDA. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products. The

federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined companies from engaging in off-label promotion. The FDA and other regulatory agencies have also required that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA-approved labelling.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Marketing Exclusivity

Market exclusivity provisions under the FDCA can also delay the submission or the approval of certain marketing applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an abbreviated new drug application (ANDA) or a 505(b)(2) NDA submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder. The FDCA alternatively provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity, except in certain circumstances. Pediatric exclusivity is another type of non-patent market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial.

Other U.S. Healthcare Laws and Compliance Requirements

Although we currently do not have any products on the market, we are and, upon approval and commercialization, will be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. In the United States, such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security, price reporting, and physician sunshine laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. Additionally, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The federal false claims laws, including the False Claims Act, which prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim under the False Claims Act includes “any request or demand” for money or property presented to the U.S. government. The federal civil False Claims Act can be enforced through private “qui tam” actions brought by individual whistleblowers in the name of the government. In addition, manufacturers can be held liable under the civil False Claims Act even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. Pharmaceutical and other healthcare companies have been prosecuted under these laws for, among other things, allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product and for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, and thus non-covered, uses. In addition, a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act.

The federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) also created new federal criminal statutes that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Additionally, the federal Physician Payments Sunshine Act, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program (with certain

exceptions) annually report information related to certain payments or other transfers of value made or distributed to physicians (currently defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, certain ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, applicable manufacturers will also be required to report information regarding payments and other transfers of value provided during the previous year to physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse-midwives.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, track and report gifts, compensation and other remuneration made to physicians and other healthcare providers, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are also potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to significant penalties, including without limitation, civil, criminal and/or administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private “qui tam” actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Data Privacy and Security

We may also be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and regulations implemented thereunder, imposes obligations on “covered entities,” including certain health care providers, health plans, and health care clearinghouses, and their respective “business associates” and covered subcontractors that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, as well as their covered subcontractors with respect to the privacy, security and transmission of individually identifiable health information. Entities that are found to be in violation of HIPAA, whether as the result of a breach of unsecured PHI, a complaint about privacy practices, or an audit by HHS, may be subject to significant civil, criminal, and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance.

In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. For example, California recently enacted the California

Consumer Privacy Act (CCPA), which went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling certain personal data of consumers or households. The CCPA requires covered businesses to provide new disclosure to consumers about such business' data collection, use and sharing practices, provide such consumers new ways to opt-out of certain sales or transfers of personal information, and provide consumers with additional causes of action. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. Additionally, a new privacy law, the California Privacy Rights Act (CPRA), was approved by California voters on November 3, 2020. When it goes into effect on January 1, 2023, the CPRA will modify significantly the CCPA, potentially resulting in further uncertainty and requiring us to incur additional costs and expenses in an effort to comply. Both the CCPA and CPRA could impact our business activities depending on how they are interpreted and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information.

We also are or will become subject to applicable privacy laws in the jurisdictions in which we are established or in which we sell or market our products or run clinical trials. For example, if we conduct EU-based clinical trials, we will be subject to Regulation (EU) 2016/679, the General Data Protection Regulation (GDPR) in relation to our collection, control, processing and other use of personal data of European data subjects (i.e. data relating to an identifiable living individual). We process personal data in relation to participants in our clinical trials in the European Economic Area (EEA), including the health and medical data of these participants. The GDPR is directly applicable in each EU and EEA Member State, however, it provides that EU and EEA Member States may introduce further conditions, including limitations which could limit our ability to collect, use and share personal data (including health and medical data), or could cause our compliance costs to increase, ultimately having an adverse impact on our business. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing activities and implement policies as part of its mandated privacy governance framework. It also requires data controllers to be transparent and disclose to data subjects (in a concise, intelligible and easily accessible form) how their personal data is to be used, imposes limitations on retention of personal data; defines pseudonymized (i.e., key-coded) data; introduces mandatory data breach notification requirements; and sets higher standards for data controllers to demonstrate that they have obtained valid consent for certain data processing activities. We are also subject to EU rules with respect to cross-border transfers of personal data out of the EU and EEA; for example, in July 2020, the Court of Justice of the European Union limited how organizations could lawfully transfer personal data from the EEA to the United States by invalidating the EU-US Privacy Shield and imposing further restrictions on use of the standard contractual clauses, which could increase our costs and our ability to efficiently process personal data from the EEA. We are subject to the supervision of local data protection authorities in those EU jurisdictions where we are established or otherwise subject to the GDPR, and we maintain an office in Switzerland, which has its own set of stringent privacy and data protection laws and regulations. Fines for certain breaches of the GDPR are significant: up to the greater of €20 million or 4% of total global annual turnover. In addition to the foregoing, a breach of the GDPR or other applicable privacy and data protection laws and regulations could result in regulatory investigations, reputational damage, orders to cease/change our use of data, enforcement notices, or potential civil claims including class action type litigation.

Further, the United Kingdom (UK)'s withdrawal from the EU and the EEA, referred to as Brexit, has created uncertainty with regard to data protection regulation in the UK. Specifically, while the Data Protection Act of 2018, which "implements" and complements the GDPR achieved Royal Assent on May 23, 2018 and is now effective in the UK, aspects of data protection in the UK, such as the transfer of data from the EEA to the UK, remain uncertain. Following the expiry of the transition period, companies have to comply with both the GDPR and the GDPR as incorporated into UK law. Beginning

in 2021, the UK is now a “third country” under the GDPR. Pursuant to the EU-UK Trade and Cooperation Agreement of December 24, 2020, transfers of personal data from the EU to the UK may continue to take place without a need for additional safeguards during a further transition period, to expire on (1) the date on which an adequacy decision with respect to the UK is adopted by the EU Commission; or (2) the expiry of four months, which shall be extended by a further two months unless either the EU or the UK objects. It remains unclear whether the EU Commission will adopt an adequacy decision with respect to the UK. In the absence of such decision after the expiry of the additional transition period, companies may need to put in place additional safeguards for transfers of personal data from the EU to the UK, such as standard contractual clauses approved by the EU Commission.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we or our collaborators obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage, and establish adequate reimbursement levels for such drug products.

In the United States, third-party payors include federal and state healthcare programs, government authorities, private managed care providers, private health insurers and other organizations. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical drug products and medical services, in addition to questioning their safety and efficacy. Such payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the FDA-approved drugs for a particular indication. We or our collaborators may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Nonetheless, our product candidates may not be considered medically necessary or cost-effective. Moreover, the process for determining whether a third-party payor will provide coverage for a drug product may be separate from the process for setting the price of a drug product or for establishing the reimbursement rate that such a payor will pay for the drug product. A payor’s decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor. As a result, one payor’s determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. For gene therapy and other products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

If we elect to participate in certain governmental programs, we may be required to participate in discount and rebate programs, which may result in prices for our future products that will likely be lower than the prices we might otherwise obtain. For example, drug manufacturers participating under the Medicaid Drug Rebate Program must pay rebates on prescription drugs to state Medicaid programs.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing

approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any product candidates for which we or our collaborators receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products and services, implementing reductions in Medicare and other healthcare funding and applying new payment methodologies. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively ACA), was enacted, which affected existing government healthcare programs and resulted in the development of new programs.

Among the ACA's provisions of importance to the pharmaceutical industry, in addition to those otherwise described above, are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively, and a cap on the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price (AMP);
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals, including individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

There have been executive judicial and Congressional challenges to certain aspects of the ACA. For example, the Tax Cuts and Jobs Act (the Tax Act) was enacted, which includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. The case is currently under review by the U.S. Supreme Court but it is unknown when a decision will be reached. Although the Supreme Court has not yet ruled on the constitutionality of the Affordable Care Act, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through May 15, 2021 for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. It is unclear how the Supreme Court ruling, other such litigation and the healthcare reform measures of the Biden administration will impact the ACA or our business.

Other legislative changes have also been proposed and adopted in the United States since the Healthcare Reform Act was enacted. On August 2, 2011, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030, with the exception of a temporary suspension from May 1, 2020 through March 31, 2021, unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. There has been heightened governmental scrutiny recently over the manner in which pharmaceutical companies set prices for their marketed products, which has resulted in several Congressional inquiries and proposed federal legislation, additional federal regulations, as well as state efforts, designed to, among other things, bring more transparency to product pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products.

Recently, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent congressional inquiries and legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, the Trump administration announced several executive orders related to prescription drug pricing that seek to implement several of the administration's proposals. As a result, the FDA released a final rule on September 24, 2020, effective November 30, 2020, providing guidance for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the United States Department of Health and Human Services (HHS) finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers,

unless the price reduction is required by law. The implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed pending review by the Biden administration until March 22, 2021. On November 20, 2020, CMS issued an interim final rule implementing President Trump's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. It is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control costs pharmaceutical and biological products. Moreover, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We anticipate that these new laws will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition, and results of operations. Further, it is possible that additional governmental action is taken in response to the evolving effects of the COVID-19 pandemic. Additionally, health reform initiatives may arise in the future, particularly in light of the new Biden administration.

The U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act of 1977 (FCPA) prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Europe / Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we or our potential collaborators obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of an application for a clinical trial authorization (CTA) much like the IND prior to the commencement of human clinical trials. In the EU, for example, a CTA must be submitted to each country's national health authority and an application made to an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements and a favorable ethics committee opinion has been issued, clinical trial development may proceed.

The requirements and process governing the conduct of clinical trials are to a significant extent harmonized at the EU level, but could vary from country to country. In all cases, the clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki. The way clinical trials are conducted in the EU will undergo a major change when the Clinical Trial Regulation (Regulation (EU No 536/2014) comes into application, expected in 2022.

To obtain regulatory approval of an investigational drug or biological product under EU regulatory systems, we must submit a marketing authorization application either under the so-called centralized or national authorization procedures. The application used to file an NDA in the United States is similar to that required in the European Union, but the exact requirements for authorization may vary.

Centralized Procedure. The centralized procedure provides for the grant of a single marketing authorization by the European Commission following a favorable opinion by the European Medicines Agency (EMA) that is valid in all EU member states, as well as Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for medicines produced by specified biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of specified diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases, other immune dysfunctions and viral diseases. The centralized procedure is optional for other products that represent a significant therapeutic, scientific or technical innovation, or whose authorization would be in the interest of public health or which contain a new active substance for indications other than those specified to be compulsory.

National Authorization Procedures. There are also two other possible routes to authorize medicinal products in several EU countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:

- ***Decentralized Procedure.*** Using the decentralized procedure, an applicant may apply for simultaneous authorizations in more than one EU Member State of medicinal products that have not yet been authorized in any EU Member State and that do not fall within the mandatory scope of the centralized procedure.
- ***Mutual Recognition Procedure.*** In the mutual recognition procedure, a medicine is first authorized in one EU Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other EU countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

The European Union also provides opportunities for market exclusivity. For example, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic or biosimilar application. During the additional two-year period of market exclusivity, a generic or biosimilar marketing authorization can be submitted, and the innovator's data may be referenced, but no generic or biosimilar product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity.

The EMA grants orphan drug designation to promote the development of products for the treatment, prevention or diagnosis of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the EU. In addition, orphan drug designation can be granted if the drug is intended for a life threatening or chronically debilitating condition in the EU and without incentives it is unlikely that sales of the drug in the EU would be sufficient to justify the investment

required to develop the drug. Orphan drug designation is only available if there is no other satisfactory method approved in the EU of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation provides opportunities for free or reduced-fee protocol assistance, fee reductions for marketing authorization applications and other post-authorization activities and ten years of market exclusivity following drug approval, which can be extended to 12 years if trials are conducted in accordance with an agreed-upon pediatric investigational plan. The exclusivity period may be reduced to six years if the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

In the European Union, early access mechanisms for innovative medicines (such as compassionate use programs and named patient supplies), pricing and reimbursement, and promotion and advertising, amongst other things, are subject to national regulations and oversight by national competent authorities and therefore significantly vary from country to country.

Sanctions for non-compliance with the aforementioned requirements, which may include administrative and criminal penalties, are generally determined and enforced at national level. However, under the EU financial penalties regime, the EMA can investigate and report on alleged breaches of the EU pharmaceutical rules by holders of a marketing authorization for centrally authorized medicinal products and the European Commission could adopt decisions imposing significant financial penalties on infringing marketing authorization holders.

The United Kingdom left the European Union on January 31, 2020. Following the transition period which ended on December 31, 2020, Brexit could materially impact the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the United Kingdom in the coming years.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we or our potential collaborators fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Facilities

We currently occupy approximately 1,000 square feet of laboratory and office space in La Jolla, California, pursuant to a three-month, automatically renewing lease. In April, 2021, we will occupy an additional 600 square feet of space pursuant to this agreement. In addition, we have access to approximately 2,120 square feet of office space in Carlsbad, California, on an as-available basis from time to time.

We entered into a new lease agreement to rent approximately 12,370 square feet of laboratory and office space in Carlsbad, California. The lease is expected to commence September 2021 and expire 72 months after commencement. We believe that this new facility will meet our current and near term needs and that suitable additional space will be available as and when needed.

Employees and Human Capital Resources

As of March 22, 2021, we had 20 full-time employees, 11 of whom have a Ph.D. or M.D. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

Legal Proceedings

From time to time, we may be involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, in the opinion of management, would have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputation harm, and other factors.

MANAGEMENT

The following table sets forth information regarding our executive officers and directors as of January 25, 2021:

Name	Age	Position(s)
Executive Officers:		
Pratik Shah, Ph.D.	51	Director, Executive Chairperson
João Siffert, M.D.	57	Director, President and Chief Executive Officer
Sean Jeffries, Ph.D.	41	Chief Operating Officer
Non-Employee Directors:		
Simeon George, M.D.(2)(3)	43	Director
Stella Xu, Ph.D.(1)(4)	50	Director
Rodney Lappe, Ph.D.(3)(4)	66	Director
John Schmid(1)(2)	58	Director
Arsani William, M.D.(1)(2)	32	Director

(1) Member of our audit committee.
(2) Member of our compensation committee.
(3) Member of our nominating and corporate governance committee.
(4) Member of the research and development committee.

Executive Officers

Pratik Shah, Ph.D. has served as our Executive Chairperson and a member of our board of directors since December 2017. Dr. Shah has also served as the Chairman of the board of directors for ARS Pharmaceuticals, Inc. since April 2016. He has also served as President of Marlinspike Group, LLC since August 2018 and of Marlinspike Group, Inc. from June 2015 to October 2020. Dr. Shah served as the Chairman of the board of directors of Synthorx, Inc. from October 2018 until its acquisition by Sanofi S.A. in January 2020. Dr. Shah also served as the President and Chief Executive Officer and Chairman of the board of directors of Auspex Pharmaceuticals, Inc. from October 2013 until its acquisition by Teva Pharmaceuticals Industries Ltd. in May 2015. From 2004 to 2014 he was a partner at Thomas, McNerney & Partners. Dr. Shah holds a B.S in Biological Sciences from the University of California at Irvine and a Ph.D. in Biochemistry and Molecular Biology and an M.B.A. in Finance, both from the University of Chicago. We believe Dr. Shah is qualified to serve on our board of directors due to his experience as a director and executive officer of biopharmaceutical companies, his extensive background as venture capitalist in the biopharmaceutical industry and his educational background.

João Siffert, M.D. has served as our Chief Executive Officer and a member of our board of directors since October 2020 and as our President since January 2021. Prior to joining Design, Dr. Siffert served as the Chief Executive Officer and member of the board of directors at Abeona Therapeutics, Inc. since November 2018, where he also led Research and Development since October 2018. From May 2016 to October 2018, Dr. Siffert served as Chief Scientific and Medical Officer at Nestle Health Science S.A. He also served as a member of the board of directors of AveXis, Inc. from May 2017 to May 2018 and Alcobra Ltd. (now Arcturus Therapeutics Inc.) from July 2015 to July 2017. From August 2011 to April 2016, Dr. Siffert served as Executive Vice President, R&D and Chief Medical Officer of Avanir Pharmaceuticals Inc. He also previously served in executive leadership roles at Ceregene Inc. and Avera Pharmaceuticals Inc. Dr. Siffert holds an M.D. from the University of São Paulo, Brazil and an M.B.A. from Columbia University. He completed residency training in Pediatrics at New York University (NYU) School of Medicine and in Neurology at Harvard Medical School, followed by a clinical fellowship in neuro-oncology at NYU. We believe Dr. Siffert is qualified to serve on our

board of directors due to his extensive experience as a director and senior executive officer of biotechnology companies and his educational background.

Sean Jeffries, Ph.D. has served as our Chief Operating Officer since January 2021 and as our Chief Business Officer from May 2019 to January 2021. Dr. Jeffries served as a principal of Marlinspike Group, Inc. from February 2018 to December 2018 and Marlinspike Group from January 2019 to February 2020. From April 2014 to January 2018, Dr. Jeffries worked as a Management Consultant at The Boston Consulting Group leading biopharma R&D strategy project as a core member of the Healthcare and Private Equity groups. Dr. Jeffries holds a B.A. in Computer Science from the College of Wooster and a Ph.D. in Physiology, Development and Neuroscience from the University of Cambridge.

Non-Employee Directors

Simeon George, M.D. has served as a member of our board of directors since February 2020. Since September 2020, Dr. George has served as the Chief Executive Officer of SR One Capital Management, LP. Dr. Dr. George was previously Chief Executive Officer of S.R. One, Limited (from January 2019 to September 2020), initially joining as an associate in September 2007. From 2006 to 2007, Dr. George was a consultant at Bain & Company, and in 2004 he was an investment banker at Goldman Sachs. Dr. George currently serves on the boards of directors of the following public companies: CRISPR Therapeutics (since April 2015), Turning Point Therapeutics, Inc. (since May 2017) and Nkarta (since February 2020 and previously from July 2015 to September 2017). Dr. George also served on the boards of directors of Principia Biopharma Inc. (from February 2011 through its acquisition in September 2020), Progyny (from May 2012 until Oct 2019), HTG Molecular Diagnostics, Inc., from June 2011 until October 2015, and Genocea Biosciences, Inc., from February 2009 to December 2014. Dr. George received his B.A. in Neuroscience from the Johns Hopkins University, where he graduated Phi Beta Kappa. He received his M.D. from the University of Pennsylvania School of Medicine and his M.B.A. (Mayer Scholar) from the Wharton School of the University of Pennsylvania. We believe that Dr. George is qualified to serve on our board of directors due to his experience in the life sciences industry and the venture capital industry, and his leadership and management experience.

Stella Xu, Ph.D. has served as a member of our board of directors since March 2020. Dr. Xu has served as Managing Director of Quan Capital since September 2017. From September 2012 to August 2017, Dr. Xu served as Vice President and site head of Roche Innovation Center Shanghai, and a member of the global management team for Roche's Immunology, Inflammation & Infectious Diseases Discovery and Translation Area. Dr. Xu has served as member of the board of directors for Centrexion Therapeutics Corporation since January 2018, Temptest Therapeutics Inc. since March 2018, Zidan Medical, Inc. since October 2018, NextCure, Inc. since November 2018, Walking Fish Therapeutics, Inc. since June 2019, and HBM Heathcare Investments AG since June 2020. She also previously served as a member of the board of directors for ARMO BioSciences, Inc. from August 2017 to July 2018 (acquired by Eli Lilly and Company). Dr. Xu received a B.S. in Biophysics from Peking University and a Ph.D. in Immunology from Northwestern University. We believe that Dr. Xu is qualified to serve on our board of directors due to her extensive, global experience in the development and commercialization of innovative therapies.

Rodney Lappe, Ph.D. has served as a member of our board of directors since July 2019. From June 2012 to February 2019, Dr. Lappe served as Executive Chairman, Chairman and a member of the board of directors for Mirati Therapeutics, Inc. Since January 2012, Dr. Lappe has served as the Senior Vice President of Tavistock Life Sciences Co., a private investment firm. From January 2004 to December 2011, Dr. Lappe was Group Senior Vice President, Pfizer Worldwide Research and Development and Chief Scientific Officer for CovX Pharmaceuticals Inc. (CovX). Dr. Lappe joined

Pfizer with the CovX acquisition in January 2008. From August 2000 to December 2003, Dr. Lappe served as Vice President for cardiovascular and metabolic diseases at Pharmacia Group. He was also site leader for Pharmacia in St. Louis. Prior to joining Pharmacia, he held positions of increasing responsibility with Wyeth Pharmaceuticals, Rorer Central Research, CIBA Geigy and Searle Pharmaceuticals. Dr. Lappe received his B.A. from Blackburn College and his Ph.D. in Pharmacology from Indiana University. We believe Dr. Lappe is qualified to serve on our board of directors due to his extensive experience managing pharmaceutical and biotechnology companies.

John Schmid has served as a member of our board of directors since November 2020. Mr. Schmid has served as a member of the boards of directors of AnaptysBio, Inc. since June 2015, Neos Therapeutics since June 2015, Forge Therapeutics, Inc. since May 2017, Poseida Therapeutics Inc. since July 2018, Xeris Pharmaceuticals since September 2017, and Helix Acquisition Corporation since October 2020. In addition, Mr. Schmid serves as chairman of the board of directors of Speak, Inc., a speakers bureau, which he helped found in 1989. From May 2016 to August 2018, Mr. Schmid served as a member of the board of directors of Patara Pharma, Inc. From September 2013 to June 2015, Mr. Schmid served as Chief Financial Officer of Auspex Pharmaceuticals, Inc. until its sale to Teva Pharmaceutical Industries Ltd. From June 2004 to September 2013, Mr. Schmid co-founded Trius Therapeutics where he served as the Chief Financial Officer until its merger with Cubist Pharmaceuticals, Inc. From 1998 to 2003, Mr. Schmid served as the Chief Financial Officer of GeneFormatics, Inc. From 1995 to 1998, Mr. Schmid served as the Chief Financial Officer of Endonetics Inc. Mr. Schmid received a B.A. in Economics from Wesleyan University and an M.B.A. from the University of San Diego. We believe Mr. Schmid is qualified to serve on our board of directors due to his extensive financial experience and leadership positions at multiple biopharmaceutical companies.

