UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2025

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission File Number: 001-40288

Design Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**82-3929248**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
6005 Hidden Valley Road, Suite 110 Carlsbad, California	**92011**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (858) 293-4900

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	DSGN	The Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The number of outstanding shares of the registrant's common stock, $0.0001 par value per share, as of October 31, 2025, was 56,963,757.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (this "Quarterly Report") contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our plans to research, develop and commercialize our product candidates;
- our expectations for clinical development of our program in Friedrich ataxia and announcing data therefrom and the timing thereof;
- the side effect profile observed in nonclinical testing and early clinical testing of DT-216P2 being indicative of the side effect profile that may be expected in the future;
- the initiation, progress, success, cost and timing of our nonclinical studies, clinical trials and product development activities;
- the therapeutic potential of our product candidates, and the disease indications for which we intend to develop our product candidates;
- our ability and timing to advance our product candidates into, and to successfully initiate, conduct, enroll and complete, clinical trials;
- our ability to manufacture our product candidates for clinical development and, if approved, for commercialization, and the timing and costs of such manufacture;
- the performance of third parties in connection with the development and manufacture of our product candidates, including third parties conducting our nonclinical studies and clinical trials as well as third-party suppliers and manufacturers;
- our ability to obtain funding for our operations, including funding necessary to initiate and complete clinical trials of our product candidates;
- the size and growth of the potential markets for our product candidates and our ability to serve those markets;
- the potential scope, duration and value of our intellectual property rights;
- our ability, and the ability of our licensors, to obtain, maintain, defend and enforce intellectual property rights protecting our platform technologies and product candidates, and our ability to develop and commercialize our product candidates without infringing the proprietary rights of third parties;
- our ability to recruit and retain key personnel;
- the effects of macroeconomic factors on our operations;
- our expected use of the net proceeds from our initial public offering; and
- other risks and uncertainties, including those described under Part II, Item 1A, "Risk Factors" of this Quarterly Report.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part II, Item 1A, "Risk Factors" of this Quarterly Report. 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 Quarterly Report, 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 you are cautioned not to unduly rely upon these statements.

Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

Unless the context otherwise indicates, references in this Quarterly Report to the terms "Design", "the Company", "we", "our", and "us" refer to Design Therapeutics, Inc.

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 Quarterly Report on Form 10-Q are the property of Design Therapeutics, Inc. All other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESS

An investment in shares of our common stock involves a high degree of risk. Below is a list of the more significant risks associated with our business. This summary does not address all of the risks that we face. Additional discussion of the risks listed in this summary, as well as other risks that we face, are set forth under Part II, Item 1A, "Risk Factors" in this Quarterly Report. Some of the material risks associated with our business include the following:

- 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 early in our development efforts, and we have only two product candidates in clinical development, with all of our other research programs currently in the nonclinical or discovery stage. We have a limited history of conducting clinical trials to test our product candidates in humans.
- Nonclinical and clinical development involves a lengthy and expensive process with uncertain timelines and outcomes, and results of nonclinical studies and clinical trials may not be predictive of future trial results. If development of our 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.
- 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 clinical trials, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.
- Early, interim, topline and preliminary data from our nonclinical studies or 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.
- 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.
- International trade policies, including tariffs, sanctions and trade barriers, may adversely affect our business, financial condition, results of operations and growth prospects.

- A health epidemic or 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.
- We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.
- We may rely on third parties to conduct, supervise, and monitor our clinical trials and perform some of our research and nonclinical 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 contract with third parties for the manufacturing and supply of our product candidates for use in nonclinical testing and clinical trials, which supply may become limited or interrupted or may not be of satisfactory quality and quantity.
- 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 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.
- 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 may not realize the benefits of any acquisitions, in-license or strategic alliances that we enter into.
- 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.
- 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 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.
- Disruptions to the operations of the FDA, the SEC, other U.S. governmental agencies or comparable foreign regulatory authorities caused by funding shortages, leadership changes, staffing cuts or other staffing shortages, along with uncertainty regarding the potential for new initiatives, laws, regulations, policies and guidance affecting our product candidates or other aspects of our business, could materially and adversely affect our business.
- 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 may not be able to protect our intellectual property rights throughout the world.
- We may rely on trade secrets 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.
- The price of our common stock could be subject to volatility related or unrelated to our operations.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS
(in thousands, except share and par value data)

	September 30, 2025 (unaudited)	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 15,088	$ 22,563
Investment securities	190,882	222,914
Prepaid expenses and other current assets	3,059	2,563
Total current assets	209,029	248,040
Property and equipment, net	1,104	1,410
Right-of-use asset, related party	1,637	2,216
Other assets	—	427
Total assets	$ 211,770	$ 252,093
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,913	$ 2,186
Accrued expenses and other current liabilities (including related party amounts of $866 and $800 respectively)	9,260	6,276
Total current liabilities	11,173	8,462
Operating lease liability, net, related party	877	1,534
Total liabilities	12,050	9,996
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Common stock, $0.0001 par value; 200,000,000 shares authorized at September 30, 2025 and December 31, 2024; 56,963,757 and 56,754,341 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	6	6
Additional paid-in capital	480,365	468,830
Accumulated deficit	(281,009)	(227,214)
Accumulated other comprehensive income	358	475
Total stockholders' equity	199,720	242,097
Total liabilities and stockholders' equity	$ 211,770	$ 252,093

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development (including related party amounts of $228, $224, $686 and $670, respectively)	$ 14,589	$ 11,876	$ 45,704	$ 32,193
General and administrative (including related party amounts of $130, $134, $395 and $405, respectively)	4,722	4,370	15,594	13,496
Total operating expenses	19,311	16,246	61,298	45,689
Loss from operations	(19,311)	(16,246)	(61,298)	(45,689)
Interest income	2,314	3,207	7,503	9,752
Net loss	$ (16,997)	$ (13,039)	$ (53,795)	$ (35,937)
Net loss per share, basic and diluted	$ (0.30)	$ (0.23)	$ (0.95)	$ (0.64)
Weighted-average shares of common stock outstanding, basic and diluted	56,950,999	56,620,731	56,856,779	56,555,312

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF COMPREHENSIVE LOSS
(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net loss	$ (16,997)	$ (13,039)	$ (53,795)	$ (35,937)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale securities	53	1,131	(117)	852
Comprehensive loss	$ (16,944)	$ (11,908)	$ (53,912)	$ (35,085)

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share data)
(unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at June 30, 2025	56,948,126	$ 6	$ 476,738	$ 305	$ (264,012)	$ 213,037
Exercises of stock options	15,631	—	37	—	—	37
Stock-based compensation	—	—	3,590	—	—	3,590
Unrealized gain on investments	—	—	—	53	—	53
Net loss	—	—	—	—	(16,997)	(16,997)
Balance at September 30, 2025	56,963,757	$ 6	$ 480,365	$ 358	$ (281,009)	$ 199,720
Balance at December 31, 2024	56,754,341	$ 6	$ 468,830	$ 475	$ (227,214)	$ 242,097
Exercises of stock options	29,968	—	60	—	—	60
Issuance of common stock under employee stock purchase plan	179,448	—	361	—	—	361
Stock-based compensation	—	—	11,114	—	—	11,114
Unrealized loss on investments	—	—	—	(117)	—	(117)
Net loss	—	—	—	—	(53,795)	(53,795)
Balance at September 30, 2025	56,963,757	$ 6	$ 480,365	$ 358	$ (281,009)	$ 199,720
Balance at June 30, 2024	56,620,014	$ 6	$ 461,977	$ (217)	$ (200,524)	$ 261,242
Exercises of stock options	1,023	—	1	—	—	1
Stock-based compensation	—	—	3,118	—	—	3,118
Unrealized gain on investments	—	—	—	1,131	—	1,131
Net loss	—	—	—	—	(13,039)	(13,039)
Balance at September 30, 2024	56,621,037	$ 6	$ 465,096	$ 914	$ (213,563)	$ 252,453
Balance at December 31, 2023	56,473,598	$ 6	$ 455,245	$ 62	$ (177,626)	$ 277,687
Exercises of stock options	28,088	—	27	—	—	27
Issuance of common stock under employee stock purchase plan	119,351	—	223	—	—	223
Stock-based compensation	—	—	9,601	—	—	9,601
Unrealized gain on investments	—	—	—	852	—	852
Net loss	—	—	—	—	(35,937)	(35,937)
Balance at September 30, 2024	56,621,037	$ 6	$ 465,096	$ 914	$ (213,563)	$ 252,453