Arsani William, M.D. has served as a member of our board of directors since January 2021. Dr. William has served as Managing Partner and Chief Investment Officer of Logos Capital since its founding in August 2019. Prior to founding Logos, Dr. William served as an investment professional at Farallon Capital Management from September 2016 to January 2019 where he helped grow the development of Farallon's biopharma portfolio. Dr. William holds an M.D. from Harvard Medical School where he was a Gerald S. Foster Scholar, an MBA from Stanford's Graduate School of Business, and a BS with Honors in Biology from Stanford University. We believe that Dr. William is qualified to serve on our board of directors due to his experience in the life sciences industry and the venture capital industry, and his leadership and management experience.

Family Relationships and Other Arrangements

Pursuant to our voting agreement, which will terminate upon the closing of this offering, the following directors were designated as directors to our board of directors:

- Dr. Shah was designated by the holders of a majority of the shares of our common stock.
- Dr. George was designated by S.R. One, Limited and elected by the holders of a majority of the shares of our Series A convertible preferred stock.
- Dr. Xu was designated by Quan Venture Fund II, L.P. and elected by the holders of a majority of the shares of our Series A convertible preferred stock.
- Dr. William was designated by Logos Opportunities Fund II, L.P. and Logos SPV 1 LP and elected by the holders of a majority of the shares of our Series B convertible preferred stock.

Board Composition

Our board of directors currently consists of seven members with no vacancies. In accordance with our amended and restated certificate of incorporation, which will be effective immediately prior to

the closing of this offering, our board of directors will be divided into three classes with staggered three-year terms. At each annual meeting of stockholders, the successors to the directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- The Class I directors will be Rodney Lappe and John Schmid, and their terms will expire at the annual meeting of stockholders to be held in 2022;
- The Class II directors will be Simeon George and Arsani William, and their terms will expire at the annual meeting of stockholders to be held in 2023; and
- The Class III directors will be Stella Xu, Pratik Shah and João Siffert, and their terms will expire at the annual meeting of stockholders to be held in 2024.

We expect that any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our management or a change in control.

Under the Nasdaq Stock Market LLC (Nasdaq), Marketplace Rules (the Nasdaq Listing Rules), independent directors must comprise a majority of our board of directors as a public company within 12 months of listing.

Our board of directors has undertaken a review of its composition, the composition of its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that all of our directors other than Drs. Shah and Siffert are independent directors, as defined by Rule 5605(a)(2) of the Nasdaq Listing Rules.

Board Committees

Our board of directors has established an audit committee, a compensation committee, a nominating and corporate governance committee and a research and development committee. Our board of directors may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below. Members serve on these committees until their resignation or until otherwise determined by our board of directors. Each committee intends to adopt a written charter that satisfies the applicable rules and regulations of the SEC and Nasdaq Listing Rules, which we will post on our website at www.designtx.com upon the closing of this offering.

Audit Committee

Our audit committee consists of John Schmid, Stella Xu and Arsani William. Our board of directors has determined that each of the members of our audit committee satisfies the Nasdaq Stock Market and SEC independence requirements. John Schmid serves as the chair of our audit committee. The functions of this committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;

- monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;
- reviewing our annual and quarterly financial statements and reports, including the disclosures contained under the caption "Management's Discussion and Analysis of Financial Condition and Results of Operations," and discussing the statements and reports with our independent auditors and management;
- reviewing, with our independent auditors and management, significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our independent auditors any earnings announcements and other public announcements regarding material developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- preparing the report that the SEC requires in our annual proxy statement;
- reviewing and providing oversight of any related-person transactions in accordance with our related person transaction policy and reviewing and monitoring compliance with legal and regulatory responsibilities, including our code of business conduct and ethics;
- reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management are implemented;
- reviewing on a periodic basis our investment policy; and
- reviewing and evaluating on an annual basis the performance of the audit committee and the audit committee charter.

Our board of directors has determined that Mr. Schmid qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the Nasdaq Listing Rules. In making this determination, our board has considered Mr. Schmid ‘s prior experience, business acumen and independence. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation Committee

Our compensation committee consists of Arsani William, Simeon George and John Schmid. Arsani William serves as the chair of our compensation committee. Our board of directors has determined that each of the members of our compensation committee satisfies the Nasdaq Stock Market independence requirements. The functions of this committee include, among other things:

- reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;

- reviewing and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) the compensation and other terms of employment of our executive officers;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;
- reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation as required by Section 14A of the Exchange Act and determining our recommendations regarding the frequency of advisory votes on executive compensation, to the extent required by law;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;
- administering our equity incentive plans;
- establishing policies with respect to equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing with management and approving our disclosures under the caption “Compensation Discussion and Analysis” in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- preparing the report that the SEC requires in our annual proxy statement (if applicable); and
- reviewing and assessing on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee consists of Rodney Lappe and Simeon George. Our board of directors has determined that each of the members of this committee satisfies the Nasdaq Stock Market independence requirements. Rodney Lappe serves as the chair of our

nominating and corporate governance committee. The functions of this committee include, among other things:

- identifying, reviewing and evaluating candidates to serve on our board of directors consistent with criteria approved by our board of directors;
- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- evaluating, nominating and recommending individuals for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles, including a code of business conduct and ethics, periodically reviewing and assessing these policies and principles and their application and recommending to our board of directors any changes to such policies and principles;
- considering questions of possible conflicts of interest of directors as such questions arise; and
- reviewing and assessing on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Research and Development Committee

Our research and development committee consists of Rodney Lappe and Stella Xu. Rodney Lappe serves as the chair of our research and development committee. The functions of this committee include, among other things:

- reviewing our current and planned research and development (R&D) and associated business development programs and initiatives and providing feedback to our management team on such programs and initiatives from a scientific perspective;
- (i) advising our board of directors regarding the quality, direction and competitiveness of our R&D programs and initiatives, (ii) providing strategic recommendations on our R&D and associated business development programs and initiatives to our board of directors and (iii) evaluating our progress in achieving long-term strategic R&D goals and objectives;
- recommending to our management team, as requested, experts to provide strategic and technical advice;
- providing a general oversight function regarding our R&D organizations and personnel and making regular reports to our board of directors, as appropriate;
- reviewing new and emerging trends in health care; science and technology to assist our management team in making well informed choices in the allocation of our R&D resources; and
- reviewing and assessing on an annual basis the performance of the research and development committee and the research and development charter.

Compensation Committee Interlocks and Insider Participation

None of the members of the compensation committee is currently, or has been at any time, one of our executive officers or employees. None of our executive officers currently serves, or has served during the last year, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving as a member of our board of directors or on our compensation committee.

Code of Business Conduct and Ethics

In connection with this offering, we intend to adopt a written code of business conduct and ethics that applies to all of our directors, officers and employees, including our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions, and agents and representatives. The full text of our code of business conduct and ethics will be posted on our website at www.designtx.com upon the closing of this offering. The nominating and corporate governance committee of our board of directors will be responsible for overseeing our code of business conduct and ethics and any waivers applicable to any director, executive officer or employee. We intend to disclose future amendments to certain provisions of our code of business conduct and ethics, or waivers of such provisions applicable to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions, and agents and representatives, on our website identified above.

Limitation on Liability and Indemnification Matters

Our amended and restated certificate of incorporation, which will become effective immediately prior to the closing of this offering, and our amended and restated bylaws, which will become effective upon the closing of this offering, limits our directors' liability, and may indemnify our directors and officers to the fullest extent permitted under Delaware General Corporation Law (DGCL). The DGCL provides that directors of a corporation will not be personally liable for monetary damages for breach of their fiduciary duties as directors, except for liability for any:

- transaction from which the director derives an improper personal benefit;
- act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payment of dividends or redemption of shares; or
- breach of a director's duty of loyalty to the corporation or its stockholders.

These limitations of liability do not apply to liabilities arising under federal securities laws and do not affect the availability of equitable remedies such as injunctive relief or recession.

The DGCL and our amended and restated bylaws provide that we will, in certain situations, indemnify our directors and officers and may indemnify other employees and other agents, to the fullest extent permitted by law. Any indemnified person is also entitled, subject to certain limitations, to advancement, direct payment or reimbursement of reasonable expenses (including attorneys' fees and disbursements) in advance of the final disposition of the proceeding.

In addition, we have entered, and intend to continue to enter, into separate indemnification agreements with some of our directors and officers. These indemnification agreements, among other things, require us to indemnify our directors and officers for certain expenses, including attorneys' fees,

judgments, fines and settlement amounts incurred by a director or officer in any action or proceeding arising out of their services as a director or officer, or any other company or enterprise to which the person provides services at our request.

We maintain a directors' and officers' insurance policy pursuant to which our directors and officers are insured against liability for actions taken in their capacities as directors and officers. We believe that these provisions in our amended and restated certificate of incorporation and amended and restated bylaws and these indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

Insofar as indemnification for liabilities arising under the Securities Act, may be permitted to directors, officers or control persons, in the opinion of the SEC, such indemnification is against public policy, as expressed in the Securities Act and is therefore unenforceable.

EXECUTIVE AND DIRECTOR COMPENSATION

Executive Compensation

Our named executive officers for the year ended December 31, 2020, consisting of our current and former principal executive officer and our only other executive officer, were:

- Pratik Shah, Ph.D., our Executive Chairperson(1);
- João Siffert, M.D., our President and Chief Executive Officer(2); and
- Sean Jeffries, Ph.D., our Chief Operating Officer(3).

(1) Dr. Shah served as our principal executive officer until October 2020 when Dr. Siffert commenced employment with us as our Chief Executive Officer.
(2) Dr. Siffert has served as our Chief Executive Officer since October 2020 and as our President since January 2021.
(3) Dr. Jeffries has served as our Chief Operating Officer since January 2021 and as our Chief Business Officer from May 2019 to January 2021.

Summary Compensation Table

The following table presents all of the compensation awarded to or earned by or paid to our named executive officers during the fiscal year ended December 31, 2020.

Name and Principal Position	Fiscal Year	Salary ($)	Bonus ($)	Option Awards ($)(1)	Non-Equity Incentive Plan Compensation ($)(2)	All Other Compensation ($)	Total ($)
Pratik Shah, Ph.D.							
Executive Chairperson	2020	250,000	—	—	150,000	—	400,000
João Siffert, M.D.							
President and Chief Executive Officer	2020	122,917	—	4,234,426	—	—	4,357,343
Sean Jeffries, Ph.D.							
Chief Operating Officer.	2020	243,333	—	—	100,000	—	343,333

(1) The amount disclosed represents the aggregate grant date fair value of the stock option granted to Dr. Siffert during fiscal year 2020 under our 2018 Equity Incentive Plan, computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the stock option are set forth in Note 10 to our audited financial statements included elsewhere in this prospectus. This amount does not reflect the actual economic value that may be realized by the named executive officer.
(2) Amounts represent the applicable named executive officer's performance bonus earned for 2020, as described below under "—Non-Equity Incentive Plan Compensation."

Annual Base Salary

The 2020 annual base salaries for our named executive officers are set forth in the table below.

Name	2020 Base Salary
Pratik Shah, Ph.D.(1)	$ 300,000
João Siffert, M.D.(2)	$ 550,000
Sean Jeffries, Ph.D.(3)	$ 270,000

(1) Dr. Shah began his employment with us on March 1, 2020 with an annualized base salary of $300,000.
(2) Dr. Siffert began his employment with us on October 12, 2020 with an annualized base salary of $550,000.
(3) Dr. Jeffries became a full-time employee effective March 1, 2020 with an annualized base salary of $270,000. Previously, Dr. Jeffries was employed part-time and had an annualized base salary of $110,000.

In January 2021, our board of directors approved annual base salaries, to be effective January 1, 2021, of $310,030 for Dr. Shah and $340,000 for Dr. Jeffries. For 2021, Dr. Siffert's annual base salary remains unchanged from 2020.

Non-Equity Incentive Plan Compensation

We seek to motivate and reward our executives for achievements relative to our corporate goals and expectations for each fiscal year. In 2020, Drs. Shah and Jeffries were eligible to receive an annual performance bonus based on the achievement of performance goals as determined by our board of directors or an authorized committee thereof. For 2020, these goals included product candidate development and operating objectives. Dr. Shah was assigned a target bonus equal to 50% of his annual base salary based on his employment agreement. In January 2021, our board of directors determined that the 2020 corporate goals were achieved at 100% and, as a result, approved an annual performance bonus for Dr. Shah for 2020 of $150,000, and approved a performance bonus for Dr. Jeffries of $100,000, in each case, as reflected in the "Non-Equity Incentive Plan Compensation" column of the Summary Compensation Table above. For purposes of the bonus determination, our board of directors used Dr. Shah's 2020 annualized base salary.

Equity-Based Incentive Awards

Our equity-based incentive awards are designed to align our interests and those of our stockholders with those of our employees and consultants, including our executive officers. Our board of directors or an authorized committee thereof is responsible for approving equity grants.

We have generally used stock options and restricted stock awards as an incentive for long-term compensation to our executive officers because stock options allow our executive officers to realize value from this form of equity compensation only if our stock price increases, and restricted stock awards align the interests of our executive officers with the interests of our stockholders generally. Certain stock options that we have granted to our executive officers permit "early exercise," whereby the executive officer can purchase shares subject to the stock option prior to vesting, subject to our right of repurchase which lapses in accordance with the vesting schedule of the stock option. Similarly, common stock issued pursuant to restricted stock awards is subject to our right of repurchase which lapses in accordance with the vesting schedule of the restricted stock award.

We may grant equity awards at such times as our board of directors determines appropriate. Our executives generally are awarded an initial grant in the form of a stock option or in the case of Dr. Jeffries, a restricted stock award, in connection with their commencement of employment with us. Additional grants may occur periodically in order to specifically incentivize executives with respect to achieving certain corporate goals or to reward executives for exceptional performance.

Prior to this offering, we granted stock options to Dr. Siffert and restricted stock awards to Dr. Jeffries pursuant to our 2018 Equity Incentive Plan, or 2018 Plan, the terms of which are described below under the subsection titled "—Employee Benefit Plans—2018 Equity Incentive Plan." Following the completion of this offering, we may grant additional equity awards to our named executive officers pursuant to our 2021 Plan, the terms of which are described below under the subsection titled "—Employee Benefit Plans—2021 Equity Incentive Plan." In October 2020, our board of directors granted options under our 2018 Plan to purchase 766,870 shares to Dr. Siffert. The option has an

exercise price of $0.95 per share and vests with respect to 25% of the shares subject to the option on the one year anniversary of Dr. Siffert's employment start date and the balance of the shares in a series of 36 successive equal monthly installments thereafter, subject to the executive's continued services to us and potential acceleration of vesting in connection with a change of control, as described below under "—Outstanding Equity Awards at Fiscal Year-End" and "—Potential Payments and Benefits upon Termination or Change in Control." Prior to the vesting date, Dr. Siffert exercised his option grant with respect to 122,699 shares and, upon such exercise, received restricted shares that are subject to repurchase by us at the lower of the fair market value or original purchase price per share of $0.95 pursuant to the terms of the award agreements. Such repurchase right will lapse on the same vesting schedule as the stock option which was exercised.

All stock options are granted with an exercise price per share that is no less than the fair market value of our common stock on the date of grant of such award. Our stock option awards generally vest over a four-year period and may be subject to acceleration of vesting and exercisability under certain termination and change in control events, as described in more detail under the subsections titled "—Potential Payments and Benefits upon Termination or Change in Control" and "—Equity Benefit Plans."

Outstanding Equity Awards as of December 31, 2020

The following table presents the outstanding equity incentive plan awards held by each named executive officer as of December 31, 2020.

	Option Awards[1]					Stock Awards[1]	
Name	Grant Date	Number of Securities Underlying Unexercised Options Exercisable (#)	Number of Securities Underlying Unexercised Options Unexercisable (#)	Option Exercise Price Per Share ($)[2]	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested ($)[3]
Pratik Shah, Ph.D.	—	—	—	—	—	—	—
João Siffert, M.D.	10/29/2020	644,171[4]	—	$ 0.95	10/28/2030	—	—
Sean Jeffries, Ph.D.	6/13/2018	—	—	—	—	302,713[5]	$1,873,793

(1) All of the option and stock awards were granted under the 2018 Plan, the terms of which plan is described below under "—Employee Benefit and Stock Plans—2018 Equity Incentive Plan."
(2) All of the option awards were granted with a per share exercise price equal to the fair market value of one share of our common stock on the date of grant, as determined in good faith by our board of directors or compensation committee.
(3) This column represents the fair market value of a share of our common stock of $6.19 as of December 31, 2020 (the determination of the fair market value by our board of directors as of the most proximate date) multiplied by the amount shown in the column "Stock Awards—Number of Shares or Units of Stock That Have Not Vested (#)".
(4) This option vests over four years from October 12, 2020, with 1/4 vesting on the first anniversary of the vesting commencement date, and the remainder vesting in 36 equal monthly installments, subject to continued service through each such vesting date. In December 2020, Dr. Siffert early exercised 122,699 shares with respect to this option.
(5) The shares are subject to a right of repurchase by the Company of which 1/4 lapses on the first anniversary of June 13, 2018, and the remainder lapses in 36 equal monthly installments, subject to continued service through each such date.

Options held by certain of our named executive officers are eligible for accelerated vesting under specified circumstances. Please see the subsection titled "—Employment Agreements" below for a description of such potential acceleration.

Emerging Growth Company Status

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act. As an emerging growth company we will be exempt from certain requirements related to executive

compensation, including the requirements to hold a nonbinding advisory vote on executive compensation and to provide information relating to the ratio of total compensation of our Chief Executive Officer to the median of the annual total compensation of all of our employees, each as required by the Investor Protection and Securities Reform Act of 2010, which is part of the Dodd-Frank Act.

Nonqualified Deferred Compensation

We do not maintain nonqualified defined contribution plans or other nonqualified deferred compensation plans. Our board of directors may elect to provide our officers and other employees with nonqualified defined contribution or other nonqualified deferred compensation benefits in the future if it determines that doing so is in our best interests.

Employment, Severance and Change in Control Agreements

Employment Agreements

Below are descriptions of our employment offer letters with our named executive officers. The employment of each of our named executive officers is at will. For a discussion of the severance pay and other benefits to be provided in connection with a termination of employment and/or a change in control under the arrangements with our named executive officers, see the subsection titled "—Potential Payments upon Termination or Change in Control" below.

Pratik Shah, Ph.D. We entered into an offer letter with Dr. Shah in March 2020, which governs the current terms of his employment with us. Pursuant to the agreement, Dr. Shah is entitled to an initial annual base salary of $300,000 and is eligible to receive an annual performance bonus of up to 50% of his annual base salary, based upon the achievement of certain corporate and individual objectives as determined by our board of directors. Dr. Shah's agreement also provides for severance benefits upon an involuntary termination, as described below under "—Potential Payments upon Termination or Change in Control." Additionally, pursuant to his offer letter, Dr. Shah is also entitled to a full gross-up of any excise taxes incurred by Dr. Shah in connection with a change in control.

João Siffert, M.D. We entered into an offer letter with Dr. Siffert in September 2020, which governs the current terms of Dr. Siffert's employment with us. Pursuant to the agreement, Dr. Siffert is entitled to (i) an initial annual base salary of $550,000, and (ii) is eligible to receive an annual performance bonus of up to 50% of his annual base salary, based upon the achievement of certain corporate and individual objectives as determined by our board of directors. Conditioned on his continued employment with the company through October 12, 2021, Dr. Siffert became entitled to receive a $150,000 one-time sign-on bonus, with an initial payment of $50,000 made in October 2020, and the remaining $100,000 to be paid before March 31, 2021. Dr. Siffert's sign-on bonus is subject to repayment if his employment with us ceases under certain circumstances within 12 months of his start date. In addition, pursuant the employment offer letter, Dr. Siffert was granted an option to purchase 766,870 shares of our common stock in October 2020, as further described above under "—Equity-Based Incentive Awards." Dr. Siffert's offer letter also provides for severance benefits upon an involuntary termination, as described below under "—Potential Payments upon Termination or Change in Control."

Sean Jeffries, Ph.D. We entered into a letter agreement with Dr. Jeffries in May 2019, which governs the current terms of Dr. Jeffries' employment with us. Pursuant to the agreement, Dr. Jeffries is entitled to an annual salary of $270,000.

Potential Payments Upon Termination or Change in Control

Regardless of the manner in which a named executive officer's service terminates, each named executive officer is entitled to receive amounts earned during his term of service, including unpaid salary and unused paid time off, as applicable. In addition, Drs. Shah and Siffert are entitled to certain severance benefits under their employment offer letters, subject to their execution of a release of claims, return of all company property, compliance with post-termination obligations and resignation from all positions with us.