The accompanying notes are an integral part of these financial statements.

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF CASH FLOWS
(in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2025	2024
Cash flows from operating activities		
Net loss	$ (53,795)	$ (35,937)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation	466	447
Stock-based compensation	11,114	9,601
Amortization of premiums on investment securities, net	(2,741)	(4,760)
Non-cash lease expense	(13)	9
Deferred financing costs	427	—
Change in operating assets and liabilities:		
Prepaid expense and other assets	(496)	(379)
Accounts payable and other liabilities	2,646	(2,226)
Net cash used in operating activities	(42,392)	(33,245)
Cash flows from investing activities		
Purchases of investment securities	(204,155)	(165,802)
Proceeds from maturities of investment securities	238,810	208,265
Purchases of property and equipment	(159)	(340)
Net cash provided by investing activities	34,496	42,123
Cash flows from financing activities		
Issuance of common stock through employee stock purchase plan	361	223
Proceeds from the exercise of stock options	60	27
Net cash provided by financing activities	421	250
Net (decrease) increase in cash and cash equivalents	(7,475)	9,128
Cash and cash equivalents at beginning of period	22,563	21,200
Cash and cash equivalents at end of period	$ 15,088	$ 30,328

The accompanying notes are an integral part of these 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 clinical-stage biopharmaceutical company pioneering the research and development of GeneTAC® molecules, which are a novel class of small-molecule gene targeted chimera therapeutic candidates designed to be disease-modifying by addressing the underlying cause of diseases caused by inherited nucleotide repeat expansion mutations. The Company's lead product candidate is in Friedreich ataxia (FA), its second product candidate is in Fuchs endothelial corneal dystrophy (FECD), its third product candidate is in myotonic dystrophy type-1 (DM1), and it is also advancing GeneTAC® programs to address other diseases.

Liquidity and Capital Resources

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. The Company has incurred net operating losses since inception and had an accumulated deficit of $281.0 million as of September 30, 2025. The Company had cash, cash equivalents and investment securities of $206.0 million as of September 30, 2025, and has not generated positive cash flow from operations.

Management expects to incur net losses for the foreseeable future. There can be no assurance that the Company will ever earn revenues or achieve profitability, or if achieved, that they will be sustained on a continuing basis. In addition, the research, product development and clinical development activities as well as the commercialization of the Company's products, if approved, will require significant additional financing. The Company may be unable to secure such financing when needed, or if available, such financings may be under terms that are unfavorable to the Company or the current stockholders. If the Company is unable to raise additional funds when needed, it may be required to delay, reduce the scope of, or eliminate development programs, which may adversely affect its business and operations. The Company's currently available cash, cash equivalents and investment securities as of September 30, 2025 are sufficient to meet its anticipated cash requirements for more than 12 months following the date the financial statements are issued.

2. Basis of Presentation and Summary of Significant Accounting Policies

Basis of Presentation and Use of Estimates

The accompanying unaudited interim condensed financial statements have been prepared in accordance with U.S. generally accepted accounting principles and following the requirements of the United States Securities and Exchange Commission ("SEC") for interim reporting. The Company's financial statements include all adjustments, consisting of only normal recurring adjustments, 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 and cash flows for the periods presented.

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 accruals for research and development expenses. 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 expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker ("CODM") in making decisions regarding resource allocation and assessing performance. The Company operates in one operating and reportable segment, Design Therapeutics, Inc., which is focused on utilizing the Company's proprietary GeneTAC® platform to design and develop therapeutic candidates for inherited diseases driven by nucleotide repeat expansion.

The Company's CODM is its Chief Executive Officer. The CODM manages the Company's operations as one segment for the purposes of assessing performance and making operating decisions. The Company's management of segment profit or loss and assets are evaluated at the consolidated level, and it manages its research activities on a consolidated basis.

Issued Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU 2023-09, ASC Topic 740, *Income Taxes: Improvements to Income Tax Disclosures,* which requires disaggregated information about a reporting entity's effective tax rate reconciliation as well as information on income taxes paid. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company is currently evaluating ASU 2023-09 and does not expect it to have a material effect on its financial statement disclosures.

In November 2024, the FASB issued Accounting Standard Update ("ASU") 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*, which is intended to enhance transparency into the nature and function of expenses. The amendments require that on an annual and interim basis, entities disclose disaggregated operating expense information about specific expense categories. The amendments are effective for fiscal years beginning after December 15, 2026, and for interim periods within fiscal years beginning after December 15, 2027. Early adoption is permitted and the amendments may be applied either prospectively or retrospectively. The Company is currently evaluating the impact that the updated standard will have on its financial statement 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. 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 method. Dilutive common stock equivalents are comprised of stock options outstanding under the Company's equity incentive plans and employee stock purchase rights under the Company's 2021 Employee Stock Purchase Plan ("ESPP"), as applicable. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to the Company's net loss position.

The following table sets forth the outstanding, potentially dilutive securities that have been excluded in the calculation of diluted net loss per share because their inclusion would be anti-dilutive:

	September 30, 2025	September 30, 2024
Stock options	13,859,522	10,634,334
Employee stock purchase plan	165,206	133,358
Total	14,024,728	10,767,692

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 following table summarizes the Company's financial instruments measured at fair value on a recurring basis at September 30, 2025 and December 31, 2024 (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 September 30, 2025:				
Assets:				
Money market funds[(1)]	$ 13,256	$ 13,256	$ —	$ —
Certificates of deposit	200	200	—	—
U.S. Treasury securities	178,310	178,310	—	—
U.S. Government agency securities	12,372	—	12,372	—
Total	$ 204,138	$ 191,766	$ 12,372	$ —
As of December 31, 2024:				
Assets:				
Money market funds[(1)]	$ 20,800	$ 20,800	$ —	$ —
Certificates of deposit	2,898	2,898	—	—
U.S. Treasury securities	197,528	197,528	—	—
U.S. Government agency securities	22,488	—	22,488	—
Total	$ 243,714	$ 221,226	$ 22,488	$ —

(1) Included in cash and cash equivalents on the accompanying balance sheets.

Interest bearing money market accounts and certificates of deposit are valued at amortized cost, which approximates fair value. 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. The Company obtains the fair value of its available-for-sale debt securities from a professional pricing service. Level 2 securities are valued using quoted market prices for similar instruments, non-binding market prices that are corroborated by observable market data and include our investments in marketable debt instruments of government sponsored enterprises. None of the Company's non-financial assets or liabilities are recorded at fair value on a non-recurring basis and no transfers between levels have occurred during the periods presented.

5. Investment Securities

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. The Company's investment securities consisted of the following at September 30, 2025 and December 31, 2024 (in thousands):

		As of September 30, 2025				
	Maturity	Amortized Cost	Unrealized Gains	Unrealized Losses	Allowance for Credit Losses	Estimated Fair Market Value
Certificates of deposits	Within 1 year	$ 200	$ —	$ —	$ —	$ 200
U.S. Treasury securities	Within 1 year	145,384	218	(6)	—	145,596
U.S. Treasury securities	Greater than 1 year	32,565	152	(3)	—	32,714
U.S. Government agency securities	Greater than 1 year	12,375	—	(3)	—	12,372
Total		$ 190,524	$ 370	$ (12)	$ —	$ 190,882

		As of December 31, 2024				
	Maturity	Amortized Cost	Unrealized Gains	Unrealized Losses	Allowance for Credit Losses	Estimated Fair Market Value
Certificates of deposits	Within 1 year	$ 2,884	$ 14	$ —	$ —	$ 2,898
U.S. Treasury securities	Within 1 year	172,178	391	—	—	172,569
U.S. Government agency securities	Within 1 year	7,500	—	(7)	—	7,493
U.S. Treasury securities	Greater than 1 year	24,878	83	(2)	—	24,959
U.S. Government agency securities	Greater than 1 year	14,999	1	(5)	—	14,995
Total		$ 222,439	$ 489	$ (14)	$ —	$ 222,914

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 require impairment. Factors considered in determining whether an unrealized loss is the result of a credit loss or other factors include the extent to which the fair value is 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.

There were 14 and 12 securities in an unrealized loss position at September 30, 2025 and December 31, 2024, respectively. The Company determined that unrealized losses on its available-for-sale investment securities were primarily attributable to changes in interest rates. Each security remained at a high credit quality rating. Further, there had been no adverse conditions noted for any of the issuers and the Company does not intend to sell any of the securities prior to maturity. As such, an allowance for credit losses has not been recognized as of September 30, 2025 or December 31, 2024.

The following tables present available-for-sale investments that were in an unrealized loss position as of September 30, 2025 and December 31, 2024, aggregated by security type and length of time in a continuous unrealized loss position (in thousands):

	As of September 30, 2025					
	Less than 12 Months		12 Months or Greater		Total	
	Estimated Fair Market Value	Unrealized Losses	Estimated Fair Market Value		Estimated Fair Market Value	Unrealized Losses
U.S. Treasury securities	$ 22,527	$ (9)	$ —		$ 22,527	$ (9)
U.S. Government agency securities	9,872	(3)	—		9,872	(3)
Total	$ 32,399	$ (12)	$ —		$ 32,399	$ (12)

	As of December 31, 2024					
	Less than 12 Months		12 Months or Greater		Total	
	Estimated Fair Market Value	Unrealized Losses	Estimated Fair Market Value		Estimated Fair Market Value	Unrealized Losses
Certificates of deposits	$ —	$ —	$ 490		$ 490	$ —
U.S. Treasury securities	7,514	(2)	—		7,514	(2)
U.S. Government agency securities	19,987	(12)	—		19,987	(12)
Total	$ 27,501	$ (14)	$ 490		$ 27,991	$ (14)

As of September 30, 2025, the Company held one domestic certificate of deposit with amortized costs below the Federal Deposit Insurance Corporation insured limit. Accrued interest receivable on available-for-sale investment securities, included in prepaid expenses and other current assets on the Company's balance sheets, was $1.4 million and $1.3 million as of September 30, 2025 and December 31, 2024, respectively.

6. Balance Sheet Details

Prepaid expenses and other current assets consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Prepaid expenses	$ 1,678	$ 1,300
Interest receivable	1,381	1,263
Total	$ 3,059	$ 2,563

Property and equipment consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Laboratory equipment	$ 2,438	$ 2,291
Computer equipment and software	102	102
Furniture and fixtures	521	521
Leasehold improvements	151	151
Construction in progress	—	—
	3,212	3,065
Less accumulated depreciation	(2,108)	(1,655)
Total	$ 1,104	$ 1,410

Accrued expenses and current liabilities consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Accrued personnel costs	$ 3,466	$ 3,576
Accrued research and development costs	4,435	1,220
Current portion of operating lease liability, net, related party	866	800
Accrued other	493	680
Total	$ 9,260	$ 6,276

7. Leases

In February 2021, the Company entered into a lease agreement with Crossing Holdings, LLC to rent laboratory and office space (the "Lease"). Dr. Pratik Shah and entities that he controls are the sole members of Crossing Holdings, LLC. The Lease commenced in September 2021 with a term of 72 months and an option to extend the lease term for a period of three years. Lease payments are subject to annual increases of 3% and the Company is responsible for its share of operating expenses and taxes, which are expensed as incurred. In March 2022, the Company entered into an amendment (the "Lease Amendment") to its Lease with Crossing Holdings, LLC to rent additional office space in the same building. The Lease Amendment commenced in June 2022 and the term of the additional premises under the Lease Amendment coincides with the term of the Lease and ends in 2027. As of September 30, 2025, the weighted-average remaining lease term for the Company's leases was 1.9 years and the weighted-average discount rate used to determine the right-of-use asset and corresponding operating lease liability was 7.37%.

Maturities of operating lease liabilities as of September 30, 2025 are as follows (in thousands):

2025 (remaining three months)	240
2026	973
2027	663
Total future minimum lease payments	1,876
Less: Present value adjustment	(133)
Operating lease liabilities	$ 1,743

Rent expense for each of the three months ended September 30, 2025 and 2024 was $0.2 million. Rent expense for each of the nine months ended September 30, 2025 and 2024 was $0.7 million.

8. Commitments and Contingencies

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 September 30, 2025 or December 31, 2024.

9. License Agreements

In May 2024, the Company entered into a license agreement pursuant to which it received exclusive, worldwide, royalty-bearing, sublicensable rights to certain patents and technology to be used in the development and commercialization of certain products. The Company paid $0.2 million in license fees during the year ended December 31, 2024, which was expensed to research and development as there is no alternative future use for the license.

The Company may be obligated to make aggregate regulatory milestone payments of up to $0.8 million for each product incorporating licensed patent rights and pay a royalty on worldwide net sales on a product-by-product basis. The Company will recognize these milestone payments and royalties when paid or payable. There were no additional payments or royalties recorded through September 30, 2025.

The Company may terminate the license agreement with 60 days' written notice and each party may terminate the license agreement upon 30 days' written notice for certain breaches of the agreement that remain uncured following 60 days' notice to the other party of such breach. Unless terminated earlier by the parties, the term of the license agreement will continue until the last valid patent claim expires.

Wisconsin Alumni Research Foundation

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 $0.3 million, which the Company immediately expensed as research and development expense in its statements of operations as there was no alternative future use for the license.

In 2022, pursuant to the License Agreement, the Company paid $0.1 million upon the acceptance of an Investigational New Drug Application in the United States. The Company 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, and is 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 will recognize these milestone payments and royalties when paid or payable. There were no additional payments or royalties recorded through September 30, 2025.

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 immaterial for the three and nine months ended September 30, 2025 and 2024, respectively.