Dr. Shah's offer letter provides that, if his employment is terminated by us without "cause" (other than as a result of death or disability) or Dr. Shah resigns for "good reason" (each, as defined in Dr. Shah's offer letter), he will be entitled to receive continued payment of his then-current base salary for 12 months. In addition, we will be required to pay the premiums for Dr. Shah's COBRA continuation health coverage for up to 12 months. Dr. Shah is also entitled to a full gross-up of any excise taxes incurred by Dr. Shah in connection with a change in control.

Dr. Siffert's offer letter provides that if his employment is terminated by us without "cause" (other than as a result of death or disability) or Dr. Siffert resigns for "good reason" (each, as defined in Dr. Siffert's offer letter), he will be entitled to receive (i) continued payment of his then-current base salary for 12 months, (ii) a pro-rata portion of his annual bonus target for the year in which his involuntary termination occurs, (iii) premiums for Dr. Siffert's COBRA continuation health coverage for up to 12 months. In addition, if such termination or resignation occurs within 12 months immediately following the consummation of a change in control (as defined in the 2018 Plan), all of the outstanding and unvested stock options granted in October 2020 will become fully vested and immediately exercisable.

Our named executive officers' stock options and restricted stock granted prior to execution of the underwriting agreement for this offering are subject to the terms of the 2018 Plan; a description of the termination and change in control provisions in the 2018 Plan and the form of stock options granted thereunder is provided below under "—Employee Benefit Plans."

Other Compensation and Benefits

All of our named executive officers are eligible to participate in our employee benefit plans, including our medical, dental, vision and life insurance plans, in each case on the same basis as all of our other employees. We pay the premiums for the life, disability, accidental death and dismemberment insurance for all of our employees, including our named executive officers. We generally do not provide perquisites or personal benefits to our named executive officers, except in limited circumstances.

Employee Benefit Plans

We believe that our ability to grant equity-based awards is a valuable and necessary compensation tool that aligns the long-term financial interests of our employees, consultants and directors with the financial interests of our stockholders. In addition, we believe that our ability to grant stock options and other equity-based awards helps us to attract, retain and motivate employees, consultants and directors, and encourages them to devote their best efforts to our business and financial success. The principal features of our equity incentive plans and our 401(k) plan are summarized below. These summaries are qualified in their entirety by reference to the actual text of the plans, which, other than the 401(k) plan, are filed as exhibits to the registration statement of which this prospectus is a part.

2021 Equity Incentive Plan

Our board of directors adopted, and our stockholders approved, our 2021 Plan in March 2021. Our 2021 Plan provides for the grant of incentive stock options (ISOs) to employees, including employees of any parent or subsidiary, and for the grant of nonstatutory stock options (NSOs), stock appreciation rights, restricted stock awards, restricted stock unit awards, performance awards and other forms of stock awards to employees, directors, and consultants, including employees and consultants of our affiliates. Our 2021 Plan is a successor to and continuation of our 2018 Plan (referred to in the 2021 Plan as our Prior Plan) and became effective on the execution of the underwriting agreement related to this offering.

Authorized Shares. Initially, the maximum number of shares of our common stock that may be issued under our 2021 Plan is 9,381,505 shares, which is the sum of (i) 6,118,648 new shares; plus (ii) 881,352 the number of shares that were available for issuance under our 2018 Plan at the time our 2021 Plan became effective; and (iii) any shares subject to outstanding stock options or other stock awards that were granted under our 2018 Plan that are forfeited, terminate, expire or are otherwise not issued. In addition, the number of shares of our common stock reserved for issuance under our 2021 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2022 through January 1, 2031, in an amount equal to 5% of the total number of shares of our capital stock outstanding on the last day of the calendar month before the date of each automatic increase, or a lesser number of shares determined by our board of directors. The maximum number of shares of our common stock that may be issued on the exercise of incentive stock options under our 2021 Plan is 28,144,515.

Shares subject to stock awards granted under our 2021 Plan that expire or terminate without being exercised in full, or that are paid out in cash rather than in shares, do not reduce the number of shares available for issuance under our 2021 Plan. Additionally, shares become available for future grant under our 2021 Plan if they were issued under stock awards under our 2021 Plan if we repurchase them or they are forfeited. This includes shares used to pay the exercise price of a stock award or to satisfy the tax withholding obligations related to a stock award.

Plan Administration. Our board of directors, or a duly authorized committee of our board of directors, will administer our 2021 Plan. Our board of directors may also delegate to one or more of our officers the authority to (i) designate employees (other than officers) to receive specified stock awards and (ii) determine the number of shares subject to such stock awards. Under our 2021 Plan, our board of directors has the authority to determine and amend the terms of awards and underlying agreements, including:

- recipients;
- the exercise, purchase or strike price of stock awards, if any; the number of shares subject to each stock award;
- the vesting schedule applicable to the awards, together with any vesting acceleration; and
- the form of consideration, if any, payable on exercise or settlement of the award.

Under the 2021 Plan, the board of directors also generally has the authority to effect, with the consent of any adversely affected participant:

- the reduction of the exercise, purchase, or strike price of any outstanding award;
- the cancellation of any outstanding award and the grant in substitution therefore of other awards, cash, or other consideration; or
- any other action that is treated as a repricing under generally accepted accounting principles.

Stock Options. ISOs and NSOs are granted under stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for stock options, within the terms and conditions of the 2021 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2021 Plan vest at the rate specified in the stock option agreement as determined by the plan administrator.

Tax Limitations on ISOs. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (i) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant; and (ii) the option is not exercisable after the expiration of five years from the date of grant.

Restricted Stock Unit Awards. Restricted stock units are granted under restricted stock unit award agreements adopted by the plan administrator. Restricted stock units may be granted in consideration for any form of legal consideration that may be acceptable to our board of directors and permissible under applicable law. A restricted stock unit may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited once the participant's continuous service ends for any reason.

Restricted Stock Awards. Restricted stock awards are granted under restricted stock award agreements adopted by the plan administrator. A restricted stock award may be awarded in consideration for cash, check, bank draft or money order, past services to us, or any other form of legal consideration that may be acceptable to our board of directors and permissible under applicable law. The plan administrator determines the terms and conditions of restricted stock awards, including vesting and forfeiture terms. If a participant's service relationship with us ends for any reason, we may receive any or all of the shares of common stock held by the participant that have not vested as of the date the participant terminates service with us through a forfeiture condition or a repurchase right.

Stock Appreciation Rights. Stock appreciation rights are granted under stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the purchase price or strike price for a stock appreciation right, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. A stock appreciation right granted under the 2021 Plan vests at the rate specified in the stock appreciation right agreement as determined by the plan administrator.

Performance Awards. The 2021 Plan permits the grant of performance-based stock and cash awards. The plan administrator may structure awards so that the shares of our stock, cash, or other property will be issued or paid only following the achievement of certain pre-established performance goals during a designated performance period. The performance criteria that will be used to establish such performance goals may be based on any measure of performance selected by the plan administrator. The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (i) in the award agreement at the time the award is granted or (ii) in such other document setting forth the performance goals at the time the goals are established, we will

appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of items that are "unusual" in nature or occur "infrequently" as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; and (12) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the FDA or any other regulatory body. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

Other Stock Awards. The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

Non-Employee Director Compensation Limit. The aggregate value of all compensation granted or paid to any non-employee director with respect to any period commencing on the date of our annual meeting of stockholders for a particular year and ending on the day immediately prior to the date of the meeting for the next subsequent year, including stock awards granted and cash fees paid by us to such non-employee director, will not exceed $750,000 in total value, or in the event such non-employee director is first appointed or elected to the board during such annual period, $1,000,000 in total value (in each case, calculating the value of any such stock awards based on the grant date fair value of such stock awards for financial reporting purposes).

Changes to Capital Structure. In the event there is a specified type of change in our capital structure, such as a stock split, reverse stock split, or recapitalization, appropriate adjustments will be made to (i) the class and maximum number of shares reserved for issuance under the 2021 Plan, (ii) the class and maximum number of shares by which the share reserve may increase automatically each year, (iii) the class and maximum number of shares that may be issued on the exercise of incentive stock options, and (iv) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

Corporate Transactions. The following applies to stock awards under the 2021 Plan in the event of a corporate transaction, unless otherwise provided in a participant's stock award agreement or other written agreement with us or one of our affiliates or unless otherwise expressly provided by the plan administrator at the time of grant.

In the event of a corporate transaction, any stock awards outstanding under the 2021 Plan may be assumed, continued or substituted for by any surviving or acquiring corporation (or its parent company), and any reacquisition or repurchase rights held by us with respect to the stock award may be assigned to the successor (or its parent company). If the surviving or acquiring corporation (or its parent company) does not assume, continue or substitute for such stock awards, then with respect to any such stock awards that are held by participants whose continuous service has not terminated prior

to the effective time of the transaction, or current participants, the vesting (and exercisability, if applicable) of such stock awards will be accelerated in full to a date prior to the effective time of the transaction (contingent upon the effectiveness of the transaction), and such stock awards will terminate if not exercised (if applicable) at or prior to the effective time of the transaction, and any reacquisition or repurchase rights held by us with respect to such stock awards will lapse (contingent upon the effectiveness of the transaction). With respect to performance awards with multiple vesting levels depending on performance level, unless otherwise provided by an award agreement or by the administrator, the award will accelerate at 100% of target. If the surviving or acquiring corporation (or its parent company) does not assume, continue or substitute for such stock awards, then with respect to any such stock awards that are held by persons other than current participants, such awards will terminate if not exercised (if applicable) prior to the effective time of the transaction, except that any reacquisition or repurchase rights held by us with respect to such stock awards will not terminate and may continue to be exercised notwithstanding the transaction. The plan administrator is not obligated to treat all stock awards or portions of stock awards in the same manner and is not obligated to take the same actions with respect to all participants.

In the event a stock award will terminate if not exercised prior to the effective time of a transaction, the plan administrator may provide, in its sole discretion, that the holder of such stock award may not exercise such stock award but instead will receive a payment equal in value to the excess (if any) of (i) the value of the property the participant would have received upon the exercise of the stock award over (ii) any exercise price payable by such holder in connection with such exercise.

Change in Control. In the event of a change in control, as defined under our 2021 Plan, awards granted under our 2021 Plan will not receive automatic acceleration of vesting and exercisability, although this treatment may be provided for in an award agreement.

Under our 2021 Plan, a corporate transaction is defined to include: (i) a sale of all or substantially all of our assets; (ii) the sale or disposition of more than 50% of our outstanding securities; (iii) the consummation of a merger or consolidation where we do not survive the transaction; and (iv) the consummation of a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding before such transaction are converted or exchanged into other property by virtue of the transaction, unless otherwise provided in an award agreement or other written agreement between us and the award holder. Under the 2021 Plan, a change in control is defined to include (1) the acquisition by any person or company of more than 50% of the combined voting power of our then outstanding stock; (2) a merger, consolidation or similar transaction in which our stockholders immediately before the transaction do not own, directly or indirectly, more than 50% of the combined voting power of the surviving entity (or the parent of the surviving entity); (3) the approval by the stockholders or the board of directors of a plan of complete dissolution or liquidation of the company, or the occurrence of a complete dissolution or liquidation of the company, except for a liquidation into a parent corporation; (4) a sale, lease, exclusive license or other disposition of all or substantially all of our assets other than to an entity more than 50% of the combined voting power of which is owned by our stockholders; and (5) an unapproved change in the majority of the board of directors.

Transferability. A participant may not transfer stock awards under our 2021 Plan other than by will, the laws of descent and distribution, or as otherwise provided under our 2021 Plan.

Plan Amendment or Termination. Our board of directors has the authority to amend, suspend, or terminate our 2021 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. Certain material amendments also require the approval of our stockholders. No incentive stock options may be granted after the tenth anniversary of the date our board of directors adopted our 2021 Plan. No stock awards may be granted under our 2021 Plan while it is suspended or after it is terminated.

2018 Equity Incentive Plan

Our 2018 Plan was originally adopted by our board of directors and approved by our stockholders in June 2018 and was amended in October 2020. Our 2018 Plan allows for the grant of ISOs, NSOs, stock appreciation rights, restricted stock and restricted stock awards to employees, directors and consultants, including employees and consultants of our affiliates. Once our 2021 Plan becomes effective, no further grants will be made under our 2018 Plan. Any outstanding awards granted under our 2018 Plan will remain subject to the terms of our 2018 Plan and applicable award agreements.

Authorized Shares. The maximum number of shares of our common stock that may be issued under our 2018 Plan is 3,955,438 shares. Shares subject to stock awards granted under our 2018 Plan that expire, are forfeited, or terminate without being exercised in full do not reduce the number of shares available for issuance under our 2018 Plan. Additionally, shares used to pay the exercise price of a stock award or to satisfy the tax withholding obligations related to a stock award become available for future grant under our 2018 Plan.

Plan Administration. Our board of directors or a duly authorized committee of our board of directors (referred to herein as the plan administrator) administers our 2018 Plan and the stock awards granted under it. Under our 2018 Plan, the plan administrator has the authority to, among other things: (i) determine stock award recipients; (ii) determine the form and terms of the stock awards; (iii) determine the number of shares or other consideration subject to awards; (iv) determine the types of stock awards to be granted: (v) determine the fair market value of our common stock; (vi) construe and interpret the 2018 Plan and any agreement thereunder; (vii) amend the 2018 Plan in any respect the plan administrator deems necessary or advisable; (viii) settle all controversies regarding the 2018 Plan or any award; (viii) accelerate awards; (x) suspend or terminate the 2018 Plan at any time; and (xi) make all other determinations necessary or advisable for the administration of the 2018 Plan.

Under the 2018 Plan, the plan administrator also generally has the authority to effect, with the consent of any adversely affected participant, (i) the reduction of the exercise, purchase, or strike price of any outstanding award; (ii) the cancellation of any outstanding award and the grant in substitution therefor of other stock awards, cash, or other consideration; or (iii) any other action that is treated as a repricing under generally accepted accounting principles.

Stock Options. ISOs and NSOs are granted pursuant to stock award agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2018 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant (or 110% of the fair market value for certain major stockholders). Stock options granted under the 2018 Plan vest at the rate specified by the plan administrator. Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (i) cash, check, bank draft or money order payable to us; (ii) subject to a program developed under Regulation T (as promulgated by the Federal Reserve Board) that, prior to the issuance of the stock subject to the option, results in either the receipt of cash (or check) by us or the receipt of irrevocable instructions to pay the aggregate exercise price to us from the sales proceeds; (iii) by delivery to us of shares of common stock; (iv) by a cashless “net exercise” arrangement if the option is a NSO; (v) a deferred payment arrangement; or (vi) other legal consideration approved by the plan administrator. The plan administrator determines the term of stock options granted under the 2018 Plan, up to a maximum of 10 years (or five years in the case of certain major stockholders). The plan administrator shall determine the effect on a stock award of the disability, death, retirement, authorized leave of absence, or any other change or purported change in a holder’s status. Unless the plan administrator provides otherwise, stock options generally are not transferable except by will, the laws of descent and distribution.

Corporate Transactions. Our 2018 Plan provides that in the event of a "corporate transaction," unless otherwise provided in an award agreement or other written agreement between us and the award holder, the plan administrator may take one or more of the following actions with respect to such stock awards:

- arrange for the assumption, continuation, or substitution of a stock award by a surviving or acquiring corporation, or a parent or subsidiary thereof;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring corporation, or a parent or subsidiary thereof;
- accelerate the vesting, in whole or in part, of the stock award and provide for its termination if not exercised (if applicable) at or before the effective time of the transaction;
- arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by us;
- cancel or arrange for the cancellation of the stock award, to the extent not vested or not exercised before the effective time of the transaction, in exchange for such cash consideration (including no consideration) as our board of directors, in its sole discretion, may consider appropriate; and
- make a payment equal to the excess, if any, of (i) the value of the property the participant would have received on exercise of the award immediately before the effective time of the transaction, over (ii) any exercise price payable by the participant in connection with the exercise.

The plan administrator is not obligated to treat all stock awards or portions of stock awards in the same manner and is not obligated to treat all participants in the same manner. Under the 2018 Plan, a "corporate transaction" is generally defined as the consummation, in a single transaction or in a series of related transactions, of: (i) a sale of all or substantially all of our assets; (ii) the sale or disposition of more than 50% of our outstanding securities; (iii) a merger or consolidation where we do not survive the transaction; or (iv) a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately before such transaction are converted or exchanged into other property by virtue of the transaction.

Changes to Capital Structure. In the event of a "capitalization adjustment," the board of directors, in its discretion, will make appropriate and proportionate adjustments to (i) the class and maximum number of shares reserved for issuance under the 2018 Plan; (ii) the class and maximum number of shares that may be issued on the exercise of ISOs; and (iii) the class and number of shares and price per share subject to outstanding stock awards. For purposes of the 2018 Plan, "capitalization adjustment" generally means any change that is made in (or other events occurring with respect to) our common stock subject to the 2018 Plan or any award without the receipt of consideration by us through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure, or any similar equity restructuring transaction (within the meaning of Statement of Financial Accounting Standards Board ASC Topic 718).

Change in Control. A stock award may be subject to additional acceleration of vesting and exercisability upon or after a change in control as may be provided in an applicable award agreement or other written agreement, but in the absence of such provision, no such acceleration will occur**.** Under the 2018 Plan, a "change in control" is generally defined as (i) certain acquisitions by a person or company of more than 50% of the combined voting power of our then outstanding stock; (ii) a merger, consolidation or similar transaction in which, immediately after the consummation of such transaction, our stockholders as of immediately before the transaction do not own, directly or indirectly,

more than 50% of the combined voting power of the surviving entity (or the parent of the surviving entity) in substantially the same proportions as their ownership immediately prior to such transaction; or (iii) a sale, lease, exclusive license or other disposition of all or substantially all of our consolidated assets other than to an entity more than 50% of the combined voting power of which is owned by our stockholders in substantially the same proportions as their ownership of our outstanding voting securities immediately prior to such transaction.

Transferability. A participant may not transfer stock awards under our 2018 Plan other than by will, the laws of descent and distribution, or as otherwise provided under our 2018 Plan or an award granted thereunder.

Plan Amendment or Termination. Our board of directors has the authority to amend, suspend, or terminate our 2018 Plan; provided that no amendment of the 2018 Plan shall materially and adversely affect any outstanding stock award without the consent of the affected holder. Certain material amendments require the approval of our stockholders. Unless terminated sooner, the 2018 Plan will automatically terminate June 18, 2028. No stock awards may be granted under the 2018 Plan while it is suspended or after it is terminated.

2021 Employee Stock Purchase Plan

Our board of directors adopted, and our stockholders approved, our 2021 Employee Stock Purchase Plan (ESPP) in March 2021. The ESPP became effective immediately prior to and contingent upon the execution of the underwriting agreement for this offering. The purpose of the ESPP is to secure and retain the services of new employees, to retain the services of existing employees, and to provide incentives for such individuals to exert maximum efforts toward our success and that of our affiliates. The ESPP is intended to qualify as an “employee stock purchase plan” within the meaning of Section 423 of the Code for U.S. employees.

Share Reserve. Following this offering, the ESPP authorizes the issuance of 600,000 shares of our common stock under purchase rights granted to our employees or to employees of any of our designated affiliates. The number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, beginning on January 1, 2022 through January 1, 2031, by the lesser of (i) 1% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of the automatic increase; and (ii) 1,200,000 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). As of the date hereof, no shares of our common stock have been purchased under the ESPP.

Administration. Our board of directors, or a duly authorized committee thereof, will administer our ESPP. Our board may delegate concurrent authority to administer the ESPP to our compensation committee under the terms of the compensation committee’s charter. The ESPP is implemented through a series of offerings under which eligible employees are granted purchase rights to purchase shares of our common stock on specified dates during such offerings. Under the ESPP, we may specify offerings with durations of not more than 27 months and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering under the ESPP may be terminated under certain circumstances.

Payroll Deductions. Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings (as defined in the ESPP) for the purchase of our

common stock under the ESPP. Unless otherwise determined by our board of directors, common stock will be purchased for the accounts of employees participating in the ESPP at a price per share that is at least the lesser of (i) 85% of the fair market value of a share of our common stock on the first date of an offering; or (ii) 85% of the fair market value of a share of our common stock on the date of purchase.

Limitations. Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our board of directors, including: (i) customary employment with us or one of our affiliates for more than 20 hours per week and more than five months per calendar year; or (ii) continuous employment with us or one of our affiliates for a minimum period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of $25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value under Section 424(d) of the Code.

Changes to Capital Structure. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure, or similar transaction, the board of directors will make appropriate adjustments to: (i) the number of shares reserved under the ESPP; (ii) the maximum number of shares by which the share reserve may increase automatically each year; (iii) the number of shares and purchase price of all outstanding purchase rights; and (iv) the number of shares that are subject to purchase limits under ongoing offerings.

Corporate Transactions. In the event of certain significant corporate transactions, including: (i) a sale of all or substantially all of our assets; (ii) the sale or disposition of more than 50% of our outstanding securities; (iii) the consummation of a merger or consolidation where we do not survive the transaction; and (iv) the consummation of a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately before such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue, or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of our common stock within ten business days before such corporate transaction, and such purchase rights will terminate immediately.

ESPP Amendment or Termination. Our board of directors has the authority to amend or terminate our ESPP, provided that except in certain circumstances such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to our ESPP as required by applicable law or listing requirements.