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. Stockholders' Deficit

Shelf Registration Statement

In April 2022, the Company filed a shelf registration statement on Form S-3 (the "2022 Shelf Registration Statement"), which became effective in May 2022. In May 2025, the Company filed a new shelf registration statement on Form S-3, which became effective in May 2025, to replace the 2022 Shelf Registration Statement (the "2025 Shelf Registration Statement"). The 2025 Shelf Registration Statement permits: (i) the offering, issuance and sale by the Company of up to a maximum aggregate offering price of $300.0 million of common stock, preferred stock, debt securities and warrants in one or more offerings and in any combination; and (ii) the offering, issuance and sale by the Company of up to a maximum aggregate offering price of $100.0 million of our common stock that may be issued and sold under an "at-the-market" sales agreement (the "ATM Program"). The $100.0 million of common stock that may be issued and sold under the ATM Program is included in the $300.0 million of securities that may be issued and sold

under the 2025 Shelf Registration Statement. As of September 30, 2025, the Company has not sold any shares of its common stock under the ATM Program.

The Company recorded a one-time non-cash charge to general and administrative expenses of approximately $0.4 million during the nine months ended September 30, 2025. The one-time charge was previously capitalized in other assets on the Company's balance sheets and related to deferred financing costs for the 2022 Registration Statement.

11. Stock-Based Compensation

Equity Incentive Award Plans

The number of shares of common stock available for issuance under the Company's 2021 Equity Incentive Plan automatically increases on January 1 of each calendar year 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. As of September 30, 2025, the total number of shares available for future issuance was 5,399,258.

The following table summarizes stock option activity for the nine months ended September 30, 2025:

	Options	Weighted-Average Exercise Price
Outstanding at December 31, 2024	10,328,177	$ 6.96
Granted	3,933,526	$ 5.74
Exercised	(29,968)	$ 2.01
Forfeited	(372,213)	$ 12.19
Balance at September 30, 2025	13,859,522	$ 6.48

Stock-based compensation expense has been reported in the Company's condensed statements of operations as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 1,674	$ 1,524	$ 5,010	$ 4,679
General and administrative	1,916	1,594	6,104	4,922
Total	$ 3,590	$ 3,118	$ 11,114	$ 9,601

In August 2023, the Company granted two stock options, each to purchase up to 525,000 shares of the Company's common stock, which contained both time-based and performance-based conditions. Stock-based compensation expense for awards with performance conditions is recognized ratably for each vesting tranche when the achievement of such performance conditions is determined to be probable. The Company assessed the probability of achieving the performance-based conditions and recorded $0.1 million in related general and administrative stock-based compensation expense during the three months ended September 30, 2025 and 2024. The Company recorded $0.6 million and $0.2 million in related general and administrative stock-based compensation expense during the nine months ended September 30, 2025 and 2024, respectively.

2021 Employee Stock Purchase Plan

The number of shares of common stock available for issuance under the Company's ESPP automatically increases on January 1 of each calendar year 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; or a lesser number of shares determined by the Company's board of directors. As of September 30, 2025, the Company had issued 555,834 shares of the Company's common stock under the ESPP and had 2,292,694 shares available for future issuance.

12. Related Party Transactions

Lease Agreement

In February 2021, the Company entered into the Lease with Crossing Holdings, LLC to rent laboratory and office space. In March 2022, the Company entered into the Lease Amendment with Crossing Holdings, LLC amending the Lease for additional office space in the same building. Dr. Pratik Shah and entities that he controls are the sole members of Crossing Holdings, LLC.

Rent, and related operating expenses recognized by the Company under the Lease and Lease Amendment during the periods presented were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 217	$ 213	$ 654	$ 638
General and administrative	70	74	215	225
Total expenses	$ 287	$ 287	$ 869	$ 863

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 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 Chief Executive Officer and Chairperson of its board of directors is an executive officer of Marlinspike Group.

In March 2020, the 2019 Consulting Agreement was terminated and replaced with an amended consulting agreement (the "2020 Consulting Agreement"), which provides for 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. There has been no termination and 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 2020 Consulting Agreement during the periods presented were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
General and administrative	$ 60	$ 60	$ 180	$ 180
Total expenses	$ 60	$ 60	$ 180	$ 180

In December 2017, the Company entered into a consulting agreement with Aseem Z. Ansari, Ph.D., a co-founder, to provide consulting services and advise on certain research and development activities (the "Research Consulting Agreement"). The Research Consulting Agreement, as amended in November 2023, provides for an option grant to Dr. Ansari to purchase 100,000 shares of the Company's common stock. The option has a grant date fair value of $1.72 per share and is being expensed to research and development over a four-year vesting period. The Company recorded approximately $11,000 in related stock-based compensation expense during each of the three months ended September 30, 2025 and 2024 and approximately $32,000 during each of the nine months ended September 30, 2025 and 2024.

Expenses recognized by the Company under the Research Consulting Agreement during the periods presented were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 11	$ 11	$ 32	$ 32
Total expenses	$ 11	$ 11	$ 32	$ 32

13. Segment Information

The Company manages the business activities at the consolidated level and operates in one operating and reportable segment. The Company's CODM is its chief executive officer. The CODM primarily utilizes long-range financial projections and cash runway in order to allocate resources and to assess performance. As of September 30, 2025, the Company has no revenue and all the Company's long-lived assets were located within the United States. The CODM is regularly provided with the following significant segment expenses:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Interest income	$ 2,314	$ 3,207	$ 7,503	$ 9,752
Less:				
Direct program expense	9,295	7,109	29,701	17,737
Personnel expense	4,158	3,747	12,935	11,679
Stock-based compensation expense	3,590	3,118	11,114	9,601
Other segment items[(1)]	2,268	2,272	7,548	6,672
Segment net loss	$ (16,997)	$ (13,039)	$ (53,795)	$ (35,937)
Net loss	$ (16,997)	$ (13,039)	$ (53,795)	$ (35,937)

(1) Other segment items included in Segment net loss includes professional services, consulting and other outside services expenses, depreciation expense, insurance, facilities and other overhead items.

Item 2. 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 our unaudited financial statements and notes thereto included in "Item 1. Financial Statements" of this Quarterly Report on Form 10-Q and our other public filings with the Securities and Exchange Commission (SEC). In addition to historical information, this Quarterly Report contains forward-looking statements that involve risks, uncertainties, and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors, including but not limited to those set forth under the captions "Special Note Regarding Forward-Looking Statements" and "Risk Factors" in this Quarterly Report, as updated by our subsequent filings under the Securities Exchange Act of 1934, as amended (the Exchange Act). Furthermore, past operating results are not necessarily indicative of results that may occur in future periods.

Overview

We are a clinical-stage biopharmaceutical company pioneering the research and development of GeneTAC® molecules, which are a novel class of small-molecule gene targeted chimera therapeutic candidates designed to be disease-modifying by addressing the underlying cause of diseases caused by inherited nucleotide repeat expansion mutations. Certain diseases caused by inherited nucleotide repeat expansion, such as Friedreich ataxia (FA) and fragile X syndrome, can result in reduced gene expression and deficiency of vital proteins; in other diseases, such as myotonic dystrophy type-1 (DM1), Fuchs endothelial corneal dystrophy (FECD), and Huntington's disease (HD), the nucleotide repeat expansions result in the generation of toxic gene products, often associated with pathological nuclear foci and broad splicing disruptions or the expression of mutant proteins that form toxic aggregates. Our GeneTAC® small molecules are designed to selectively target expanded genetic repeat sequences, modulate gene expression either by dialing up or down mRNA transcription, depending on the cause of the disease, and restore cellular health. As a platform, we believe that GeneTAC® molecules have broad potential applicability across currently unaddressed degenerative, monogenic nucleotide repeat expansion diseases affecting millions of individuals worldwide.

In preclinical studies for our lead program in FA, we have observed restoration of frataxin (FXN) levels in multiple cell types from FA patients and an *in vivo* murine model of FA using our FA GeneTAC® molecules. At doses that were observed to be well-tolerated in rodents and non-human primates (NHPs), FA GeneTAC® molecules achieved biodistribution to brain and heart, key organs affected by FA, at concentrations that exceeded those observed to restore FXN levels in FA patient cells. Further, and consistent with this favorable target-organ biodistribution, we observed increased endogenous FXN expression in the brain and heart in an animal model of FA after treatment with our FA GeneTAC® molecules. In February 2022, the Investigational New Drug Application (IND) for our lead FA GeneTAC® small molecule, DT-216, formulated as the prior DT-216 product candidate, was cleared by the U.S. Food and Drug Administration (FDA) to commence Phase 1 clinical trials. In December 2022, we reported positive initial data from the Phase 1 single-ascending dose (SAD) clinical trial and in August 2023, we reported data from the Phase 1 multiple-ascending dose (MAD) clinical trial of the prior DT-216 product candidate. Both studies showed that DT-216 was generally well-tolerated and exhibited the ability to overcome the FXN transcription impairment that causes FA in FA patients. Data from the Phase 1 MAD clinical trial suggests more sustained exposure is likely needed to achieve a more durable increase in FXN expression, and we observed five cases of injection site thrombophlebitis in the Phase 1 MAD clinical trial. We then shifted focus to developing DT-216 with a potentially improved formulation to enable higher exposure and chronic administration for treatment of FA. These efforts resulted in a new product candidate, DT-216P2, which uses the same drug substance, DT-216. In nonclinical studies, we observed higher and more sustained DT-216 plasma levels after administration of DT-216P2 than was seen in studies with the prior DT-216 product candidate. Additionally, we observed favorable injection site tolerability following multiple intravenous (IV) administrations of DT-216P2.

A Phase 1 SAD clinical trial of DT-216P2 in normal healthy volunteers in Australia to evaluate single doses using multiple routes of administration, specifically IV infusion and subcutaneous (SC) injection and infusion routes, has shown that human plasma pharmacokinetics (PK) profiles of DT-216P2 were consistent with NHP data following both IV and SC single-dose administration. Early human PK data has demonstrated that DT-216P2 exhibited improved exposure and PK parameters compared to the prior DT-216 product candidate, including higher AUC and sustained plasma levels at comparable doses.

We are conducting our RESTORE-FA (Reactivating Expression Suppressed Through Overcoming Repeat Expansion for FA) Phase 1/2 MAD clinical trial of DT-216P2. The RESTORE-FA trial is designed to evaluate the safety, tolerability, PK and pharmacodynamics (PD) of IV and SC of DT-216P2 in patients with FA. We anticipate providing an update from the RESTORE-FA trial on the effect of DT-216P2 on endogenous FXN levels following 12 weeks of dosing in the second half of 2026.

DT-216P2 has been generally well-tolerated in these clinical trials. Based on the data from these clinical trials and non-clinical studies of DT-216P2, we believe the injection site thrombophlebitis seen with the prior DT-216 product candidate is no longer an issue limiting continued development of DT-216.

In June 2025, we received a clinical hold notice from the FDA regarding our IND application for DT-216P2. The FDA's request pertains to the starting dose in the United States, which we plan to address with clinical data and, if needed, nonclinical data, in order to initiate studies for DT-216P2 in the United States.

In December 2022, we nominated our second GeneTAC® small molecule, DT-168, an eye drop for the treatment of FECD. When tested in vitro in FECD patient-derived corneal endothelial cells, our FECD GeneTAC® molecules led to robust reductions in the pathogenic nuclear RNA foci and corrected key mis-spliced transcripts to levels observed in control corneal endothelial cells from unaffected donors. DT-168 was well-tolerated and distribution of DT-168 was observed in and through the cornea in animal models after administration via eye drop. In addition, DT-168 has been evaluated in chronic toxicity studies of up to nine months in duration. We believe these preclinical data support the potential of our novel GeneTAC® small molecules to correct the most common underlying genetic cause of FECD. We are currently conducting an observational study in FECD to confirm disease characteristics and evaluate deterioration in the context of running a trial and to identify characteristics of FECD patients at risk of more rapid disease progression. We have achieved our enrollment goal for the observational study by recruiting and completing baseline assessments on approximately 250 FECD patients. Based on the baseline characteristics data, we have chosen approximately 100 patients for future follow-up visits. This will inform our clinical development efforts and we believe it could potentially increase the probability of DT-168 programmatic success.

In May 2025, we reported results from a completed Phase 1, double-masked, placebo-controlled, randomized, SAD/MAD clinical trial evaluating the safety, tolerability and systemic PK of DT-168 ophthalmic solution in normal healthy volunteers. Twenty-four normal healthy volunteers received either placebo or single- and multiple-ascending doses of DT-168 eye drops twice daily for seven days (up to a maximum dose of two 0.5% drops twice-daily). DT-168 eye drops were well-tolerated in all participants. There were no serious adverse events, no ocular adverse events (AEs) and no treatment discontinuations due to AEs in the trial. All observed AEs were deemed not related to DT-168 by the trial investigator. PK analysis demonstrated systemic exposure below the limit of quantitation for all participants across all timepoints and all dose groups. In parallel with the Phase 1 trial, we conducted reference range studies which showed consistently different splicing in the corneal endothelium between unaffected eye donors and surgical samples from mutant TCF4 FECD patients, supporting the potential for corneal endothelium biomarkers as a clinical proof-of-concept measure of drug activity. We are conducting a Phase 2 biomarker trial of DT-168 to evaluate safety, tolerability, and corneal endothelium biomarkers in patients with FECD. FECD Patients will receive 0.5% DT-168 eye drops twice-daily for approximately four weeks or more before corneal transplant surgery. Following surgery, tissues from the treated eyes of FECD patients will undergo testing to assess corneal endothelium biomarkers including the abnormal splicing of genes known as spliceopathy. We anticipate reporting data from the Phase 2 biomarker trial in the second half of 2026.

In November 2025, we nominated our third GeneTAC® small molecule, DT-818, as a development candidate for the treatment of DM1. The underlying cause of DM1 is a CTG repeat expansion in the DMPK gene, which DT-818 is designed to address by selectively reducing transcription of the mutant expanded allele. In preclinical studies, DT-818 has demonstrated a potential best-in-disease profile for DM1, including a greater than 90% reduction in toxic RNA foci in DM1 patient cells, corresponding splicing correction and selective targeting of mutant DMPK. In preclinical models of DM1 (HSA^{LR} mouse model with CTG repeats under the control of the actin promoter), DT-818 treatment resulted in improved myotonia and foci reduction. In tissue distribution studies in NHPs, DT-818 levels were observed to be at expected pharmacologic levels in key target tissues at well-tolerated doses. We have obtained regulatory clearance to initiate clinical development of DT-818 and plan to begin dosing DM1 patients in a Phase 1 MAD trial in Australia to assess safety and correction of mis-splicing in the first half of 2026, with splicing data expected in 2027.