401(k) Plan

We maintain a 401(k) plan that provides eligible U.S. employees with an opportunity to save for retirement on a tax advantaged basis. Eligible employees are able to defer eligible compensation up to certain Code limits, which are updated annually. We have the ability to make matching and discretionary contributions to the 401(k) plan. Currently, we do not make matching contributions or

discretionary contributions to the 401(k) plan. The 401(k) plan is intended to be qualified under Section 401(a) of the Code, with the related trust intended to be tax exempt under Section 501(a) of the Code. As a tax-qualified retirement plan, contributions to the 401(k) plan are deductible by us when made, and contributions and earnings on those amounts are not generally taxable to the employees until withdrawn or distributed from the 401(k) plan.

Non-Employee Director Compensation

The following table sets forth in summary form information concerning the compensation that we paid or awarded during the year ended December 31, 2020 to each of our non-employee directors who served on our board of directors during 2020:

Name	Fees Earned or Paid in Cash ($)	Option Awards ($)(1)(2)	Total ($)
Simeon George, M.D.	—	—	—
Stella Xu, Ph.D.	—	—	—
Rodney Lappe, Ph.D.	—	—	—
John Schmid	5,370	168,916	174,286

(1) The amount disclosed represent the aggregate grant date fair value of the stock option granted to Mr. Schmid during fiscal year 2020 under our 2018 Equity Incentive Plan, computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the stock option are set forth in Note 10 to our audited financial statements included elsewhere in this prospectus. This amount does not reflect the actual economic value that may be realized by the named non-employee director.

(2) Immediately following his option grant, Mr. Schmid exercised the option and received shares of our common stock subject to forfeiture provisions that lapse over the option's vesting schedule. As of December 31, 2020, Mr. Schmid held 852 vested shares and 29,822 unvested shares of our common stock. Dr. George, Dr. Xu and Dr. Lappe did not hold any options to purchase shares of our common stock. As of December 31, 2020, none of our non-employee directors held other unvested stock awards other than Dr. Lappe who held 59,433 shares of common stock that were unvested as of such date.

We have reimbursed and will continue to reimburse all of our non-employee directors for their reasonable out-of-pocket expenses incurred in attending board of directors and committee meetings.

Pratik Shah, Ph.D. has served as the Executive Chairperson and a member of our board directors since December 2017. João Siffert, M.D. has served as Chief Executive Officer and a member of the board of directors since October 2020 and as our President since January 2021. See the section titled "Executive Compensation" for more information regarding their compensation.

We entered into a letter agreement with Mr. Schmid in November 2020 confirming his appointment as a member of our board of directors. Pursuant to his agreement, Mr. Schmid receives an annual cash retainer of $40,000, paid quarterly, and was entitled to a stock option award, which was granted in November 2020 and vests monthly over a period of three years subject to Mr. Schmid continued service to us. Mr. Schmid "early exercised" this option and purchased shares subject to our right of repurchase, which will lapse in accordance with the vesting schedule of the stock option.

In March 2021, our board of directors approved the grant of options to purchase 15,000 shares to each of Dr. George, Dr. Xu, Dr. Lappe, Mr. Schmid and Dr. William. These options will be granted under our 2021 Plan, contingent and effective upon the execution and delivery of the underwriting agreement relating to this offering, and will have a per share exercise price that is equal to the price per share at which our common stock is first sold to the public in this offering. The shares underlying each option will vest over one year on a monthly basis, subject to continued service through each vesting date. In addition, if a change in control occurs and the holder's continuous service has not terminated as of immediately prior, the vesting and exercisability of the options will be accelerated in full.

Our board of directors adopted a non-employee director compensation policy in March 2021 that became effective upon the execution and delivery of the underwriting agreement related to this offering and will be applicable to all of our non-employee directors. This compensation policy provides that each such non-employee director will receive the following compensation for service on our board of directors:

- an annual cash retainer of $35,000;
- an additional annual cash retainer of $30,000 for service as Chairman of board of directors;
- an additional annual cash retainer of $7,500, $5,000, $4,000 and $7,500 for service as a member of the audit committee, compensation committee, nominating and corporate governance committee and research and development committee, respectively;
- an additional annual cash retainer of $15,000, $10,000, $8,000 and $15,000 for service as chair of the audit committee, compensation committee, nominating and corporate governance committee and research and development committee, respectively (in lieu of the committee member retainer described above);
- an initial option grant to purchase 30,000 shares of our common stock on the date of each such non-employee director's appointment to our board of directors; and
- an annual option grant to purchase 15,000 shares of our common stock on the date of each of our annual stockholder meetings for each continuing director (pro-rated for directors who have not served on the board for 12 months prior to the applicable annual stockholder meeting based on the number of full months served on the board).

Each of the option grants described above will be granted under our 2021 Plan, the terms of which are described in more detail above under the section titled "Executive Compensation—Employee Benefit Plans—2021 Equity Incentive Plan." Each such option grant will vest and become exercisable subject to the director's continuous service to us through the earlier of the first anniversary of the date of grant or the next annual stockholder meeting, provided that each option will vest in full upon a change in control (as defined in the 2021 Plan). The term of each option will be ten years, subject to earlier termination as provided in the 2021 Plan. An eligible director may decline all or any portion of his or her compensation by giving notice to us prior to the date cash may be paid or equity awards are to be granted, as the case may be.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following includes a summary of transactions since January 1, 2018 and any currently proposed transactions, to which we were or are to be a participant, in which (i) the amount involved exceeded or will exceed the lesser of $120,000 or one percent of the average of our total assets at year-end for the last two completed fiscal years; and (ii) any of our directors, executive officers or holders of more than 5% of our capital stock, or any affiliate or member of the immediate family of the foregoing persons, had or will have a direct or indirect material interest, other than compensation and other arrangements that are described under the section of this prospectus titled "Executive and Director Compensation."

Financings

Convertible Promissory Note Financing

From May 2018 to February 2019, we issued convertible promissory notes in the aggregate principal amount of $450,000 with an annual interest rate of 8% per annum, pursuant to note purchase agreements, with various investors, or the note financing. In connection with the issuance of the Series A convertible preferred stock as described below, the convertible promissory notes were cancelled and converted into shares of Series A convertible preferred stock.

The table below sets forth the principal amount of convertible promissory notes purchased by our executive officers, directors, holders of more than 5% of our capital stock and their affiliated entities or immediate family members.

Name	Principal Amount of Notes ($)
Executive Officers and Directors:	
Pratik Shah, Ph.D.(1)	250,000

(1) The convertible promissory note was held by Pratik Shah Living Trust dtd 6/15/11 until conversion thereof into shares of our Series A convertible preferred stock.

Promissory Note Financing

In January 2019, we entered into a promissory note with Pratik Shah, Ph.D., Executive Chairperson and a member of our board of directors, in the aggregate amount of up to $500,000 with an annual interest rate of 10.0% per annum. An aggregate principal amount of $200,000 was advanced and fully repaid in 2020, including interest.

In November 2019, we entered into a promissory note with Pratik Shah, Ph.D., Executive Chairperson and a member of our board of directors, in the aggregate amount of up to $500,000 with an annual interest rate of 9.25% per annum. An aggregate principal amount of $400,000 was advanced and fully repaid in 2020, including interest.

Series A Convertible Preferred Stock Financing

In February 2020, we entered into a Series A preferred stock purchase agreement with various investors, pursuant to which, in two separate tranches, we issued and sold an aggregate of 21,710,814 shares of our Series A convertible preferred stock at a price per share of $2.0727 for gross proceeds of $45.5 million. In connection with the Series A Financing, we issued an additional 301,685 shares of our Series A convertible preferred stock in February 2020 upon the conversion and extinguishment of our convertible notes.

The table below sets forth the number of shares of our Series A convertible preferred stock purchased by our executive officers, directors, holders of more than 5% of our capital stock and their affiliated entities or immediate family members.

Name	Series A Convertible Preferred Stock (#)	Aggregate Purchase Price ($)
Executive Officers and Directors:		
Pratik Shah, Ph.D.(1)	171,025	283,589
Greater than 5% stockholders:		
Quan Venture Fund II, L.P.(2)	7,236,938	15,000,001
SR One Capital Fund I Aggregator, LP(3)	7,236,938	15,000,001
Cormorant and its affiliated entities	4,824,625	10,000,000

(1) The convertible preferred stock is held by Pratik Shah Living Trust dtd 6/15/11 (Shah Trust). The shares were issued upon conversion of certain convertible notes held by the Shah Trust.
(2) Dr. Stella Xu, a member of our board directors, is one of the general partners of Quan Venture Fund II, L.P.
(3) Dr. Simeon George, a member of our board directors, is employed as the President of SR One Capital Management LP, an entity affiliated with SR One Capital Fund I Aggregator, LP.

Series B Convertible Preferred Stock Financing

In January 2021, we entered into a Series B preferred stock purchase agreement with various investors, pursuant to which we issued and sold an aggregate of 19,083,979 shares of our Series B convertible preferred stock at a price per share of $6.55 for gross proceeds of $125.0 million.

The table below sets forth the number of shares of our Series B convertible preferred stock purchased by our executive officers, directors, holders of more than 5% of our capital stock and their affiliated entities or immediate family members.

Name	Series B Convertible Preferred Stock (#)	Aggregate Purchase Price ($)
Greater than 5% stockholders:		
Quan Venture Fund II L.P.(1)	458,016	3,000,005
SR One Capital Fund I Aggregator, LP(2)	1,526,718	10,000,003
Cormorant and its affiliated entities(3)	2,290,077	15,000,004
Logos and its affiliated entities(3)	3,312,978	21,700,006

(1) Dr. Stella Xu, a member of our board directors, is one of the general partners of Quan Venture Fund II, L.P.
(2) Dr. Simeon George, a member of our board directors, is employed as the President of SR One Capital Management LP, an entity affiliated with SR One Capital Fund I Aggregator, LP.
(3) Dr. Arsani William, a member of our board of directors, is a managing member of Logos Opportunities Fund II, L.P. and Logos SPV 1 LP.

Investors' Rights, Management, Voting and Co-Sale Agreements

In connection with our convertible preferred stock financing, we entered into investors' rights, voting and right of first refusal and co-sale agreements containing registration rights, information rights, rights of first offer, voting rights and rights of first refusal, among other things, with certain holders of our capital stock. The holders of more than 5% of our capital stock listed above are parties to these agreements. Our executive officers and directors who are parties to these agreements or who are related to parties to these agreements are Pratik Shah Ph.D., João Siffert, M.D., Sean Jeffries, Ph.D., Simeon George, M.D., Stella Xu, Ph.D. and Arsani William, M.D.

These stockholder agreements will terminate upon the closing of this offering, except for the registration rights granted under our investors' rights agreement, which will terminate upon the earliest of (i) the closing of a deemed liquidation event, as defined in our amended and restated certificate of incorporation, as currently in effect; (ii) with respect to each stockholder, the date when such stockholder can sell all of its registrable shares without limitation during a three-month period without registration pursuant to Rule 144 of the Securities Act (Rule 144), or another similar exemption under the Securities Act; and (iii) the fifth year anniversary of the date of the investors' rights agreement. For a description of the registration rights, see the section of this prospectus titled "Description of Capital Stock—Registration Rights."

Consulting Arrangements

In January 2019, we entered into a consulting agreement with Marlinspike Group, LLC (Marlinspike) which provided management and business consulting services as well as business development support and access to office space on an as-available basis for a monthly fee of $83,000. Pratik Shah, Ph.D., Executive Chairperson and a member of our board of directors, is an executive officer of Marlinspike and Sean Jeffries, Ph.D., Chief Operating Officer, was an executive officer of Marlinspike until February 2020. Per its terms, the consulting agreement has expired. In March 2020, we entered into a consulting agreement with Marlinspike for substantially the same terms for a monthly fee of $20,000.

In December 2017, we entered into a consulting agreement with Aseem Z. Ansari, Ph.D., as amended in May 2018 and October 2020. Dr. Ansari provides consulting services regarding molecules that modulate gene expression for commercial applications, including but not limited to human therapeutics, and oversees the research and development activities for a monthly fee of $15,000. In March 2021, in accordance with the terms of the agreement, our board of directors approved extending the monthly fee under the agreement indefinitely, subject to any shorter period as determined in the sole discretion of our board of directors.

Lease Agreement

In February 2021, we entered into a lease agreement with Crossing Holdings, LLC (Crossing Holdings) to rent approximately 12,370 square feet of laboratory and office space. Pratik Shah, Ph.D., Executive Chairperson, co-founder and a member of our board of directors, is the sole member and Manager of Crossing Holdings. The anticipated delivery date of the space is September 1, 2021, and the lease is expected to commence at that time. The term of the lease is 72 months after commencement with an option to extend the lease term for a period of three years.

Indemnification Agreements

We have entered into indemnification agreements with certain of our current directors and executive officers, and intend to enter into new indemnification agreements with each of our current directors and executive officers before the completion of this offering. Our amended and restated certificate of incorporation and our amended and restated bylaws will provide that we will indemnify our directors and officers to the fullest extent permitted by applicable law. See the section of this prospectus titled "Management—Limitation on Liability and Indemnification Matters."

Directed Share Program

At our request, the underwriters have reserved for sale, at the initial public offering price, up to 3% of the shares offered by this prospectus, excluding the additional shares that the underwriters have a 30-day option to purchase, for sale to certain of our employees, certain of our directors and certain other parties.

Policies and Procedures for Related Party Transactions

We intend to adopt a written related-person transactions policy prior to the completion of this offering that sets forth our policies and procedures regarding the identification, review, consideration and oversight of “related-person transactions.” For purposes of our policy only, a “related-person transaction” is a transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we and any “related person” are participants involving an amount that exceeds $120,000. Transactions involving compensation for services provided to us as an employee, consultant or director are not considered related-person transactions under this policy. A related person is any executive officer, director, nominee to become a director or a holder of more than five percent of our common stock, including any of their immediate family members and affiliates, including entities owned or controlled by such persons.

Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our audit committee (or, where review by our audit committee would be inappropriate, to another independent body of our board of directors) for review. The presentation must include a description of, among other things, all of the parties thereto, the direct and indirect interests of the related persons, the purpose of the transaction, the material facts, the benefits of the transaction to us and whether any alternative transactions are available, an assessment of whether the terms are comparable to the terms available from unrelated third parties and management’s recommendation. To identify related-person transactions in advance, we rely on information supplied by our executive officers, directors and certain significant stockholders. In considering related-person transactions, our audit committee or another independent body of our board of directors takes into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;
- the impact on a director’s independence in the event the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the terms of the transaction;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties.

In the event a director has an interest in the proposed transaction, the director must recuse himself or herself from the deliberations and approval.

PRINCIPAL STOCKHOLDERS

The following table sets forth, as of January 25, 2021, information regarding beneficial ownership of our capital stock by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our named executive officers;
- each of our directors; and
- all of our current executive officers and directors as a group.

The percentage ownership information under the column titled “Before Offering” is based on 41,817,322 shares of common stock outstanding as of January 25, 2021 (which includes 622,893 shares outstanding that are subject to forfeiture or our right to repurchase as of such date) assuming the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 25,212,548 shares of common stock in connection with the closing of this offering. The percentage ownership information under the column titled “After Offering” is based on the sale of 12,000,000 shares of common stock in this offering. The percentage ownership information assumes no purchases of any shares of common stock in this offering by the beneficial owners identified in the table below. The percentage ownership information does not reflect any potential purchases pursuant to the directed share program or otherwise of any shares of common stock in this offering by the beneficial owners identified in the table below.

Beneficial ownership is determined according to the rules of the SEC and generally means that a person has beneficial ownership of a security if he, she or it possesses sole or shared voting or investment power of that security. In addition, the rules include shares of common stock issuable upon the exercise of stock options that are currently exercisable or exercisable within 60 days of December 31, 2020. These shares are deemed to be outstanding and beneficially owned by the person holding those options for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. The information contained in the following table does not necessarily indicate beneficial ownership for any other purpose. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them, subject to applicable community property laws.

Unless otherwise noted below, the address for each beneficial owner listed in the table below is c/o Design Therapeutics, Inc., 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011.

Name of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
Greater than 5% Stockholders:			
SR One Capital Fund I Aggregator, LP(1)	5,376,476	12.86%	9.99%
Quan Venture Fund II, L.P.(2)	4,720,830	11.29%	8.77%
Cormorant and its affiliated entities(3)	4,364,845	10.44%	8.11%
Trusts for the benefit of Dr. Shah's family(4)	7,668,710	18.34%	14.25%
Aseem Z. Ansari, Ph.D.(5)	7,668,711	18.34%	14.25%
Named Executive Officers and Directors:			
Pratik Shah, Ph.D.(6)	104,923	*	*
João Siffert, M.D.(7)	766,870	1.81%	1.41%
Sean Jeffries, Ph.D.(8)	807,233	1.93%	1.50%
Stella Xu, Ph.D.(9)	4,720,830	11.29%	8.77%
Simeon George, M.D.(10)	5,376,476	12.86%	9.99%
Rodney Lappe, Ph.D.(11)	92,024	*	*
John Schmid(12)	30,674	*	*
Arsani William, M.D.(13)	2,032,501	4.86%	3.78%
All current executive officers and directors as a group (8 persons)(14)	13,931,531	32.81%	25.58%

* Represents beneficial ownership of less than 1%.

(1) Consists of 5,376,476 shares of common stock issuable upon conversion of convertible preferred stock held by SR One Capital Fund I Aggregator, LP (SR One Capital Fund). SR One Capital Partners I, LP (SR One Capital Partners) is the general part of SR One Capital Fund. SR One Capital Management, LLC (SR One Capital Management) is the general partner of SR One Capital Partners. Simeon George, M.D., a member of our board of directors, is the managing member of SR One Capital Management. By virtue of such relationships, SR One Capital Partners and SR One Capital Management may be deemed to have voting and investment power with respect to the shares held by SR One Capital Fund and as a result may be deemed to have beneficial ownership of such shares. Each of SR One Capital Partners and SR One Capital disclaims beneficial ownership of the shares held by SR One Capital Fund, except to the extent of its or his pecuniary interest therein if any. The address for SR One Capital Fund I Aggregator, LP is 985 Old Eagle School Road, Suite 511, Wayne, PA 19087.

(2) Consists of 4,720,830 shares of common stock issuable upon conversion of convertible preferred stock held by Quan Venture Fund II, L.P. (Quan Venture Fund). Samanth Du, Marietta Wu and Stella Xu, Ph.D., a member of our board of directors, are the general partners of Quan Venture Fund. By virtue of such relationship, Dr. Xu may be deemed to have voting and investment power with respect to the shares held by Quan Venture Fund and as a result may be deemed to have beneficial ownership of such shares. Dr. Xu disclaims beneficial ownership of the shares held by Quan Venture Fund, except to the extent of her pecuniary interest therein if any. The address for Quan Venture Fund II, L.P is c/o Maples Corporate Services Ltd., PO Box 309, Ugland House Grand Cayman, Cayman Islands KY1-1104.

(3) Consists of (i) 887,541 shares of common stock issuable upon conversion of convertible preferred stock held by Cormorant Global Healthcare Master Fund, LP (Cormorant Master Fund); (ii) 2,335,405 shares of common stock issuable upon conversion of convertible preferred stock held by Cormorant Private Healthcare Fund II, LP (Cormorant Fund II); (iii) 1,084,906 shares of common stock issuable upon conversion of convertible preferred stock held by Cormorant Private Healthcare Fund III, LP (Cormorant Fund III); and (iv) 56,993 shares of common stock issuable upon conversion of convertible preferred stock held by CRMA SPV, LP (CRMA). Cormorant Global Healthcare GP, LLC (Global GP) is the general partner of Cormorant Master Fund, Cormorant Private Healthcare II GP, LLC (Private GP II) is the general partner of Cormorant Fund II and Cormorant Private Healthcare III GP, LLC (Private GP III) is the general partner of Cormorant Fund III. Bihua Chen serves as the managing member of Global GP, Private GP II and Private GP III, and as the general partner of Cormorant Asset Management, LP (Cormorant). Cormorant serves as the investment manager to Cormorant Fund II, Cormorant Master Fund, Cormorant Fund III and CRMA. Ms. Chen has sole voting and investment control over the shares held by the Cormorant Master Fund, Cormorant Fund II and CRMA. The address for each of the entities is 200 Clarendon Street, 52nd Floor, Boston Massachusetts 02116. The address of the principal business office for the above referenced entities is 200 Clarendon Street, 52nd Floor, Boston, MA 02116.

(4) Jason Howerton is the trustee of the trusts for the benefit of Dr. Shah's family, and in such capacity has the sole power to vote and dispose of such shares. Mr. Howerton disclaims beneficial ownership of the shares held by the trusts.
(5) Consists of 7,668,711 shares of common stock held by Aseem Z. Ansari, Ph.D.
(6) Consists of 104,923 shares of common stock issuable upon conversion of convertible preferred stock held by Pratik Shah Living Trust dtd 6/15/11.
(7) Consists of (a) 122,699 shares of common stock acquired by Dr. Siffert upon the exercise of a stock option, all of which will be subject to our right of repurchase as of March 26, 2021 and (b) 644,171 shares of common stock that Dr. Siffert has the right to acquire from us within 60 days of December 31, 2020 pursuant to the exercise of stock options, all of which will be unvested but exercisable as of March 26, 2021.
(8) Consists of 807,233 shares of common stock held by Dr. Jeffries, 252,261 of which will be subject to our right of repurchase as of March 26, 2021.
(9) Consists of the shares listed in footnote (2) above, which are held by Quan Venture Fund. Dr. Xu shares voting and dispositive power with respect to the shares held by Quan Venture Fund.
(10) Consists of the shares listed in footnote (1) above, which are held by SR One Capital Fund. Dr. George shares voting and dispositive power with respect to the shares held by SR One Capital Fund.
(11) Consists of 92,024 shares of common stock held by Dr. Lappe, 53,681 of which will be subject to our right of repurchase as of March 26, 2021.
(12) Consists of 30,674 shares of common stock acquired by Mr. Schmid upon the early exercise of a stock option, 27,266 of which will be subject to our right of repurchase as of March 26, 2021.
(13) Consists of: (i) 1,217,627 shares of shares of common stock issuable upon conversion of convertible preferred stock held by Logos Opportunities Fund II, L.P. (LOF); and (ii) 814,874 shares of shares of common stock issuable upon conversion of convertible preferred stock held by Logos SPV 1 LP (SPV, and together with LOF, the Logos entities). Logos Opportunities GP, LLC (Logos Opportunities GP) is the general partner of the Logos entities. Arsani William, M.D. and Graham Walmsley are the managing members of the Logos entities and share voting and dispositive power with respect to the shares held of record by LOS I LP and LGMF LP. The address for these entities is c/o Logos Global Management, LP, 1 Letterman Drive, Building D, Suite D3-700, San Francisco, California 94129.
(14) Consists of the shares described in note (6) through note (13) above.

DESCRIPTION OF CAPITAL STOCK

Upon filing and effectiveness of our amended and restated certificate of incorporation and the closing of this offering, our authorized capital stock will consist of 200,000,000 shares of common stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share. All of our authorized preferred stock upon the closing of this offering will be undesignated. The following is a summary of the rights of our common and preferred stockholders and some of the provisions of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective immediately prior to and upon the closing of this offering, respectively, and of the Delaware General Corporation Law. This summary is not complete. For more detailed information, please see our amended and restated certificate of incorporation and amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is a part, as well as the relevant provisions of the Delaware General Corporation Law.

Common Stock

Outstanding Shares

As of December 31, 2020, we had 16,604,774 shares of common stock outstanding (which includes 646,953 shares outstanding that are subject to forfeiture or our right to repurchase as of such date), held of record by 9 stockholders. This amount excludes our outstanding shares of convertible preferred stock, which will convert into 25,212,548 shares of our common stock in connection with the closing of this offering. Based on the number of shares of common stock outstanding as of December 31, 2020, and assuming (i) the conversion of all of our outstanding shares of convertible preferred stock and (ii) the issuance by us of 12,000,000 shares of our common stock in this offering, there will be 53,817,322 shares of common stock outstanding upon the closing of this offering.

As of December 31, 2020, there were 1,601,214 shares of common stock subject to outstanding options under the 2018 Plan.

Voting Rights

Each holder of common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders. The affirmative vote of holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of capital stock, voting as a single class, will be required to amend certain provisions of our amended and restated certificate of incorporation, including provisions relating to amending our amended and restated bylaws, the classified structure of our board of directors, the size of our board of directors, removal of directors, director liability, vacancies on our board of directors, special meetings, stockholder notices, actions by written consent and exclusive jurisdiction.

Dividends

Subject to preferences that may apply to any outstanding preferred stock, holders of our common stock are entitled to receive ratably any dividends that our board of directors may declare out of funds legally available for that purpose on a non-cumulative basis.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

Rights and Preferences

Holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

Preferred Stock

Upon the closing of this offering, all of our currently outstanding shares of convertible preferred stock will convert into common stock and we will not have any preferred stock outstanding. Immediately after the completion of this offering, our certificate of incorporation will be amended and restated to delete all references to such shares of convertible preferred stock. Under the amended and restated certificate of incorporation, our board of directors will have the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control that may otherwise benefit holders of our common stock and may adversely affect the market price of the common stock and the voting and other rights of the holders of common stock. We have no current plans to issue any shares of preferred stock.

Registration Rights

After the closing of this offering, certain holders of shares of our common stock, including all of the current preferred stockholders, including certain holders of more than five percent of our capital stock and entities affiliated with certain of our directors, will be entitled to certain rights with respect to registration of the shares of common stock issued upon conversion of our convertible preferred stock under the Securities Act. These shares are referred to as registrable securities. The holders of these registrable securities possess registration rights pursuant to the terms of our investors' rights agreement and are described in additional detail below. The registration of shares of our common stock pursuant to the exercise of the registration rights described below would enable the holders to trade these shares without restriction under the Securities Act when the applicable registration statement is declared effective. We will pay the registration expenses, other than underwriting discounts, selling commissions, stock transfer taxes and certain fees and disbursements of counsel for the selling holders, of the shares registered pursuant to the demand, piggyback and Form S-3 registrations described below.

Generally, in an underwritten offering, the managing underwriter, if any, has the right, subject to specified conditions and limitations, to limit the number of shares the holders may include. The

demand, piggyback and Form S-3 registration rights described below will expire upon the earliest to occur of (i) the closing of our first registered public offering of our common stock, with respect to any holder who then holds an amount of shares equal to less than one percent of our outstanding securities and may sell all such shares under Rule 144 during any three-month period; (ii) the fifth anniversary of the date of the investors' rights agreements; or (iii) with respect to any particular holder, at such time that such holder can sell its shares under Rule 144 of the Securities Act or another similar exemption during any three-month period.

Demand Registration Rights

The holders of registrable securities will be entitled to certain demand registration rights. Beginning 180 days following the effectiveness of the registration statement of which this prospectus is a part, certain investors holding, collectively, at least 50% of registrable securities then outstanding may , on not more than two occasions, request that we register all or a portion of their shares, subject to certain specified exceptions. If any of these holders exercises its demand registration rights, then holders of all registrable securities will be entitled to register their shares, subject to specified conditions and limitations, in the corresponding offering. Such request for registration must cover shares with an anticipated aggregate offering price of at least $10.0 million.

Piggyback Registration Rights

In connection with this offering, the holders of registrable securities are entitled to their rights to notice of this offering and to include their shares of registrable securities in this offering. The requisite percentage of these stockholders have waived all such stockholders' rights to notice of this offering and to include their shares of registrable securities in this offering. In the event that we propose to register any of our securities under the Securities Act in another offering, either for our own account or for the account of other security holders, the holders of registrable securities will be entitled to certain "piggyback" registration rights allowing them to include their shares in such registration, subject to specified conditions and limitations.

S-3 Registration Rights

Upon the closing of this offering, the holders of registrable securities will initially be entitled to certain Form S-3 registration rights. Certain investors may, on not more than two registrations on Form S-3 within any 12-month period, request that we register all or a portion of their shares on Form S-3 if we are qualified to file a registration statement on Form S-3, subject to specified exceptions. Such request for registration on Form S-3 must cover securities with an aggregate offering price which equals at least $3.0 million, net of selling expenses. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Anti-Takeover Provisions of Delaware Law and Our Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Section 203 of the Delaware General Corporation Law

We are subject to Section 203 of the DGCL, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;

- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

Section 203 defines a “business combination” to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an “interested stockholder” as an entity or person who, together with the person’s affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

The statute could prohibit or delay mergers or other takeover or change in control attempts and, accordingly, may discourage attempts to acquire us even though such a transaction may offer our stockholders the opportunity to sell their stock at a price above the prevailing market price.

Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Among other things, our amended and restated certificate of incorporation and amended and restated bylaws will:

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate, including the right to approve an acquisition or other change in control;
- provide that the authorized number of directors may be changed only by resolution of our board of directors;
- provide that our board of directors will be classified into three classes of directors;
- provide that, subject to the rights of any series of preferred stock to elect directors, directors may only be removed for cause, which removal may be effected, subject to any limitation imposed by law, by the holders of at least 66 2/3% of the voting power of all of our then-outstanding shares of the capital stock entitled to vote generally at an election of directors;

- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent or electronic transmission;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder's notice;
- provide that special meetings of our stockholders may be called only by the chairman of our board of directors, our chief executive officer or president or by our board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors, and not by our stockholders; and
- not provide for cumulative voting rights, therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose.

The amendment of any of these provisions would require approval by the holders of at least 66 2/3% of the voting power of all of our then-outstanding common stock entitled to vote generally in the election of directors, voting together as a single class.

The combination of these provisions will make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Because our board of directors has the power to retain and discharge our officers, these provisions could also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of Forum

Our amended and restated certificate of incorporation to be effective immediately prior to the closing of this offering will provide that the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) and any appellate court therefrom will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other

employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine. This provision will not apply to suits brought to enforce a duty or liability created by the Securities Act, Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction.

Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. Accordingly, both state and federal courts have jurisdiction to entertain such Securities Act claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation to be effective immediately prior to the closing of this offering will provide that, unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause or causes of action arising under the Securities Act, including all causes of action asserted against any defendant to such complaint. For the avoidance of doubt, this provision is intended to benefit and may be enforced by us, our officers and directors, the underwriters to any offering giving rise to such complaint, and any other professional entity whose profession gives authority to a statement made by that person or entity and who has prepared or certified any part of the documents underlying the offering. However, there is uncertainty as to whether a court would enforce such provision, and investors cannot waive compliance with federal securities laws and the rules and regulations thereunder.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions, and there can be no assurance that such provisions will be enforced by a court in those other jurisdictions. We note that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. Any person or entity purchasing or otherwise acquiring any interest in our securities shall be deemed to have notice of and consented to these provisions. Although we believe these provisions benefit us by providing increased consistency in the application of law for the specified types of actions and proceedings, the provisions may have the effect of discouraging lawsuits against us or our directors and officers. Please also see the section titled “Risk Factors—Our amended and restated certificate of incorporation, which will become effective upon the closing of this offering designates the state courts the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the feral court for the District of Delaware, and the federal district courts of the United States of America will be the exclusive forums for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees.”

Limitation on Liability and Indemnification

See the section of this prospectus titled “Management—Limitation on Liability and Indemnification Matters.”

Listing

Our common stock has been approved for listing on the Nasdaq Global Select Market under the trading symbol “DSGN.”

Transfer Agent and Registrar

Upon the closing of this offering, the transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company, LLC. The transfer agent's address is 6201 15th Avenue, Brooklyn, New York 11219, and its telephone number is (800) 937-5449.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of our common stock, including shares issued upon the exercise of outstanding options, in the public market after the completion of this offering, or the perception that those sales may occur, could adversely affect the prevailing market price for our common stock from time to time or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after the completion of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate.

Sale of Restricted Shares

Based on the number of shares of our common stock outstanding as of December 31, 2020, upon the closing of this offering and assuming (i) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 25,212,548 shares of our common stock in connection with the closing of this offering; (ii) no exercise of the underwriters' option to purchase additional shares of common stock; and (iii) no exercise of outstanding options, we will have outstanding an aggregate of approximately 53,817,322 shares of common stock. Of these shares, all of the 12,000,000 shares of common stock to be sold in this offering will be freely tradable in the public market without restriction or further registration under the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 or subject to lock-up agreements. All remaining shares of common stock held by existing stockholders immediately prior to the consummation of this offering will be "restricted securities," as such term is defined in Rule 144. These restricted securities were issued and sold by us in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

As a result of the lock-up agreements referred to below and the provisions of Rule 144 and Rule 701 under the Securities Act, based on the number of shares of our common stock outstanding (calculated as of December 31, 2020 on the basis of the assumptions described above and assuming no exercise of the underwriter's option to purchase additional shares, if any, and no exercise of outstanding options), the shares of our common stock (excluding the shares sold in this offering) that will be available for sale in the public market are as follows:

Approximate Number of Shares	First Date Available For Sale Into Public Market
41,817,322 shares	181 days after the date of this prospectus, upon expiration of the lock-up agreements referred to below, subject in some cases to applicable volume, manner of sale and other limitations under Rule 144 and Rule 701.

We may issue shares of common stock from time to time as consideration for future acquisitions, investments or other corporate purposes. In the event that any such acquisition, investment or other transaction is significant, the number of shares of common stock that we may issue may in turn be significant. We may also grant registration rights covering those shares of common stock issued in connection with any such acquisition and investment.

In addition, the shares of common stock reserved for future issuance under our 2021 Plan will become eligible for sale in the public market to the extent permitted by the provisions of various vesting

schedules, the lock-up agreements, a registration statement under the Securities Act or an exemption from registration, including Rule 144 and Rule 701.

Rule 144

In general, persons who have beneficially owned restricted shares of our common stock for at least six months, and any affiliate of the company who owns shares of our common stock, are entitled to sell their securities without registration with the SEC under an exemption from registration provided by Rule 144.

Under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, and we are current in our Exchange Act reporting at the time of sale, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the 90 days preceding a sale and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to below, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least 12 months, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to above, if applicable).

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months, are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- 1% of the number of shares of common stock then outstanding, which will equal approximately 538,173 shares of common stock immediately upon the closing of this offering (calculated as of December 31, 2020 on the basis of the assumptions described above and assuming no exercise of the underwriter's option to purchase additional shares, if any, and no exercise of outstanding options); or
- the average weekly trading volume of our common stock on Nasdaq during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us. Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted securities have entered into lock-up agreements as referenced above and their restricted securities will become eligible for sale (subject to the above limitations under Rule 144) upon the expiration of the restrictions set forth in those agreements.

Rule 701

In general, under Rule 701 as currently in effect, any of our employees, directors, officers, consultants or advisors who acquired common stock from us in connection with a written compensatory stock or option plan or other written agreement in compliance with Rule 701 before the

effective date of the registration statement of which this prospectus is a part (to the extent such common stock is not subject to a lock-up agreement) and who are not our “affiliates” as defined in Rule 144 during the immediately preceding 90 days, is entitled to rely on Rule 701 to resell such shares beginning 90 days after the date of this prospectus in reliance on Rule 144, but without complying with the notice, manner of sale, public information requirements or volume limitation provisions of Rule 144. Persons who are our “affiliates” may resell those shares beginning 90 days after the date of this prospectus without compliance with minimum holding period requirements under Rule 144 (subject to the terms of the lock-up agreement referred to below, if applicable).

Lock-Up Agreements

In connection with this offering, we, our directors, our executive officers and holders of substantially all of our other outstanding shares of common stock or securities convertible into or exchangeable for shares of our common stock outstanding upon the closing of this offering, have agreed, subject to certain limited exceptions, with the underwriters not to directly or indirectly offer, sell, contract to sell, pledge, grant any option to purchase, make any short sale or otherwise dispose of or hedge any shares of our common stock or any options to purchase shares of our common stock, or any securities convertible into or exchangeable for shares of common stock during the period from the date of the lock-up agreement continuing through and including the date 180 days after the date of this prospectus, except with the prior written consent of the representatives of the underwriters, and certain other limited exceptions. These agreements are described in the section of this prospectus titled “Underwriting.”

In addition to the restrictions contained in the lock-up agreements described above, we have entered into agreements with certain security holders, including the amended and restated investors’ rights agreement, our standard form of option agreement, our standard form of restricted stock agreement and our standard form of restricted stock purchase agreement, that contain market stand-off provisions or incorporate market stand-off provisions from our equity incentive plan imposing restrictions on the ability of such security holders to offer, sell or transfer our equity securities for a period of 180 days following the date of this prospectus.

Registration Rights

Upon the closing of this offering, the holders of an aggregate of 25,212,548 shares of our common stock will have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. See the section of this prospectus titled “Description of Capital Stock—Registration Rights” for additional information regarding these registration rights.

Equity Incentive Plans

We intend to file with the SEC a registration statement on Form S-8 under the Securities Act covering the shares of common stock reserved for issuance under outstanding options under the 2018 Plan and reserved for issuance under the 2021 Plan and the ESPP. The registration statement is expected to be filed and become effective as soon as practicable after the completion of this offering. Accordingly, shares registered under the registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the acquisition, ownership and disposition of our common stock issued pursuant to this offering. This discussion is not a complete analysis of all potential U.S. federal income tax consequences relating thereto, does not address the potential application of the Medicare contribution tax on net investment income, and does not address any estate or gift tax consequences or any tax consequences arising under any state, local or foreign tax laws, or any other U.S. federal tax laws. This discussion is based on the Internal Revenue Code of 1986, as amended (the Code), and applicable Treasury Regulations promulgated thereunder, judicial decisions and published rulings and administrative pronouncements of the Internal Revenue Service (IRS), all as in effect as of the date hereof. These authorities are subject to differing interpretations and may change, possibly retroactively, resulting in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the IRS with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions.

This discussion is limited to non-U.S. holders who purchase our common stock pursuant to this offering and who hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all of the U.S. federal income tax consequences that may be relevant to a particular holder in light of such holder's circumstances. This discussion also does not consider any specific facts or circumstances that may be relevant to holders subject to special rules under the U.S. federal income tax laws, including:

- certain former citizens or long-term residents of the United States;
- partnerships or other pass-through entities (and investors therein);
- "controlled foreign corporations;"
- "passive foreign investment companies;"
- corporations that accumulate earnings to avoid U.S. federal income tax;
- banks, financial institutions, investment funds, insurance companies, brokers, dealers or traders in securities;
- tax-exempt organizations and governmental organizations;
- tax-qualified retirement plans;
- "qualified foreign pension funds" as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds
- persons subject to the alternative minimum tax;
- persons that own, or have owned, actually or constructively, more than 5% of our common stock at any time;
- accrual-method taxpayers subject to special tax accounting rules under Section 451(b) of the Code; and
- persons holding our common stock as part of a hedging or conversion transaction, straddle, synthetic security, constructive sale, or other risk reduction strategy or integrated investment.

If an entity or arrangement that is classified as a partnership for U.S. federal income tax purposes holds our common stock, the U.S. federal income tax treatment of a partner in the partnership will generally depend on the status of the partner, the activities of the partnership and certain

determinations made at the partner level. Partnerships holding our common stock and the partners in such partnerships are urged to consult their tax advisors about the particular U.S. federal income tax consequences to them of holding and disposing of our common stock.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. PROSPECTIVE INVESTORS SHOULD CONSULT THEIR TAX ADVISORS REGARDING THE PARTICULAR U.S. FEDERAL INCOME TAX CONSEQUENCES TO THEM OF ACQUIRING, OWNING AND DISPOSING OF OUR COMMON STOCK, AS WELL AS ANY TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL OR FOREIGN TAX LAWS AND ANY OTHER U.S. FEDERAL TAX LAWS.

Definition of Non-U.S. Holder

For purposes of this discussion, a non-U.S. holder is any beneficial owner of our common stock that is not a “U.S. person” or a partnership (including any entity or arrangement treated as a partnership) for U.S. federal income tax purposes. A U.S. person is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or entity treated as a corporation for U.S. federal income tax purposes) created or organized in or under the laws of the United States, any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust (i) whose administration is subject to the primary supervision of a U.S. court and which has one or more U.S. persons who have the authority to control all substantial decisions of the trust or (ii) that has a valid election in effect under applicable Treasury Regulations to be treated as a U.S. person.

Distributions on Our Common Stock

We have never declared or paid any cash dividends on our capital stock and we do not currently intend to pay any cash dividends on our capital stock in the foreseeable future. However, if we do make cash or other property distributions on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and will first be applied against and reduce a holder’s tax basis in our common stock, but not below zero. Any excess will be treated as gain realized on the sale or other disposition of our common stock and will be treated as described under the section titled “Gain on Disposition of Our Common Stock” below.

Subject to the discussions below regarding effectively connected income, backup withholding and Sections 1471 through 1474 of the Code (commonly referred to as FATCA), dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends or such lower rate specified by an applicable income tax treaty. To receive the benefit of a reduced treaty rate, a non-U.S. holder must furnish us or our paying agent with a valid IRS Form W-8BEN or IRS Form W-8BEN-E (or applicable successor form) and satisfy applicable certification and other requirements. This certification must be provided to us or our paying agent before the payment of dividends and must be updated periodically. If the non-U.S. holder holds the stock through a financial institution or other agent acting on the non-U.S. holder’s behalf, the non-U.S. holder will be required to provide appropriate documentation to the agent, which then will be required to provide certification to us or our paying agent, either directly or through other intermediaries.

Non-U.S. holders that do not provide the required certification on a timely basis, but that qualify for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS.

If a non-U.S. holder holds our common stock in connection with the conduct of a trade or business in the United States, and dividends paid on our common stock are effectively connected with such holder's U.S. trade or business (and are attributable to such holder's permanent establishment in the United States if required by an applicable tax treaty), the non-U.S. holder will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must generally furnish a valid IRS Form W-8ECI (or applicable successor form) to the applicable withholding agent.

However, any such effectively connected dividends paid on our common stock generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items. Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Gain on Disposition of Our Common Stock

Subject to the discussions below regarding backup withholding and FATCA, a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized on the sale or other disposition of our common stock, unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States, and if required by an applicable income tax treaty, is attributable to a permanent establishment maintained by the non-U.S. holder in the United States;
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the disposition, and certain other requirements are met; or
- our common stock constitutes a "United States real property interest" by reason of our status as a United States real property holding corporation (USRPHC) for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding the disposition or the non-U.S. holder's holding period for our common stock, and our common stock is not "regularly traded" on an established securities market (as defined by applicable Treasury Regulations).