Our fourth program based on the GeneTAC® platform is focused on HD. We are currently conducting preclinical studies on promising HD GeneTAC® candidate molecules. We have observed reduced mutant huntingtin (mtHTT) mRNA and protein and preservation of wild type huntingtin (wtHTT) in HD patient cells after treatment with our HD GeneTAC® candidate molecules. In *in vivo* studies in zQ175DN mice, an animal model of HD, we observed a reduction of over 50% in mtHTT mRNA and protein in the brain striatum after eight weeks of systemic administration of our HD GeneTAC® candidate molecules. In the same study, wtHTT mRNA and protein levels were shown to be preserved after treatment with our HD GeneTAC® candidate molecules. We plan to continue to evaluate these HD candidate molecules in nonclinical studies. The final development candidate will be based on the molecules that perform favorably in relevant studies.

We have continued to make significant progress in advancing our GeneTAC® portfolio in preclinical studies to address other diseases and intend to declare additional product candidates as they progress towards the clinic.

We believe the structure and mechanism of action of our GeneTAC® molecules 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.

To date, we have incurred net losses and negative cash flows from operations since our inception and as of September 30, 2025 had an accumulated deficit of $281.0 million. Our cash, cash equivalents and investment securities balance as of September 30, 2025 was $206.0 million. Our net losses have resulted primarily from costs incurred in connection with organizing and staffing our company, business planning, raising capital, developing and optimizing our technology platform, identifying potential product candidates, undertaking research and nonclinical studies, clinical development activities, 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 expect our expenses and operating losses will increase substantially for the foreseeable future as we continue to conduct nonclinical 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.

Components of Our Results of Operations

Research and Development Expenses

To date, our research and development expenses have consisted primarily of direct and indirect costs incurred in connection with the clinical development, nonclinical development and manufacturing of our product candidates and our discovery efforts. 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 clinical, nonclinical and discovery activities;
- expenses related to manufacturing our product candidates for clinical and nonclinical 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 expenses which include allocated expenses for rent, depreciation and other overhead expenses, costs for general laboratory consumables and other indirect expenses.

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

We expect that our research and development expenses will increase for the foreseeable future as we continue the development of our FA, FECD, DM1 and HD programs and our other discovery programs, in particular as we advance our product candidates into and through clinical development. As of the date of this Quarterly Report, we cannot reasonably determine with certainty the timing of initiation, the duration or the completion costs of current or future nonclinical studies and clinical programs of our product candidates due to the inherently unpredictable nature of nonclinical and clinical development. Nonclinical 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 nonclinical 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, nonclinical and clinical 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 scope and costs of designing and implementing drug product improvements (including alternate formulations) and manufacturing our product candidates;
- 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 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 or 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; 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 nonclinical 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 insurance costs, legal fees relating to intellectual property and corporate matters and professional fees for accounting, tax and consulting services.

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.

Results of Operations

Comparison of the Three Months Ended September 30, 2025 and 2024

The following table summarizes our operating expenses for the three months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 14,589	$ 11,876	$ 2,713
General and administrative	4,722	4,370	352
Total operating expenses	$ 19,311	$ 16,246	$ 3,065

Research and Development Expenses. Research and development expenses for our FA program increased during the three months ended September 30, 2025 compared to the same period of the prior year primarily due to costs attributable to DT-216 clinical activities and costs for additional development activities.

Expenses for our FECD program increased compared to the same period of the prior year primarily due to costs associated with DT-168 clinical activities and costs for additional development activities.

Other direct expenses increased compared to the same period of the prior year primarily due to additional activities related to our early stage research programs. The increase in indirect expenses compared to the same period of the prior year was primarily due to employee related expenses including compensation, stock-based compensation and other support for our ongoing development programs.

The following table summarizes our research and development expenses by program, direct and indirect costs for the three months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
FA	$ 3,445	$ 3,238	$ 207
FECD	1,579	1,213	366
Other direct	4,271	2,658	1,613
Indirect	5,294	4,767	527
Total research and development expense	$ 14,589	$ 11,876	$ 2,713

General and Administrative Expenses. The increase in general and administrative expenses was primarily due to a $0.3 million increase in stock-based compensation expenses and $0.1 million in other expenses incurred during the three months ended September 30, 2025 as compared to the same period in 2024.

Comparison of the Nine Months Ended September 30, 2025 and 2024

The following table summarizes our operating expenses for the nine months ended September 30, 2025 (in thousands):

	Nine Months Ended September 30,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 45,704	$ 32,193	$ 13,511
General and administrative	15,594	13,496	2,098
Total operating expenses	$ 61,298	$ 45,689	$ 15,609

Research and Development Expenses. Research and development expenses for our FA program increased during the nine months ended September 30, 2025 compared to the same period of the prior year primarily due to costs attributable to DT-216 clinical activities and costs for additional development activities.

Expenses for our FECD program increased compared to the same period of the prior year primarily due to costs associated with DT-168 clinical activities and costs for additional development activities.

Other direct expenses increased compared to the same period of the prior year primarily due to additional activities related to our early stage research programs. The increase in indirect expenses compared to the same period of the prior year was primarily due to employee related expenses including compensation, stock-based compensation and other support for our ongoing development programs.

The following table summarizes our research and development expenses by program, direct and indirect costs for the nine months ended September 30, 2025 (in thousands):

	Nine Months Ended September 30,		
	2025	2024	Change
FA	$ 10,851	$ 5,601	$ 5,250
FECD	4,825	4,066	759
Other direct	14,025	8,070	5,955
Indirect	16,003	14,456	1,547
Total research and development expenses	$ 45,704	$ 32,193	$ 13,511

General and Administrative Expenses. The increase in general and administrative expenses was primarily due to a $1.4 million increase in employee compensation, including a $1.2 million in stock-based compensation expenses, a $0.6 million increase in professional services expenses including a $0.4 million one-time charge to write off previously deferred financing costs and $0.1 million in other expenses incurred during the nine months ended September 30, 2025 as compared to the same period in 2024.

Liquidity and Capital Resources

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

As of September 30, 2025, we had $206.0 million of combined cash, cash equivalents and investment securities, a decrease of $39.5 million from the $245.5 million of cash, cash equivalents and investment securities at December 31, 2024. Further detail of the change in our cash and cash equivalents for the nine months ended September 30, 2025 and 2024 is summarized below (in thousands):

	Nine Months Ended September 30,	
	2025	2024
Net cash (used in) provided by:		
Operating activities	$ (42,392)	$ (33,245)
Investing activities	34,496	42,123
Financing activities	421	250
Net (decrease) increase in cash and cash equivalents	$ (7,475)	$ 9,128

Operating Activities. The increase in our net cash used in operating activities was primarily due to the $17.9 million increase in net loss, partially offset by an increase in non-cash expenses and net working capital for the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024.

Investing Activities. The decrease in net cash provided by investing activities was primarily due to a net decrease in cash provided from the maturities and purchases of our investment securities during the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024. We have classified our investment securities as available-for-sale and all investments are made in accordance with our investment policy.

Financing Activities. Net cash provided by financing activities for the nine months ended September 30, 2025 and 2024 was comprised of proceeds received from the issuance of common stock through our employee stock purchase plan and from employee stock option exercises.