Determining whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our other trade or business assets and our foreign real property interests. We believe that we are not currently and do not anticipate becoming a USRPHC for U.S. federal income tax purposes, although there can be no assurance we will not in the future become a USRPHC. If we are or become a USRPHC and the "regularly traded" exception noted above does not apply to the disposition, a non-U.S. holder will generally be taxed on any gain in the same manner as gain that is effectively connected with the conduct of a U.S. trade or business, except that the branch profits tax generally will not apply. Prospective investors are encouraged to consult their own tax advisors regarding the possible consequences to them if we are, or were to become, a USRPHC.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable

income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items. A non-U.S. holder described in the second bullet point above will be subject to U.S. federal income tax at a flat 30% rate (or such lower rate specified by an applicable income tax treaty) on gain realized upon the sale or other taxable disposition of our common stock, but may be offset by certain U.S.-source capital losses (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses. Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Information Reporting and Backup Withholding

Annual reports are required to be filed with the IRS and provided to each non-U.S. holder indicating the amount of distributions on our common stock paid to such holder and the amount of any tax withheld with respect to those distributions. These information reporting requirements apply even if no withholding was required because the distributions were effectively connected with the holder's conduct of a U.S. trade or business, or withholding was reduced or eliminated by an applicable income tax treaty. This information also may be made available under a specific treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established. Backup withholding, currently at a 24% rate, generally will not apply to payments to a non-U.S. holder of dividends on or the gross proceeds of a disposition of our common stock provided the non-U.S. holder furnishes the required certification for its non-U.S. status, such as by providing a valid IRS Form W-8BEN, W-8BEN-E or W-8ECI, or certain other requirements are met. Backup withholding may apply if the payor has actual knowledge, or reason to know, that the holder is a U.S. person.

Backup withholding is not an additional tax. If any amount is withheld under the backup withholding rules, the non-U.S. holder should consult with a U.S. tax advisor regarding the possibility of and procedure for obtaining a refund or a credit against the non-U.S. holder's U.S. federal income tax liability, if any.

Withholding on Foreign Entities

FATCA imposes a U.S. federal withholding tax of 30% on certain payments made to a "foreign financial institution" (as specially defined under these rules) unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities certain information regarding certain U.S. account holders of such institution (which includes certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners) or an exemption applies. FATCA also generally imposes a U.S. federal withholding tax of 30% on certain payments made to a non-financial foreign entity unless such entity provides the withholding agent a certification identifying certain direct and indirect U.S. owners of the entity or an exemption applies. An intergovernmental agreement between the United States and an applicable foreign country may modify these requirements. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes. FATCA currently applies to dividends paid on our common stock. The U.S. Treasury released proposed Treasury Regulations which, if finalized in their present form, would eliminate the federal withholding tax of 30% applicable to the gross proceeds of a sale or other disposition of our common stock. In its preamble to such proposed Treasury Regulations, the U.S. Treasury stated that taxpayers may generally rely on the proposed regulations until final regulations are issued.

Prospective investors are encouraged to consult with their own tax advisors regarding the potential implications of FATCA on their investment in our common stock.

UNDERWRITING

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, SVB Leerink LLC and Piper Sandler & Co. are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	5,280,000
SVB Leerink LLC	3,960,000
Piper Sandler & Co.	2,760,000
Total	12,000,000

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an option to buy up to an additional 1,800,000 shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by us. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase 1,800,000 additional shares.

	No Exercise	Full Exercise
Per Share	$ 1.40	$ 1.40
Total	$ 16,800,000	$ 19,320,000

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $0.84 per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

We and our officers, directors, and holders of substantially all of our common stock have agreed with the underwriters, subject to certain exceptions, not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of the representatives. This agreement does not apply to any existing employee benefit plans. See "Shares Eligible for Future Sale" for a discussion of certain transfer restrictions.

At our request, the underwriters have reserved for sale, at the initial public offering price, up to 3% of the shares offered by this prospectus, excluding the additional shares that the underwriters have a 30-day option to purchase, for sale to certain of our employees, certain of our directors, and

certain other parties. If these persons purchase reserved shares, this will reduce the number of shares available for sale to the general public. Any reserved shares that are not so purchased will be offered by the underwriters to the general public on the same terms as the other shares offered by this prospectus. If purchased by any of our officers or directors, these shares will be subject to the terms of lock-up agreements described above. Other than the underwriting discount described on the front cover of this prospectus, the underwriters will not be entitled to any commission with respect to shares of our common stock sold pursuant to the directed share program.

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among us and the representatives. Among the factors considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, was our historical performance, estimates of the business potential and our earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

Our common stock has been approved for listing on the Nasdaq Global Select Market under the symbol “DSGN”.

In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A “covered short position” is a short position that is not greater than the amount of additional shares for which the underwriters’ option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. “Naked” short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on NYSE, NASDAQ NMS or relevant exchange, in the over-the-counter market or otherwise.

We estimate that our share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $2.9 million. We will reimburse the underwriters for certain of their expenses incurred in connection with this offering in an amount up to $50,000.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities and/or instruments of the issuer (directly, as collateral securing other obligations or otherwise) and/or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Selling Restrictions

European Economic Area

In relation to each Member State of the EEA (each a “Relevant State”), no shares have been offered or will be offered pursuant to the offering to the public in that Relevant State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation, except that the shares may be offeredto the public in that Relevant State at any time:

(i) to any legal entity which is a qualified investor as defined under Article 2 of the Prospectus Regulation;

(ii) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the Prospectus Regulation), subject to obtaining the prior consent of representatives for any such offer; or

(iii) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of the shares shall require us or any of the representative to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation.

For the purposes of this provision, the expression an “offer to the public” in relation to the shares in any Relevant State means the communication in any form and by any means of sufficient

information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression “Prospectus Regulation” means Regulation (EU) 2017/1129.

United Kingdom

(i) No shares have been offered or will be offered pursuant to the offering to the public in the United Kingdom prior to the publication of a prospectus in relation to the Shares which has been approved by the Financial Conduct Authority, except that the shares may be offered to the public in the United Kingdom at any time: to any legal entity which is a qualified investor as defined under Article 2 of the UK Prospectus Regulation;

(ii) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the UK Prospectus Regulation), subject to obtaining the prior consent of the representatives for any such offer; or

(iii) in any other circumstances falling within Section 86 of the FSMA,

provided that no such offer of the shares shall require the Issuer or any Manager to publish a prospectus pursuant to Section 85 of the FSMA or supplement a prospectus pursuant to Article 23 of the UK Prospectus Regulation. For the purposes of this provision, the expression an “offer to the public” in relation to the shares in the United Kingdom means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares and the expression “UK Prospectus Regulation” means Regulation (EU) 2017/1129 as it forms part of domestic law by virtue of the European Union (Withdrawal) Act 2018.

Canada

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser’s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser’s province or territory of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) (“Companies (Winding Up and Miscellaneous Provisions) Ordinance”) or which do not constitute an

invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) ("Securities and Futures Ordinance"), or (ii) to "professional investors" as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the SFA)) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore ("Regulation 32").

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32.

Singapore Securities and Futures Act Product Classification—Solely for the purposes of its obligations pursuant to Sections 309B(1)(a) and 309B(1)(c) of the SFA, we have determined, and hereby notify all relevant persons (as defined in Section 309A of the SFA) that the shares are “prescribed capital markets products” (as defined in the Securities and Futures (Capital Markets Products) Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended), or the FIEA. The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

LEGAL MATTERS

The validity of the issuance of our common stock offered in this prospectus will be passed upon for us by Cooley LLP, San Diego, California. Certain legal matters in connection with this offering will be passed upon for the underwriters by Latham & Watkins LLP.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements as of December 31, 2019 and December 31, 2020 and for each of the two years in the period ended December 31, 2020, as set forth in their report. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1, including exhibits and schedules, under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to us and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You may read our SEC filings, including this registration statement, over the Internet at the SEC's website at www.sec.gov. Upon the closing of this offering, we will be subject to the information reporting requirements of the Exchange Act and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for review on the web site of the SEC referred to above. We also maintain a website at www.designtx.com, at which, following the completion of this offering, you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on or accessible through our website is not a part of this prospectus or the registration statement of which it forms a part, and the inclusion of our website address in this prospectus is an inactive textual reference only.

DESIGN THERAPEUTICS, INC.

INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Shareholders and the Board of Directors of Design Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Design Therapeutics, Inc. (the Company) as of December 31, 2020 and 2019, the related statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit and cash flows for each of the two years then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2020 and 2019, and the results of its operations and its cash flows for the years then ended in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2020.

San Diego, California
March 5, 2021
except for paragraphs four through eleven of Note 13, as to which the date is
March 22, 2021

DESIGN THERAPEUTICS, INC.
BALANCE SHEETS
(in thousands, except share and par value data)

	December 31, 2019	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 77	$ 2,379
Investment securities	—	33,712
Prepaid expense and other current assets	13	142
Total current assets	90	36,233
Property and equipment, net	—	71
Deferred offering costs	—	212
Total assets	$ 90	$ 36,516
Liabilities, Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities:		
Accounts payable and accrued liabilities (including related party amounts of $1,844 and $20, respectively)	$ 2,723	$ 2,330
Convertible notes payable (including related party amounts of $276 and $0, respectively)	486	—
Notes payable—related party	209	—
Deferred revenue	201	—
Other current liabilities (including related party amounts of $62 and $0, respectively)	112	—
Total current liabilities	3,731	2,330
Other long-term liabilities	1	145
Total liabilities	3,732	2,475
Commitments and contingencies (See Note 8)		
Convertible preferred stock, $0.0001 par value; zero and 22,500,000 shares authorized, zero and 22,012,499 shares issued and outstanding at December 31, 2019 and 2020, respectively; liquidation preference of zero and $45,625 as of December 31, 2019 and 2020, respectively	—	45,356
Stockholders' deficit:		
Common stock, par value $0.0001; 50,000,000 and 60,000,000 shares authorized, 16,451,401 and 16,604,774 shares issued, 15,640,133 and 15,957,821 shares outstanding at December 31, 2019 and 2020, respectively	1	1
Additional paid-in capital	—	451
Accumulated deficit	(3,643)	(11,923)
Accumulated other comprehensive income	—	156
Total stockholders' deficit	(3,642)	(11,315)
Total liabilities, convertible preferred stock and stockholders' deficit	$ 90	$ 36,516

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	Year Ended December 31,	
	2019	2020
Revenue:		
Grant revenue	$ 834	$ 226
Operating expenses:		
Research and development (including related party amounts of $185 and $53, respectively)	1,654	6,060
General and administrative (including related party amounts of $580 and $292, respectively)	1,088	2,496
Total operating expenses	2,742	8,556
Loss from operations	(1,908)	(8,330)
Other (expense) income, net (including related party expense amounts of $80 and $30, respectively)	(139)	50
Net loss	$ (2,047)	$ (8,280)
Other comprehensive gain (loss):		
Unrealized gain on available-for-sale securities	—	156
Comprehensive loss	$ (2,047)	$ (8,124)
Net loss per share, basic and diluted	$ (0.13)	$ (0.52)
Weighted-average shares of common stock outstanding, basic and diluted	15,475,415	15,796,674

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(in thousands, except share data)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount				
Balance at December 31, 2018	—	$ —	15,337,421	$ 1	$ —	$ —	$ (1,596)	$ (1,595)
Vesting of restricted common stock	—	—	302,712	—	—	—	—	—
Net loss	—	—	—	—	—	—	(2,047)	(2,047)
Balance at December 31, 2019	—	—	15,640,133	1	—	—	(3,643)	(3,642)
Issuance of Series A convertible preferred stock, net of $270 of issuance costs	21,710,814	44,731	—	—	—	—	—	—
Conversion of convertible debt and interest to Series A convertible preferred stock	301,685	500	—	—	—	—	—	—
Settlement of bifurcated conversion liability	—	125	—	—	—	—	—	—
Vesting of restricted common stock	—	—	317,688	—	1	—	—	1
Stock-based compensation	—	—	—	—	450	—	—	450
Unrealized gain on investments	—	—	—	—	—	156	—	156
Net loss	—	—	—	—	—	—	(8,280)	(8,280)
Balance at December 31, 2020	22,012,499	$ 45,356	15,957,821	$ 1	$ 451	$ 156	$ (11,923)	$ (11,315)

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2019	2020
Cash flows from operating activities		
Net loss	$ (2,047)	$ (8,280)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation	—	5
Stock-based compensation	—	450
Amortization of premiums on investment securities, net	—	(71)
Non-cash interest expense	46	12
Non-cash interest expense—related party	49	26
Change in operating assets and liabilities:		
Prepaid expense and other assets	(11)	(341)
Deferred revenue	201	(201)
Accounts payable and accrued liabilities	836	1,430
Accounts payable and accrued liabilities—related party	786	(1,843)
Other long-term liabilities	1	144
Net cash used in operating activities	(139)	(8,669)
Cash flows from investing activities		
Purchases of investment securities	—	(55,589)
Proceeds from maturities of investment securities	—	22,104
Purchases of property and equipment	—	(76)
Net cash used in investing activities	—	(33,561)
Cash flows from financing activities		
Proceeds from the sale of convertible preferred stock, net of issuance costs	—	44,731
Proceeds from the issuance of notes payable, net of issuance costs—related party	396	200
Repayment of notes payable—related party	(200)	(400)
Proceeds from the exercise of common stock options and restricted stock awards	—	1
Net cash provided by financing activities	196	44,532
Net increase in cash and cash equivalents	57	2,302
Cash and cash equivalents at beginning of period	20	77
Cash and cash equivalents at end of period	$ 77	$ 2,379
Supplemental disclosures		
Issuance of convertible notes for services rendered	$ 200	$ —
Conversion of convertible notes to convertible preferred shares, including bifurcated conversion liability	$ —	$ 271
Conversion of convertible notes to convertible preferred shares, including bifurcated conversion liability - related party	$ —	$ 354
Interest paid	$ 9	$ 24

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS

1. Organization

Design Therapeutics, Inc. (the "Company") was incorporated in Delaware in December 2017 and is based in Carlsbad, California. The Company is a biopharmaceutical company pioneering a novel class of disease-modifying small-molecule therapeutics, called gene targeted chimeras ("GeneTACs"), that are designed to target the underlying cause of inherited nucleotide repeat expansion diseases. The Company's lead product candidate is in Friedreich ataxia ("FA"), its second GeneTAC program is in myotonic dystrophy type-1 ("DM1"), and it is also advancing its GeneTAC portfolio to address other serious nucleotide repeat-driven monogenic diseases.

Liquidity

From inception through December 31, 2020, the Company has devoted substantially all of its resources to organizing and staffing the company, business planning, raising capital, developing and optimizing its technology platform, identifying potential product candidates, undertaking research and preclinical studies, engaging in manufacturing for its development programs, and providing general and administrative support for these operations. The Company has funded its operations through December 31, 2020, primarily through the sale of convertible preferred stock, grant revenue and the issuance of convertible notes and debt. The Company has experienced net losses and negative cash flows from operating activities since inception and expects to incur net losses for the foreseeable future as it advances its research and development programs, conducts clinical trials for any future product candidates and commercializes any such product candidates for which it receives regulatory approval. As the Company continues to incur losses, its transition to profitability will depend on the successful development, approval and commercialization of its future product candidates, and on the achievement of sufficient revenue to support its cost structure. The Company may never achieve profitability, and unless and until it does, it will need to continue to raise additional capital to fund its operations. The Company plans to raise additional capital through public and private equity offerings, debt financings, or other capital sources which may include strategic collaborations, licensing arrangements or other arrangements with third parties. If the Company is unable to raise capital when needed or on attractive terms, it would be forced to delay, reduce or eliminate its research and development programs or other operations. Further, if the Company raises funds through strategic collaborations or other similar arrangements with third parties, it may have to relinquish valuable rights to its platform technology, future revenue streams, research programs or product candidates or may have to grant licenses on terms that may not be favorable to it and/or may reduce the value of its common stock. If any of these actions are taken, the Company's ability to achieve the development and commercialization goals could be adversely affected. The Company believes that it has sufficient working capital on hand to fund operations through at least the next twelve months from the date these financial statements were issued.

2. Basis of Presentation and Summary of Significant Accounting Policies

Basis of Presentation

The Company's financial statements are prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and include all adjustments, consisting of only normal recurring accruals, which in the opinion of management are necessary to present fairly the Company's financial position as of the reporting date and results of operations for the periods presented.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Stock Splits

In February 2020, the Company effected a 5-for-1 forward stock split of its issued and outstanding common stock. The par value of the common stock was not adjusted as a result of the forward stock split. The accompanying financial statements and notes to the financial statements have been retroactively adjusted to reflect the stock split for the periods presented.

Use of Estimates

The preparation of the Company's financial statements requires it to make estimates and assumptions that impact the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in the Company's financial statements and accompanying notes. The most significant estimates in the Company's financial statements relate to the recognition of grant revenue, accruals for research and development expenses and the valuation of equity-based awards. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of revenue and expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates.

The full extent to which the novel coronavirus-2019 ("COVID-19") pandemic will directly or indirectly impact the Company's business, results of operations and financial condition, including research and development costs, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain or treat COVID-19, as well as the economic impact on local, regional, national and international markets. The Company has considered potential impacts arising from the COVID-19 pandemic and is not presently aware of any events or circumstances that would require the Company to update its estimates, judgments or revise the carrying value of its assets or liabilities.

Cash and Cash Equivalents

The Company considers all highly liquid investments with maturities at the date of acquisition of three months or less to be cash equivalents. These investments may include money market accounts, money market funds, U.S. Government agency securities, corporate debt securities and commercial paper. The Company's cash reserves are in a readily available checking account.

Investment Securities

Investments in securities with maturities at the date of acquisition of more than three months are considered marketable securities. The Company determines the appropriate classification of its investments at the time of acquisition and reevaluates such determination at each balance sheet date. The Company has classified its investment holdings as available-for-sale, as the sale of such securities may be required prior to maturity to implement management strategies. The Company's investment policy sets minimum credit quality criteria and maximum maturity limits on its investments to provide for safety of principle, liquidity and a reasonable rate of return. Available-for-sale securities are recorded at fair value, based on current market valuations. Unrealized holding gains and losses on available-for-sale securities are excluded from earnings and are reported as a separate component of other comprehensive income (loss) until realized. Allowances for credit losses are reported on the balance sheet, if any.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

The cost of available-for-sale investment securities is adjusted for amortization of premiums and accretion of discounts until the securities mature. Such amortization and accretion is included in other income (expense) on the statements of operations. Realized gains and losses, if any, are also included in other income (expense) on the statement of operations and are derived using the specific identification method for determining the cost of the securities sold. During the periods presented, no realized gains or losses were recorded on the sale of investment securities and no impairments to reduce the value of any security was taken. See Note 5 for further discussion.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of the Company's cash, cash equivalents and investment securities. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits and invests cash reserves in money market accounts, money market funds, certificates of deposit, U.S. Treasury securities and high-quality marketable debt instruments of corporations in accordance with its investment policy. The investment policy's objectives are to preserve principal, achieve liquidity requirements and safeguard funds. The policy limits the amounts of credit exposure the Company make take and limits its concentrations of investments. The Company has not experienced any losses in such accounts and management believes that it is not exposed to significant credit risk related to these instruments.

Revenue Recognition

The Company has generated revenue from grants awarded to it by the National Science Foundation ("NSF"), the National Institutes of Health ("NIH") and the Friedreich's Ataxia Research Alliance ("FARA"). These grants provide the Company with funding for certain research and development activities on a best-efforts basis and do not require scientific achievement as a performance obligation. The Company has determined that the entities funding these grant awards do not meet the definition of a "customer", as defined by Accounting Standards Codification ("ASC") 606, *Revenue from Contracts with Customers (Topic 606*), and does not consider there to be a transfer of control of goods or services to the entities funding the grant. As such, the Company recognizes revenue from these awards in the period during which the related qualifying services are rendered and costs are incurred in accordance ASC 730, *Research and Development.* ASC 730 requires an assessment, at the inception of the grant, to determine whether the agreement is a liability or a contract to perform research and development services for others. Further, these grants are subject to the contribution's guidance under ASC 958, *Not-for-Profit Entities-Revenue Recognition,* and as such, the Company determines whether it is obligated to repay the funds received to the grantor regardless of the outcome of the related research and development activities. If it determines there is such a liability, it estimates the obligation and recognize that liability. Alternatively, if the Company is not required to repay the funds, the grant agreement is accounted for as a contract to perform research and development services for others, in which case, the grant revenue is recorded as income in the statements of operations as the expenses are incurred.

The Company's current grant revenue is not deemed refundable, and therefore, no liability is recognized when income is recorded. Grant funding received prior to expenses being incurred are recorded as deferred revenue on the Company's balance sheets. See Note 7 for further discussion.

Research and Development Expenses

Research and development expenses consist primarily of direct and indirect costs incurred in connection with its discovery efforts, and the preclinical and formulation development of its product

candidates. In the future, the Company expects a substantial portion of its research and development expenses will relate to the clinical development of its product candidates. Direct costs include contracted research development and manufacturing, consulting fees, license fees, laboratory supplies and other expenses incurred to sustain research and development programs. Indirect costs include personnel-related expenses, consisting of employee salaries, related benefits, and stock-based compensation expense for employees engaged in research and development activities, facilities related expenses, and other indirect expenses. A significant portion of the Company's research and development expenses have been direct costs, which the Company tracks by stage of development, preclinical or clinical. However, the Company does not track its internal research and development expenses on a program specific basis, unless specific to research grants, because these costs are deployed across multiple projects and, as such, are not separately classified. Research and development expenses are charged to operating expenses as incurred when these expenditures relate to the Company's research and development efforts and have no alternative future uses.

The Company has entered into various contracts with research and development organizations, vendors and consultants. Payments for these activities are based on the terms of the individual agreements, which may differ from the of periods over which materials or services are provided. Payments made in advance of performance are reflected in the accompanying balance sheets as prepaid expenses. The Company records accruals for estimated costs incurred for ongoing research and development activities. The Company determines accrual estimates through review of the underlying contracts along with discussions with research and other key personnel as to the progress of the research and development activities, invoices received and contracted costs. During the course of a study or trial, the Company adjusts its rate of expense recognition if actual results differ from its estimates. Significant judgments and estimates may be made in determining the prepaid or accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. Since its inception, the Company has not experienced any material differences between accrued or prepaid costs and actual costs.

Patent Costs

Costs related to filing and pursuing patent applications are recorded as general and administrative expenses in the statements of operations and expensed as incurred since recoverability of such expenditures is uncertain.

License Fees

Costs incurred to acquire technology licenses and milestone payments made on existing agreements are charged to research and development expense or capitalized based upon the asset achieving technological feasibility in accordance with management's assessment regarding the ultimate recoverability of the amounts paid and the potential for alternative future use. Management has concluded that the Company's existing license agreement does not currently have an alternative future use and therefore has recorded all fees incurred to date as research and development expense. See Note 9 for further discussion.

Deferred Offering Costs

The Company capitalizes costs that are directly associated with equity financings until such financings are consummated at which time such costs are recorded against the gross proceeds of the offering. These costs may include legal, accounting, printing, filing fees and other costs directly

associated with such financings. Should an in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the statements of operations.

Property and Equipment, Net

Property and equipment consists of laboratory equipment. Property and equipment is stated at cost and depreciated over the estimated useful lives of the assets using the straight-line method. The useful life of laboratory equipment is generally 5 years. Repairs and maintenance costs are charged to expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment and deferred offering costs. An impairment loss is recorded if and when events and circumstances indicate that assets might be impaired and the undiscounted cash flows estimated to be generated by those assets are less than the carrying amount of those assets. The Company has not recognized any impairment losses through December 31, 2020.

Stock-Based Compensation

The Company incurs stock-based compensation through this issuance of stock options and restricted stock, as described further in Note 10. Stock-based compensation expense represents the grant date fair value of equity awards recognized in the period using the Black-Scholes option pricing model. This option pricing model involves a number of estimates, including the expected lives of the stock options, the Company's anticipated stock volatility and interest rates. The Company recognizes the expense for equity awards on a straight-line basis over the requisite service periods of the awards, which is usually the vesting period. Forfeitures are recognized as they occur.

The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of stock option grants for the periods presented were as follows:

	Year Ended December 31,	
	2019	2020
Fair value of common stock	$ 0.003	$ 5.08
Expected term (years)	6.08	6.32
Expected volatility	80.0%	72.93%
Risk-free interest rate	1.72%	0.62%
Expected dividend yield	0.0%	0.0%

The Company determines the assumptions used in the option pricing model in the following manner:

Fair Value of Common Stock – Historically, there has been no public market of the Company's common stock. The fair value of the shares of common stock underlying the Company's share-based awards was estimated on each grant date by the Company's board of directors. To determine the fair value of the Company's common stock underlying option grants, the board of directors considered, among other things, input from management and valuations of the Company's common stock prepared by unrelated third-party valuation firms. As part of the preparation of these financial statements, the Company performed a retrospective reassessment

of its stock option fair values for all awards granted in 2020 and concluded that for awards granted from October 2020 through December 2020, the fair value per share for financial reporting purposes was $6.19 per share, equal to the valuation determined for the Company's common stock as of December 31, 2020.

Expected Term—The expected term of stock options represents the period of time that the awards are expected to be outstanding. Because the Company does not have sufficient historical exercise behavior, it determines the expected term assumption using the simplified method for employees and board members, which calculates the expected term as the average time-to-vesting and the contractual life of the award. The expected term for non-employees is generally the contractual term.

Expected Volatility—As the Company is not yet a public company and does not have a trading history for its common stock, the expected volatility assumption was determined by examining the historical volatilities of a group of industry peers whose share prices are publicly available.

Risk-Free Interest Rate—The risk-free rate assumption is based on the U.S. Treasury yield in effect at the time of the grant with maturities consistent with the expected term of the awards.

Expected Dividend Yield—The expected dividend yield assumption is based on the Company's history and expectation of dividend payouts. The Company has not paid and does not intend to pay dividends and, therefore, used an expected dividend yield of zero.

The weighted-average grant date fair value per share of option grants for the years ended December 31, 2019 and 2020 was $0.0023 and $4.49, respectively.

Stock-based compensation expense recognized in the Company's statements of operations during the years ended December 31, 2019 and 2020, were as follows (in thousands);

	Year Ended December 31,	
	2019	2020
Research and development	$ —	$ 177
General and administrative	—	273
Total	$ —	$ 450

The total unrecognized compensation cost related to outstanding unvested share-based awards as of December 31, 2020 was $7.3 million and is expected to be recognized over approximately 3.5 years.

Liability for Restricted Shares Issued

Certain stock options granted under the Company's stock-based compensation plan provides option holders the right to elect to exercise unvested options in exchange for restricted common stock. Further, the Company may issue restricted stock awards that are subject to vesting. These shares of restricted stock are not deemed, for accounting purposes, to be outstanding until those shares vest. The cash received in exchange for exercised and unvested shares related to restricted shares is recorded as a liability for the early exercise of stock options and restricted stock awards on the accompanying balance sheets in other long-term liabilities and are transferred into common stock and additional paid-in capital as the shares vest.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Income Taxes

The Company accounts for income taxes under the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date.

The Company recognizes net deferred tax assets to the extent that the Company believes these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

The Company records uncertain tax positions on the basis of a two-step process whereby (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50 percent likely to be realized upon ultimate settlement with the related tax authority. The Company recognizes interest and penalties related to unrecognized tax benefits within income tax expense. Any accrued interest and penalties are included within the related tax liability.

Comprehensive Loss

Comprehensive loss is defined as the change in equity during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss comprises of all components of net loss and all components of other comprehensive income (loss). The Company's only component of other comprehensive income (loss) is the unrealized gains (losses) recorded on its available-for-sale investment securities.

Segment Reporting

Operating segments are components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker for purposes of making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business as one operating segment. Further, no product revenue has been generated since inception and all assets are held in the United States.

Emerging Growth Company Status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 ("JOBS Act"). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. The Company has irrevocably elected not to avail itself of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Recently Adopted Accounting Pronouncements

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments,* which modifies the disclosure requirements for fair value measurements. The amendments relate to disclosures regarding unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and the narrative description of measurement uncertainty and are to be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption. All other amendments are to be applied retrospectively to all periods presented upon their effective date. The Company adopted the new standard as of January 1, 2020, which had no material effect on the Company's financial statements and related disclosures.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*. The new guidance removes, modifies and adds to certain disclosure requirements on fair value measurements in Topic 820. The Company adopted the new standard as of January 1, 2020, which had no material effect on the Company's and related disclosures.

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements (Topic 808)*, which clarifies the interaction between Topic 808 and Topic 606. The guidance, among other items, clarifies that certain transactions between collaborative participants should be accounted for as revenue under Topic 606 when the collaborative arrangement participant is a customer in the context of a unit of account. ASU 2018-18 is effective for fiscal years beginning after December 15, 2019. The Company adopted the new standard as of January 1, 2020, which had no material effect on the Company's financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*. ASU 2019-12 simplifies the accounting for income taxes by removing certain exceptions to the general principles in ASC 740 and also improves consistent application by clarifying and amending existing guidance. This guidance is effective for interim and annual periods beginning after December 15, 2020, with early adoption permitted. The Company early adopted the new standard as of January 1, 2020, which had no material effect on the Company's financial statements and related disclosures. See Note 11 for further discussion.

Recently Issued Accounting Pronouncements

In August 2020, the FASB issued ASU 2020-06, *Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging— Contracts in Entity's Own Equity*. The guidance, among other items, clarifies that certain transactions between collaborative participants should be accounted for as revenue under Topic 606 when the collaborative arrangement participant is a customer in the context of a unit of account. The ASU's amendments are effective for fiscal years beginning after December 15, 2021, and interim periods within those fiscal years, with early adoption
permitted for periods beginning after December 15, 2020. The Company does not currently expect the adoption of this guidance will have a material impact on its financial statements and related disclosures.

3. Net Loss Per Share

Basic net loss per share is computed by dividing the net loss by the weighted-average number of common shares outstanding for the period, without consideration for potentially dilutive securities.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Diluted net loss per share is computed by dividing the net loss by the weighted average number of common shares and dilutive common stock equivalents outstanding for the period determined using the treasury-stock and if-converted methods. Dilutive common stock equivalents are comprised of convertible preferred stock, restricted common stock subject to repurchase, stock options outstanding under the Company's equity incentive plan, and shares of common stock that are issuable under convertible debt. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding as inclusion of the potentially dilutive securities would be antidilutive.

Potentially dilutive securities not included in the calculation of diluted net loss per share because to do so would be anti-dilutive for the periods presented were as follows (in common stock equivalent shares):

	December 31,	
	2019	2020
Convertible preferred stock	—	13,504,598
Restricted common stock subject to repurchase	811,268	646,953
Common stock issuable under convertible debt	185,081	—
Stock options outstanding	30,674	1,601,214
Total	1,027,023	15,752,765

4. Fair Value Measurements

Accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or non-recurring basis. Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

Level 1: Observable inputs such as quoted prices in active markets.

Level 2: Inputs, other than the quoted prices in active markets that are observable either directly or indirectly.

Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

The carrying value of the Company's cash, accounts payable and accrued liabilities are considered to be representative of their respective fair values due to the short-term nature of those instruments. The Company's investment securities, which may include money market accounts, money market funds, certificates of deposits, U.S. Treasury securities, and high quality, marketable debt instruments of corporations and government sponsored enterprises, are measured at fair value in accordance with the fair value hierarchy. Financial liabilities measured at fair value the bifurcated conversion feature from the Company's convertible notes issued in 2018 and 2019. None of the Company's non-financial assets or liabilities are recorded at fair value on a non-recurring basis. No transfers between levels have occurred during the periods presented. See Note 7 for further discussion of the convertible notes.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

The Company had no investments or other financial assets recorded at fair value on a recurring basis at December 31, 2019. Further, the Company's bifurcated conversion liability was extinguished upon the conversion of the convertible notes to Series A convertible preferred stock in February 2020.

The following table summarize the Company's financial instruments measured at fair value on a recurring basis at December 31, 2019 and 2020 (in thousands):

	Fair Value Measurement at End of Period Using:			
	Total	Quoted Prices In Active Markets For Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
As of December 31, 2019:				
Liabilities:				
Bifurcated conversion feature liability	$ 112	$ —	$ —	$ 112
As of December 31, 2020:				
Assets:				
Money market accounts(1)	$ 2,100	$ 2,100	$ —	$ —
Certificates of deposit	1,754	1,754	—	—
U.S. Treasury securities	31,958	31,958	—	—
Total	$ 35,812	$ 35,812	$ —	$ —

(1) Included in cash and cash equivalents on the accompanying balance sheets.

The bifurcated conversion feature was recorded as a debt discount and classified as a liability within Level 3 of the fair value hierarchy as the Company is utilizing a significant unobservable input in the price of the underlying preferred shares. The fair value of the conversion feature at December 31, 2019 was determined based on a pricing model that incorporated the actual conversion price determined upon the sale of the Company's Series A preferred stock in February and March 2020. See Note 8 for further discussion of the convertible notes.

The conversion feature is recorded on the Company's balance sheet at fair value each reporting period with other current liabilities. Changes in fair value are recorded as a non-operating expense in the statements of operations, as applicable. The following table provides a reconciliation of liabilities measured at fair value using significant unobservable inputs (Level 3) for the periods presented (in thousands):

	Bifurcated Conversion Feature Liability
Balance at December 31, 2018	$ 62
Issuance of convertible notes with bifurcated conversion feature	50
Balance at December 31, 2019	112
Change in fair value of bifurcated conversion feature	13
Settlement of bifurcated conversion feature	(125)
Balance at December 31, 2020	$ —

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Interest bearing money market accounts and certificates of deposit are valued at amortized cost, which approximates fair value.

5. Investments

The Company's investment policy defines allowable investment securities and establishes guidelines relating to credit quality, diversification, and maturities of its investments to preserve principal and maintain liquidity. In accordance with the Company's investment policy, it has invested funds in marketable securities at December 31, 2020. The Company had no investments in marketable securities at December 31, 2019.

The cost, gross unrealized holding gains, gross unrealized holding losses and fair value of available-for-sale investments by types and classes of security at December 31, 2020 consisted of the following (in thousands):

	December 31, 2020			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Market Value
Certificates of deposits	$ 1,750	$ 4	$ —	$ 1,754
U.S. Treasury securities	31,806	152	—	31,958
Total	$ 33,556	$ 156	$ —	$ 33,712

The Company reviews its investments at each reporting date to identify and evaluate whether a decline in fair value below the amortized cost basis of available-for-sale securities is due to credit-related factors and determines if such unrealized losses are the result of credit losses that requires impairment. Factors considered in determining whether an unrealized loss is the result of a credit loss of other factors include the length of time and extent to which fair value has been less than the cost basis, any changes to the rating of the security by a rating agency, the financial condition and near-term prospects of the issuer, any historical failure of the issuer to make scheduled interest or principal payments, any adverse legal or regulatory events affecting the issuer or issuer's industry, any significant deterioration in economic condition and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value. At December 31, 2020, there were no securities in unrealized loss positions and the Company did not record a reserve for credit losses.

None of the Company's available-for-sale investment securities had maturities of greater than 12 months at December 31, 2020. Further, no gains or losses were realized on sales of investment securities during the years ended December 31, 2019 or 2020. As of December 31, 2020, the Company held seven domestic certificates of deposit with amortized costs at the Federal Deposit Insurance Corporation ("FDIC") insured limit.

Accrued interest receivable on available-for-sale investment securities, included in prepaids and other current assets on the Company's balance sheets, was less than $0.1 million at December 31, 2020. There was no such receivable at December 31, 2019.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

6. Balance Sheet Details

Prepaid expenses and other current assets consisted of the following (in thousands):

	December 31, 2019	December 31, 2020
Prepaid expenses	$ 5	$ 60
Security deposits	8	47
Interest receivable	—	10
Other	—	25
Total	$ 13	$ 142

Property and equipment consisted of the following (in thousands):

	December 31, 2019	December 31, 2020
Laboratory equipment	$ —	$ 76
	—	76
Less accumulated depreciation	—	(5)
Total	$ —	$ 71

Accounts payable and accrued liabilities consisted of the following (in thousands):

	December 31, 2019	December 31, 2020
Accounts payable	$ 2,479	$ 1,399
Accrued research and development costs	225	266
Accrued personnel costs	—	565
Accrued other	19	100
Total	$ 2,723	$ 2,330

7. Grant Revenue

As of December 31, 2020, the Company had been awarded research grants from the NSF, NIH and FARA totaling $1.0 million. The Company recognizes revenue pursuant to these grants, as described further in Note 2, by measuring the progress of the applicable research and development services provided over time, based on the effort the Company expends and costs incurred, relative to the estimated total effort and costs to be incurred under the grant. This results in a percentage that the Company multiplies by the grant award amount to determine the amount of grant revenue to be recognized each period. This approach requires the Company to use judgement and make estimates of future expenditures. If the Company's estimates or judgements change over the course of the term of the grant, it may affect the timing and amount of revenue that it recognizes in the current and future periods.

During the years ended December 31, 2019 and 2020, the Company recognized $0.8 million and $0.2 million of grant revenue from awards by the NSF, NIH and FARA, respectively. Grant funding that

had not yet been recognized as revenue totaling $0.2 million was included as deferred revenue on the Company's balance sheet at December 31, 2019, as the applicable research and development services and costs had not yet been incurred. As of December 31, 2020, the Company had fully recognized all revenue pursuant to the grant awards.

8. Commitments and Contingencies

Notes Payable-Related Party

In January 2019, the Company issued an unsecured promissory note to borrow up to $0.5 million to a co-founder (the "January Note") for working capital. The January Note bore interest at prime plus 4.5%, was scheduled to mature on or before January 30, 2021 and included a final payment of 3% on the amounts advanced at maturity. In February and March 2019, the Company borrowed an aggregate of $0.2 million under the January Note with interest payable at 10% per annum. The Company recorded an immaterial amount of debt issuance costs associated with the January Note. The debt issuance costs and final payment for the advances were amortized to interest expense using the effective interest rate method over the loan term. The principal and interest payable on the January Note were repaid in full in 2020.

In November 2019, the Company issued an unsecured promissory note to borrow up to $0.5 million to a co-founder (the "November Note") for working capital. The November Note bore interest at prime plus 4.5% and matured on or before January 1, 2022 and included a final payment of 3% on the amounts advanced at maturity. In December 2019 and January 2020, the Company borrowed a total of $0.4 million under the November Note with interest payable at 9.25% per annum. The final payment on the advances was amortized to interest expense using the effective interest rate method over the loan term. The principal and interest payable on the November Note were repaid in full in 2020.

Convertible Notes

In May and July 2018, the Company issued $0.3 million of convertible notes to a co-founder for cash with a maturity date on or after February 5, 2021, if not converted earlier. In February 2019, the Company issued an additional $0.2 million of convertible notes to consultants for services rendered with a maturity date on or after May 16, 2020, if not converted earlier. The convertible notes bore interest of 8% per annum and were convertible into equity securities sold at the next financing at 80% of the selling price per share of such equity financing. The conversion features of the convertible notes were recorded as a discount to the notes payable at issuance (see Note 4) and amortized as interest expense over the loan term using the effective interest rate method.

In February 2020, the outstanding principal and accrued interest of the convertible notes totaling $0.5 million were converted into 301,685 shares of the Company's Series A convertible preferred stock at $1.65816 per share. See Note 10 for further discussion.

Lease

In May 2019, as amended, the Company entered into an agreement to lease laboratory space pursuant to a three-month, automatically renewing lease. The lease is subject to annual rent increases of 3% and the Company had paid security deposits totaling $47,000 as of December 31, 2020 which was included in prepaid expenses and other current assets on the Company's balance sheets. Due to

the short-term nature of the lease, it has not been included as an operating lease right of use asset nor as an operating lease liability on the Company's balance sheets. The Company also has access to office space on an as-available basis from time to time pursuant to a consulting agreement. See Note 12 for further discussion.

Rent expense for the years ended December 31, 2019 and 2020 was $19,000 and $146,000, respectively.

Contingencies

From time to time, the Company may become subject to claims or suits arising in the ordinary course of business. The Company accrues a liability for such matters when it is probable that the future expenditures will be made and such expenditures can be reasonably estimated. The Company had no such contingent liabilities as of December 31, 2020.

9. License Agreement

In February 2019, the Company entered into a Human Therapeutics Exclusive License Agreement (the "License Agreement") with the Wisconsin Alumni Research Foundation ("WARF"). Under the License Agreement, the Company licensed the exclusive, worldwide, royalty-bearing, sublicensable, rights to certain WARF patents and the nonexclusive worldwide rights to certain know-how to develop and commercialize products for the prevention, diagnosis and treatment of disease. As consideration for the license, the Company agreed to pay an upfront fee of $250,000, which the Company immediately expensed as research and development expense in the statements of operations and comprehensive loss as there was no alternative future use for the license. The Company paid $25,000 of the upfront fee upon execution of the agreement and the remaining $225,000 was due upon on the earlier of the first anniversary of the License Agreement or the Company receiving a certain level of gross proceeds from an equity financing. At December 31, 2019, the second payment of $225,000 was recorded as an accrued liability on the Company's balance sheet. In February 2020, the Company paid the $225,000 balance upon the initial closing of its Series A preferred stock financing.

Pursuant to the License Agreement, the Company is required to pay $125,000 upon the acceptance of an investigation new drug ("IND") application in the U.S. and will be required to make further aggregate milestone payments of up to $17.5 million upon achievement of certain other regulatory and commercial milestones. The Company may also be required to pay royalties based on annual net product sales in the low single digits on its or its sublicensees' net product sales on a country-by-country and product-by-product basis, upon first subject to a minimum royalty of $0.1 million per calendar year upon first commercial product sale. Further, the Company may be required to pay sublicense fees in the mid-single digits percentage for fees, royalties or other payments earned from the granting of sublicenses to the WARF patents and know how. The Company has paid no milestone or royalty payments as of December 31, 2020.

The Company is responsible for reimbursing WARF for costs incurred in connection with prosecuting and maintaining patent rights that are specific to the License Agreement. Expenses recognized in connection with legal patent fees under this License Agreement were $0.1 million and less than $0.1 million for the years ended December 31, 2019 and 2002, respectively, which were recorded as general and administrative expenses in the statements of operations.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

The Company may terminate the License Agreement with 90 days written notice or for certain breaches of the agreement. WARF may terminate the License Agreement with 90 days written notice if first commercial sale does not occur before December 31, 2031. Unless terminated earlier by the parties, the term of the License Agreement will continue until the last licensed patent expires in all countries.