Shelf Registration Statement

In April 2022, the Company filed a shelf registration statement on Form S-3 (the “2022 Shelf Registration Statement”), which became effective in May 2022. In May 2025, the Company filed a new shelf registration statement on Form S-3, which became effective in May 2025, to replace the 2022 Shelf Registration Statement (the 2025 Shelf Registration Statement). The 2025 Shelf Registration Statement permits: (i) the offering, issuance and sale by us of up to a maximum aggregate offering price of $300.0

million of common stock, preferred stock, debt securities and warrants in one or more offerings and in any combination; and (ii) the offering, issuance and sale by us of up to a maximum aggregate offering price of $100.0 million of our common stock that may be issued and sold under an "at-the-market" sales agreement (ATM Program). The $100.0 million of common stock that may be issued and sold under the ATM Program is included in the $300.0 million of securities that may be issued and sold under the 2025 Shelf Registration Statement. As of September 30, 2025, the Company has not sold any shares of its common stock under the ATM Program.

The Company recorded a one-time non-cash charge to general and administrative expenses of approximately $0.4 million during the nine months ended September 30, 2025. The one-time charge was previously capitalized in other assets on the Company's balance sheets and related to deferred financing costs for the 2022 Registration Statement.

Funding Requirements

Based on our current operating plan, we believe that our existing cash, cash equivalents and investments will be sufficient to fund our planned operating expenses and capital expenditure requirements for more than the next 12 months following the date of this Quarterly Report.

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, nonclinical development activities and clinical trials for any product candidates;
- the number and scope of clinical programs we decide to pursue;
- the scope and costs of designing and implementing drug product improvements (including alternate formulations) and manufacturing our product candidates and any future commercial manufacturing activities;
- the emergence of competing therapies and other adverse market developments;
- the cost, timing and outcome of seeking FDA, European Medicines Agency (EMA) and any other regulatory approvals for any 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 our current and 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;
- the costs associated with expanding our facilities or building out additional laboratory space; and
- the cost associated with commercialization activities for any of our current or 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 as a result of macroeconomic factors, including geopolitical conflicts (such as the geopolitical tensions between the U.S. and China, the Russia/Ukraine conflict, the conflict in the Middle East which has recently included actions by Iran, Hamas, Israel and the United States), inflation, tariffs, other fiscal and trade policy changes, bank failures, global supply chain and labor shortage challenges, and the effects of a health epidemic or pandemic. 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, Commitments and Material Cash Requirements

In February 2021, we entered into a lease agreement to rent lab and office space with a related party. The delivery date of the space was September 1, 2021, and the lease commenced 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 are approximately $0.8 million per year, subject to annual increases of 3%, plus our share of operating expenses and taxes.

In March 2022, we entered into an amendment to our existing lease agreement to rent additional space with a related party. The delivery date of the space was June 16, 2022, and the lease amendment commenced at that time. The term of the lease coincides with our existing lease. Annual rent payments for the additional space are approximately $0.1 million, subject to annual increases of 3%, plus our share of operating expenses and taxes.

In February 2019, we entered into a Human Therapeutics Exclusive License Agreement (License Agreement) with the Wisconsin Alumni Research Foundation (WARF). Under the License Agreement, we 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, we agreed to pay an upfront fee of $0.3 million, which we immediately expensed as a research and development expense in our statements of operations as there was no alternative future use for the license.

For the nine months ended September 30, 2025, no payments were made pursuant to the License Agreement. In 2022, pursuant to the License Agreement, we paid $0.1 million to WARF upon the acceptance of an IND in the United States. We will be required to make further aggregate milestone payments of up to $17.5 million upon achievement of certain other regulatory and commercial milestones. We may also be required to pay royalties based on annual net product sales in the low single digits on our or our sublicensees' net product sales on a country-by-country and product-by-product basis, and are subject to a minimum royalty of $0.1 million per calendar year upon first commercial product sale. Further, we 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 will recognize these milestone payments and royalties when paid or payable. There were no additional payments or royalties recorded through September 30, 2025.

We are 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 immaterial for each of the nine months ended September 30, 2025 and 2024.

We 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.

In May 2024, we entered into a license agreement pursuant to which we received exclusive, worldwide, royalty-bearing, sublicensable rights to certain patents and technology to be used in the development and commercialization of certain products. We paid $0.2 million in license fees during the year ended December 31, 2024, which was expensed to research and development as there is no alternative future use for the license.

We may be obligated to make aggregate regulatory milestone payments of up to $0.8 million for each product incorporating licensed patent rights and pay a royalty on worldwide net sales on a product-by-product basis. The Company will recognize these milestone payments and royalties when paid or payable. There were no additional payments or royalties recorded through September 30, 2025.

We may terminate the license agreement with 60 days' written notice and each party may terminate the license agreement upon 30 days' written notice for certain breaches of the agreement that remain uncured following 60 days' notice to the other party of such breach. Unless terminated earlier by the parties, the term of the license agreement will continue until the last valid patent claim expires.

Additionally, we enter into agreements in the normal course of business with third-party vendors for nonclinical studies, clinical trial related services, research supplies and other services and products for operating purposes. These contracts generally provide for termination following a certain period after notice and therefore we believe that our non-cancelable obligations under these agreements are not material.

In addition to the contractual obligations above, we also expect to have future material cash requirements related to our planned clinical trials, discovery and nonclinical programs, personnel and facilities-related expenses, external research and development and product development.

Critical Accounting Policies 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. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities 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 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.

Our critical accounting policies are those that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. For a description of our critical accounting policies, please see Item 1 of Part I, "Notes to Condensed Financial Statements—Note 2—Basis of Presentation and Summary of Significant Accounting Policies" of this Quarterly Report on Form 10-Q, and Note 2 to our Financial Statements and our Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K filed with the SEC on March 10, 2025. There have not been any material changes to our critical accounting policies since December 31, 2024.

Recent Accounting Pronouncements

See Item 1 of Part I, "Notes to Condensed Financial Statements—Note 2—Basis of Presentation and Summary of Significant Accounting Policies" for a discussion of recent accounting pronouncements.

Other Information

Emerging Growth Company Status

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act, as amended (JOBS Act), and we may remain an emerging growth company until as late as December 31, 2026 (the fiscal year-end following the fifth anniversary of the completion of our initial public 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.

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 $1.235 billion in annual revenue; (ii) the date upon which we are deemed to be a "large accelerated filer," as defined in Rule 12b-2 under the Exchange Act, which would occur if we have been subject to the reporting requirements of the Exchange Act for at least 12 months as of December 31st and 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) December 31, 2026 (the last day of the fiscal year ending after the fifth anniversary of our initial public offering).

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

As a "smaller reporting company," we are not required to provide the information contemplated by this item.

Item 4. Controls and Procedures.

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our periodic and current reports that we file with the SEC is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer (our principal executive and financial officer), as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. In addition, the design of any system of controls is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

As of September 30, 2025, we carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer (who is also our principal financial officer), of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Based on this evaluation, our Chief Executive Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of September 30, 2025.

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer, of any change in our internal control over financial reporting that occurred during our last fiscal quarter and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. That evaluation did not identify any changes in our internal control over financial reporting that occurred during our latest fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may become involved in litigation or other legal proceedings. We are not currently a party to any litigation or legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors.

We operate in a dynamic and rapidly changing environment that involves numerous risks and uncertainties. Certain factors may have a material adverse effect on our business, financial condition and results of operations, and you should carefully consider them. Accordingly, in evaluating our business, we encourage you to consider the following discussion of risks described below, together with the other information contained in this Quarterly Report on Form 10-Q and in our other public filings with the Securities and Exchange Commission (SEC). If any of the following risks actually occur, our business, financial condition, results of operations, and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline. Other events that we do not currently anticipate or that we currently deem immaterial may also affect our results of operations and financial condition. We have marked with an asterisk (*) those risk factors that were not included as separate risk factors in, or reflect changes from the similarly titled risk factors included in, Item 1A of our Annual Report on Form 10-K, filed with the SEC on March 10, 2025.