10. Convertible Preferred Stock and Stockholders' Deficit

Convertible Preferred Stock

In February and March 2020, the Company issued 21,710,814 shares of Series A convertible preferred stock at $2.0727 per share for net cash proceeds of $44.7 million (the "Series A Financing"). In connection with the Series A Financing, the Company issued an additional 301,685 shares of its Series A convertible preferred stock at $1.65816 per share in February 2020 upon the conversion and extinguishment of its convertible notes, as discussed further in Note 8. The Series A convertible preferred shares are not redeemable at the option of the holder or the Company unless pursuant to a deemed liquidation, dissolution, or winding up of the Company (a "Liquidation Event").

The Company's convertible preferred stock has been classified as temporary equity in the accompanying balance sheets in accordance with authoritative guidance for the classification and measurement of potentially redeemable securities whose redemption is based upon certain change in control events outside of the Company's control, including liquidation, sale or transfer of control of the Company. The Company has determined not to adjust the carrying values of the convertible preferred stock to the liquidation preferences of such shares because of the uncertainty of whether or when such events would occur.

The authorized, issued and outstanding shares of convertible preferred stock as of December 31, 2020 consist of the following (in thousands, except share amounts):

	Shares Authorized	Shares Issued and Outstanding	Liquidation Preference	Carrying Value
Series A	22,500,000	22,012,499	$ 45,625	$ 45,356

Dividends

The holders of the convertible preferred stock are entitled to receive noncumulative dividends at the rate of 8% per annum of the applicable original stock purchase price when and if declared by the Company's board of directors, and in preference and in priority to any dividends on common stock. No dividends have been declared as of December 31, 2020.

Liquidation Preferences

In the event of any Liquidation Event, the holders of convertible preferred stock shall be entitled to receive, prior and in preference to any distribution of any of the assets or surplus funds to the holders of common stock, an amount equal to the greater of the convertible preferred stock original issue price plus any declared and unpaid dividend or such amount per share were the convertible preferred stock converted into common stock. Liquidation payments to the holders of convertible preferred stock have priority and are made in preference to any payments to the holders of common stock.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Voting Rights

The holder of each share of convertible preferred stock is entitled to one vote for each share of common stock into which it would convert.

Conversion

The shares of convertible preferred stock are convertible into one share of common stock at any time, at the option of the holder, subject to certain antidilutive adjustments, including stock splits, combinations, common stock dividends and distributions, reclassification, recapitalization, merger, and consolidation. As of December 31, 2020, all of the shares of convertible preferred stock would automatically convert into shares of common stock upon the closing of an initial public offering at a price of at least $10.14 per share resulting in gross proceeds of at least $50.0 million. Pursuant to the Company's Series B convertible preferred stock financing in January 2021, the Company's Certificate of Incorporation was amended and restated to provide that all shares of convertible preferred stock will be automatically converted into shares of common stock upon the closing of an initial public offering at a price of at least $13.04 per share resulting in gross proceeds of at least $50.0 million. See Note 13 for further discussion.

Authorized Shares

In connection with the completion of the Series A financing, in February 2020, the Company amended its Certificate of Incorporation to authorize 60,000,000 shares of common stock, par value $0.0001 per share, and 22,500,000 shares of preferred stock, par value $0.0001 per share, respectively. Pursuant to the Company's Series B convertible preferred stock financing, in January 2021, the Company's Certificate of Incorporation was amended and restated to authorize a total of 80,000,000 shares of common stock, par value $0.0001 per share, and 41,096,478 shares of preferred stock, par value $0.0001 per share. See Note 13 for further discussion.

Founders Stock

During 2017, the Company issued 7,668,711 shares of its common stock at $0.00003 per share for cash to a co-founder and 7,668,710 shares of its common stock, valued at $0.00003 per share, to another co-founder in exchange for the transfer of certain worldwide intellectual patent rights.

2018 Equity Incentive Plan

In June 2018, the Company adopted the 2018 Equity Incentive Plan, as amended (the "2018 Plan"), which provides for the grant of incentive stock options, non-statutory stock options, restricted stock awards, restricted stock unit awards and stock appreciation rights to its employees, members of its board of directors and consultants. As of December 31, 2020, a total of 3,955,438 shares had been authorized for issuance under the 2018 Plan. Recipients of stock options are eligible to purchase shares of the Company's common stock at an exercise price equal to the estimated fair market value of such stock on the date of grant. The maximum term of options granted under the 2018 Plan is ten years and, in general, the options issued under the plan vest over a four-year period from the vesting commencement date. The Company issues new shares of common stock upon the exercise of stock options and issuance of restricted stock awards.

Pursuant to the 2018 Plan, the Company has issued shares of restricted common stock to employees, consultants and members of its board of directors. Additionally, certain stock options granted pursuant to the 2018 Plan provide for the right to elect to exercise unvested options early in

exchange for restricted shares of common stock. These restricted shares of common stock generally vest over a four-year period and are subject to repurchase by the Company at the original purchase price or, in certain instances the fair market value if such fair market value is lower than the purchase price, in the event the recipient's service is terminated either voluntarily or involuntarily prior to vesting.

A summary of the Company's restricted shares of common stock and unvested stock liability, recorded as a long-term liability on the Company's balance sheets, for the periods presented were as follows (in thousands, except share data):

	Shares	Liability
Balance at December 31, 2018	807,233	$ —
Restricted shares issued	306,747	1
Vested shares	(302,712)	—
Balance at December 31, 2019	811,268	1
Shares early exercised	153,373	145
Vested shares	(317,688)	(1)
Balance at December 31, 2020	646,953	$ 145

A summary of the Company's stock option activity for the periods presented was as follows (in thousands, except year, share and per share data):

	Number of Options and Awards Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding at December 31, 2018	—	$ —		
Granted	30,674	$ 0.003		
Exercised	—	$ —		
Canceled	—	$ —		
Outstanding at December 31, 2019	30,674	$ 0.003	9.95	$ —
Granted	1,723,913	$ 0.93		
Exercised	(153,373)	$ 0.95		
Canceled	—	$ —		
Outstanding at December 31, 2020	1,601,214	$ 0.91	9.71	$ 8,468
Vested at December 31, 2019	—	$ —	—	$ —
Exercisable at December 31, 2019	—	$ —	—	$ —
Vested at December 31, 2020	10,224	$ 0.003	8.94	$ 63
Exercisable at December 31, 2020	654,395	$ 0.93	9.82	$ 3,444

Common Stock Reserved for Future Issuance

Common stock reserved for future issuance at December 31, 2020 was as follows:

	Shares
Conversion of preferred stock	13,504,598
Common stock options outstanding	1,601,214
Equity plan shares available for future issuance under the 2018 Plan	1,086,871
	16,192,683

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

11. Income Taxes

The Company is subject to taxation in the United States and various state jurisdictions. All of the Company's tax years are subject to examination by federal and state tax authorities due to the carryforward of unutilized net operating losses and research and development credits. The Company's policy is to recognize interest and penalties related to income tax matters as tax expense. The Company had no accrued interest or penalties related to income tax matters on its balance sheets at December 31, 2019 or 2020. and has not recognized interest or penalties in its statements of operations and comprehensive loss for the years ended December 31, 2019 and 2020, respectively. Further, the Company is not currently under examination by any federal, state or local tax authority.

At December 31, 2020, the Company had federal and state net operating loss ("NOL") carryforwards of $10.1 million and $10.3 million, respectively. Federal NOL carryforwards totaling $0.1 million begin to expire in 2037, unless previously utilized, and federal NOL carryforwards of $10.0 million generated after 2017, may be carryforward indefinitely but can only be utilized to offset 80% of future taxable income. State NOL carryforwards totaling $10.3 million begin to expire in 2037, unless previously utilized. In addition, the Company also has federal and state research and development ("R&D") credit carryforwards totaling $41,000 and $155,000, respectively. The federal R&D credit carryforwards will begin to expire in 2038 unless previously utilized. The state R&D credit carryforwards do not expire.

Utilization of the Company's NOL and R&D credit carryforwards may be subject to substantial annual limitations in the event a cumulative ownership change has occurred, or that could occur in the future, as required by Section 382 of the Internal Revenue Code of 1986, as amended (the "Code"). In general, an "ownership change," as defined by Section 382 of the Code, results from a transaction, or series of transactions over a three-year period, resulting in an ownership change of more than 50% of the outstanding common stock of a company by certain stockholders or public groups. Such an ownership changes may limit the amount of NOL and R&D credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. The Company has not completed such an ownership change analysis pursuant to Section 382 of the Code and therefore has established a full valuation allowance as the realization of such deferred tax assets has not met the more likely than not threshold requirement. If ownership changes have occurred or occurs in the future, the amount of remaining tax attribute carryforwards available to offset taxable income and income tax expense in future years may be restricted or eliminated. If eliminated, the related asset would be removed from deferred tax assets with a corresponding reduction in the valuation allowance. Due to the existence of the valuation allowance, limitations created by future ownership changes, if any, will not impact the Company's effective tax rate.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Significant components of the Company's net deferred tax assets at December 31, 2019 and 2020 were as follows (in thousands):

	December 31, 2019	December 31, 2020
Deferred tax assets:		
Net operating losses	$ 787	$ 2,836
Research and development credits	82	153
Depreciation and amortization	114	80
Stock-based compensation	—	25
Accrued expenses	56	116
Total gross deferred tax assets	1,039	3,210
Valuation allowance	(1,039)	(3,177)
Total deferred tax assets	—	33
Deferred tax liabilities:		
Other	—	(33)
Total deferred tax liabilities	—	(33)
Net deferred tax assets	$ —	$ —

A reconciliation of the Company's income tax expense (benefit) to the amount computed by applying the federal statutory income tax rate for the periods presented were as follows (in thousands):

	Years Ended December 31, 2019	Years Ended December 31, 2020
Expected tax benefit at federal statutory rate	$ (430)	$ (1,738)
State income taxes, net of federal benefit	(131)	(552)
Debt financing	25	6
Research and development credits	(30)	(71)
Other	14	174
Change in valuation allowance	552	2,181
Provision for income taxes	$ —	$ —

The Company recognizes a tax benefit from an uncertain tax position when it is more likely than not that the position will be sustained upon examination by tax authorities. The Company does not expect that there will be a significant change in the unrecognized tax benefits over the next twelve months. Further, due to the existence of the valuation allowance, future changes in the Company's unrecognized tax benefits will not impact the effective tax rate

The following table summarizes the changes to the Company's unrecognized tax benefits for the periods presented (in thousands):

	Years Ended December 31, 2019	Years Ended December 31, 2020
Balance at beginning of period	$ 5	$ 9
Increase to current year tax positions	4	6
Balance at end of period	$ 9	$ 15

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Effective January 1, 2020, the Company adopted ASU 2019-12. Under ASU 2019-12, the Company, having a full valuation and a loss in continuing operations, will no longer include the impacts of items in other comprehensive income in determining intra-period allocation of tax expense for continuing operations. There was no cumulative effect to be recognized in connection with the adoption of ASU 2019-12. For the twelve months ended December 31, 2020, the Company applied the tax allocation rules of ASU 2019-12 to the unrealized gains on available-for-sale investments recognized in other comprehensive income, which did not have a material impact on the consolidated financial statements or related disclosures.

On March 27, 2020, the Coronavirus Aid, Relief and Economic Security ("CARES") Act was signed into law. The CARES Act is an emergency economic stimulus package that includes spending and tax breaks to strengthen the U.S. economy and fund a nationwide effort to curtail the effect of COVID-19. While the CARES Act provides sweeping tax changes in response to the COVID-19 pandemic, some of the more significant provisions which may impact the Company's future financial statements include removal of certain limitations on utilization of NOLs, increasing the loss carryback period for certain losses to five years, as well as amending certain provisions of the previously enacted JOBS Act. The Company has not recognized the provisional tax impacts related to the CARES Act in relation to its financial statements for the year ended December 31, 2020.

12. Related Party Transactions

Consulting Agreements

In January 2019, the Company entered into an agreement with the Marlinspike Group, LLC ("Marlinspike Group") for research support, management, and business consulting services (the "2019 Consulting Agreement"). Further, Marlinspike Group provides the use of approximately 2,120 square feet of its office space in Carlsbad, California to the Company on an as-available basis from time to time pursuant to the agreement. The Company's Executive Chairperson, co-founder and member of its board of directors is an executive officer of Marlinspike Group and, the Company's Chief Operating Officer was an executive officer of Marlinspike Group until February 2020. From December 2017 to December 2018, the Company was subject to a similar agreement with Marlinspike Group, Inc.

The term of the 2019 Consulting Agreement was for a one-year period, subject to automatic one-month renewals unless terminated upon 14 days' written notice. In March 2020, the Consulting Agreement was terminated and replaced with an amended consulting agreement (the "2020 Consulting Agreement"), which provides for the similar services and use of office space for a monthly fee of $20,000. Pursuant to the terms of the 2020 Consulting Agreement, it shall remain in effect until otherwise terminated. Termination may occur at any time upon mutual agreement or unilaterally upon 30 days' written notice. If the Company unilaterally terminates the 2020 Consulting Agreement for any reason other than cause, it would be subject to a $240,000 termination fee. The Company cannot determine when, or if, such a termination will occur and hence has not recorded a liability for the fee.

Expenses recognized by the Company under the Consulting Agreement during the periods presented were as follows (in thousands):

	Year Ended December 31,	
	2019	2020
Research and development	$ 185	$ 8
General and administrative	580	292
Total expenses	$ 765	$ 300

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

As of December 31, 2019, the Company had accounts payable due to Marlinspike Group of $1.8 million, including amounts due for services provided prior to 2019. As of December 31, 2020, the Company had accounts payable due to Marlinspike Group of less than $0.1 million.

In December 2017, the Company entered into a consulting agreement with a co-founder to provide consulting services and oversee certain the research and development activities. Pursuant to the agreement, as amended, the co-founder performs these services for a monthly fee of $15,000. During 2020, the Company incurred less than $0.1 million pursuant to the agreement, which were recorded as research and development expenses in the statements of operations. No similar expenses were incurred in 2019.

Convertible Notes and Notes Payable

In May and July 2018, the Company issued a total of $0.3 million of convertible notes to a co-founder for cash with a maturity date on or after February 5, 2021, if not converted earlier. The notes bore interest of 8% per annum and were convertible into equity securities sold at the next financing at 80% of the selling price per share of such equity financing. In February 2020, these notes converted into 171,025 shares of the Company's convertible preferred stock pursuant to the closing of the Series A preferred stock financing. See Notes 8 and 13 for further discussion.

Further, the Company has issued unsecured promissory notes to this co-founder for working capital as described further in Note 8.

13. Subsequent Events

Convertible Preferred Stock Financing

In January 2021, the Company issued 19,083,979 shares of its Series B convertible preferred stock at $6.55 per share for net cash proceeds of approximately $124.7 million ("the Series B Financing"). The holders of the Series B convertible preferred stock are entitled to the same general provisions as the Series A convertible preferred stock holders.

In connection with the Series B Financing, the Company's Certificate of Incorporation was amended and restated to provide that all shares of convertible preferred stock will be automatically converted into shares of common stock upon the closing of an initial public offering at a price of at least $13.04 per share resulting in gross proceeds of at least $50.0 million. Further, the Company amended its Certificate of Incorporation to authorize a total of 80,000,000 shares of common stock, par value $0.0001 per share, and 41,096,478 shares of preferred stock, par value $0.0001 per share.

Related Party Lease

In February 2021, the Company entered into a lease agreement with Crossing Holdings, LLC (Crossing Holdings) to rent approximately 12,370 square feet of laboratory and office space. The Company's Executive Chairperson, co-founder and member of its board of directors, is the sole member and Manager of Crossing Holdings. The anticipated delivery date of the space is September 1, 2021 and the lease is expected to commence at that time. The term of the lease is 72 months after commencement with an option to extend the lease term for a period of three years. Annual rent will be $0.8 million per year, subject to annual increases of 3%, plus the Company's share of operating expenses and taxes.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

Lease

In March 2021, the Company further amended its lease for laboratory space and effective April 15, 2021, will be subject to monthly rent payments of $45,000. In addition, the security deposit required by the lease will increase to $90,000.

Reverse Stock Split

In March 2021, the Company's board of directors approved an amendment to the Company's certificate of incorporation to effect a reverse split of shares of the Company's common stock on a one-for-1.63 basis, which was effected on March 22, 2021 (the "Reverse Stock Split"). The number of authorized shares and the par values of the common stock and redeemable convertible preferred stock were not adjusted as a result of the Reverse Stock Split. In connection with the Reverse Stock Split, the conversion ratio for the Company's outstanding redeemable convertible preferred stock was proportionately adjusted such that the common stock issuable upon conversion of such preferred stock was decreased in proportion to the Reverse Stock Split. All references to common stock and options to purchase common stock share data, per share data, and related information contained in the consolidated financial statements have been retroactively adjusted to reflect the effect of the Reverse Stock Split for all periods presented.

2018 Equity Incentive Plan

From January 1, 2021 through March 1, 2021, the Company granted an aggregate of 328,217 common stock options to certain of its employees and consultants under the 2018 Plan at a weighted-average exercise price of $7.71 per share.

2021 Equity Incentive Award Plan

In March 2021, the Company's board of directors and stockholders adopted the 2021 Equity Incentive Award Plan (the "2021 Plan"). The 2021 Plan will become effective upon the date of the underwriting agreement related to the IPO. Under the 2021 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units, performance stock awards, performance cash awards and other forms of stock awards to employees, directors and consultants, including employees and consultants of the Company's affiliates. A total of 6,118,648 new shares of common stock were approved to be initially reserved for issuance under the 2021 Plan. The number of shares reserved that are remaining under the 2018 Plan as of the effective date of the 2021 Plan will be added to the shares initially reserved under the 2021 Plan upon its effectiveness. In addition, the number of shares of common stock available for issuance under the 2021 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2022 (assuming the 2021 Plan becomes effective in 2021) through January 1, 2031, in an amount equal to 5% of the total number of shares of the Company's common stock on the last day of the calendar month before the date of each automatic increase, or a lesser number of shares determined by the Company's board of directors.

In March 2021, the Company's board of directors approved the grant of options to purchase 15,000 shares of common stock to each of the non-employee directors of the Company. These awards will be issued under the 2021 Equity Incentive Plan and have been approved to be granted contingent and effective upon the execution and delivery of the underwriting agreement relating to the proposed IPO. The per share exercise price will be equal to the price per share at which the Company's common stock is first sold to the public in the IPO.

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

2021 Employee Stock Purchase Plan

In March 2021, the Company's board of directors and stockholders adopted the 2021 Employee Stock Purchase Plan (the "ESPP"). The ESPP will become effective immediately prior to and contingent upon the date of the underwriting agreement related to the IPO. The ESPP permits eligible employees who elect to participate in an offering under the ESPP to have up to 15% of their eligible earnings withheld, subject to certain limitations, to purchase shares of common stock pursuant to the ESPP. The price of common stock purchased under the ESPP is equal to 85% of the lower of the fair market value of the common stock at the commencement date of each offering period or the relevant date of purchase. A total of 600,000 shares of common stock were approved to be initially reserved for issuance under the ESPP. In addition, the number of shares of common stock available for issuance under the ESPP will automatically increase on January 1 of each calendar year, starting on January 1, 2022 (assuming the ESPP becomes effective in 2021) through January 1, 2031, in an amount equal to the lesser of (i) 1% of the total number of shares of the Company's common stock on the last day of the calendar month before the date of each automatic increase and (ii) 1,200,000 shares; provided that before the date of any such increase, the Company's board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). As of the date hereof, no shares of the Company's common stock have been purchased under the ESPP.

Co-Founder Consulting Agreement

In March 2021, in accordance with the terms of the December 2017 consulting agreement the Company has with a co-founder as described in Note 12, the Company's board of directors approved extending the monthly fee under the agreement indefinitely, subject to any shorter period as determined in the sole discretion of the board of directors.

401K Plan

In March 2021, the Company's board approved a defined contribution 401(k) plan available to eligible employees. Employee contributions are voluntary and are determined on an individual basis, limited to the maximum amount allowable under federal tax regulations. The Company, at its discretion, may make certain matching contributions to the 401(k) plan. To date, no contributions to the 401(k) plan have been made by the Company.

COVID-19

The global COVID-19 pandemic continues to rapidly evolve, and the Company will continue to monitor the COVID-19 situation closely. The extent of the impact of the COVID-19 on the Company's business, operations and clinical development timelines and plans remains uncertain, and will depend on certain developments, including the duration and spread of the outbreak and its impact on its contract research organizations, third-party manufacturers, and other third parties with whom the Company does business, as well as its impact on regulatory authorities and its key scientific and management personnel. The ultimate impact of the COVID-19 pandemic or a similar health epidemic is highly uncertain and subject to change. To the extent possible, the Company is conducting business as usual, with necessary or advisable modifications to employee travel and most of its office employees working remotely. The Company will continue to actively monitor the rapidly evolving situation related to COVID-19 and may take further actions that alter its operations, including those that may be required by federal, state or local authorities, or that the Company determines are in the best interests of its employees and other third parties with whom it does business. At this point, the extent to which

DESIGN THERAPEUTICS, INC.
NOTES TO FINANCIAL STATEMENTS—(Continued)

the COVID-19 pandemic may affect the Company's business, operations and clinical development timelines and plans, including the resulting impact on its expenditures and capital needs, remains uncertain and is subject to change.

12,000,000 Shares

Design Therapeutics, Inc.

Common Stock

Goldman Sachs & Co. LLC **SVB Leerink** **Piper Sandler**

Through and including April 19, 2021 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.