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 a clinical-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 nonclinical studies for our lead program in FA and our other development programs, early clinical development for our FA and FECD programs, establishing and enhancing our intellectual property portfolio, and providing general and administrative support for these operations. As a result, none of our product candidates have been approved for commercial sale. We have never generated any revenue from product sales and have incurred net losses each year since we commenced operations, including a net loss of $53.8 million and $49.6 million for the nine months ended September 30, 2025 and the year ended December 31, 2024, 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 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 nonclinical 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.

If we are unable to raise additional 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 nonclinical 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.

As of September 30, 2025, we had $206.0 million in cash, cash equivalents and investment securities. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investment securities will be sufficient to fund our operating expenses and capital expenditure requirements for more than the next 12 months. However, we believe that our existing cash, cash equivalents and investment securities 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, nonclinical development activities, laboratory testing and clinical trials for any current or future product candidates;
- the number and scope of clinical programs we decide to pursue;
- the scope and costs of manufacturing our product candidates and any future commercial manufacturing activities;
- the emergence of competing therapies and other adverse market developments;
- the cost, timing and outcome of seeking FDA, European Medicines Agency (EMA) and any other regulatory approvals for any 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 our current and 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 additional laboratory space; and
- the cost associated with commercialization activities for any of our 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. 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 as a result of macroeconomic factors, including geopolitical conflicts (such as the geopolitical tensions between the U.S. and China, the Russia/Ukraine conflict, the conflict in the Middle East which has recently included actions by Iran, Hamas, Israel and the United States), inflation, tariffs, other fiscal and trade policy changes, bank failures, global supply chain and labor shortage challenges, and the effects of a health epidemic or pandemic. 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, 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, current 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 existing 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 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. 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.

Our financial condition could be adversely affected if the financial institutions in which we hold our cash fail.

We maintain cash deposits in Federal Deposit Insurance Corporation (FDIC) insured banks. The bank deposit balances may exceed the FDIC insurance limits, and, currently, we hold our cash in a limited number of accounts. Our access to these balances could be impacted if one or more of the financial institutions in which we deposit monies fails or is subject to other adverse conditions in the financial or credit markets. For example, multiple banks failed and were taken into receivership by the FDIC in the first half of 2023. Our access to cash may be adversely affected in the future by actual or anticipated bank failures.

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

We are early in our development efforts and we have only two product candidates in clinical development, with all of our other research programs currently in the nonclinical or discovery stage. We have a limited 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 nonclinical studies. Our lead FA GeneTAC® small molecule, DT-216, formulated as the prior DT-216 product candidate, entered into a Phase 1 SAD clinical trial in March 2022, the first clinical trial for one of our product candidates and we completed the Phase 1 MAD clinical trial of the prior DT-216 product candidate in August 2023. We withdrew our IND for the prior DT-216 product candidate in October 2023, and have initiated a Phase 1 SAD clinical trial in normal healthy volunteers in Australia to evaluate single doses using multiple routes of administration of DT-216P2, which uses the same drug substance, DT-216. We are conducting our RESTORE-FA Phase 1/2 MAD clinical trial of DT-216P2 and have also completed a Phase 1 clinical trial in normal healthy volunteers for DT-168, our FECD GeneTAC® product candidate. Furthermore, we are conducting a Phase 2 biomarker trial of DT-168 to evaluate safety, tolerability, and corneal endothelium biomarkers in patients with FECD. We have limited infrastructure, experience conducting clinical trials as a company and regulatory interactions, and cannot be certain that our 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 product candidates, our ability to eventually generate significant revenues from product sales will depend on a number of factors, including:

- completion of nonclinical 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 personnel 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, FECD, DM1 and HD 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.

Nonclinical and clinical development involves a lengthy and expensive process with uncertain timelines and outcomes, and results of nonclinical studies and clinical trials may not be predictive of future trial results. If development of our 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 having promising results in earlier nonclinical studies or clinical trials. These setbacks have been caused by, among other things, nonclinical findings emerging while clinical trials were underway and safety or efficacy observations made in nonclinical studies or clinical trials, including previously unreported adverse events. Although we have initiated clinical trials of DT-216P2, conducted clinical trials of the prior DT-216 product candidate and nonclinical studies of the prior DT-216 product candidate and DT-216P2 for the treatment of patients with FA, completed a Phase 1 clinical trial of DT-168 in normal healthy volunteers and conducted certain nonclinical studies of DT-168 and certain nonclinical studies of other potential product candidates targeting expansion repeat driven diseases, we do not know whether DT-216P2 or DT-168 or other potential product candidates targeting expansion repeat driven diseases will perform in ongoing or future clinical trials as they have performed in prior studies. Furthermore, for some indications that we are pursuing, such as FECD caused by a nucleotide repeat expansion mutation in the TCF4 gene, there are no animal models of the human disease and therefore our ability to predict human disease outcomes may be reduced.

The results of nonclinical and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Adverse safety or efficacy observations in nonclinical studies or clinical trials may result in delays in timelines of our programs before or after clinical trials have commenced. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through nonclinical 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.

While we have conducted nonclinical studies of the prior DT-216 product candidate for the treatment of patients with FA, released initial clinical data from our Phase 1 SAD clinical trial in December 2022 and released initial results from the Phase 1 MAD clinical trial of the prior DT-216 product candidate in August 2023 with results showing that DT-216 was generally well-tolerated and exhibited the ability to overcome the FXN transcription impairment that causes FA, previously reported results from the Phase 1 SAD clinical trial showed that three patients on the prior DT-216 product candidate had injection site thrombophlebitis that were mild or moderate and resolved without treatment. DT-216 was generally well-tolerated in the Phase 1 MAD clinical trial. However, self-limited injection site thrombophlebitis was observed in five patients across all three dose levels (100 mg, 200 mg and 300 mg), whereas injection site thrombophlebitis was only observed at higher doses (the 400 mg and 600 mg cohorts) in the Phase 1 SAD clinical trial. Nonclinical studies showed that the injection site reactions were attributable to the formulation excipients in the prior DT-216 product candidate formulation, and that improving the formulation composition could enable higher doses and chronic administration. We have since shown that a potentially improved formulation using a novel and proprietary excipient with DT-216P2 had favorable injection site tolerability following multiple intravenous administrations and enabled dosing to increase tissue exposure. Given the injection site thrombophlebitis observed in the Phase 1 MAD clinical trial and concern for potential worsening of injection site thrombophlebitis at higher doses with multiple administration, and the initial results from animal studies using a novel and proprietary excipient in DT-216P2, instead of advancing the prior DT-216 product candidate through to Phase 2 in the second half of 2023 as originally planned, we decided to pursue development of DT-216P2 to better support the future clinical development and regulatory pathway for the drug substance DT-216. We withdrew our IND for the prior DT-216 product candidate in October 2023. We are conducting our RESTORE-FA Phase 1/2 MAD clinical trial of DT-216P2. The RESTORE-FA trial is designed to evaluate the safety, tolerability, PK and PD of IV and subcutaneous administration of DT-216P2 in patients with FA. We anticipate providing an update from the RESTORE-FA trial on the effect of DT-216P2 on endogenous FXN levels following 12 weeks of dosing in the second half of 2026. We believe the injection site thrombophlebitis seen with the prior DT-216 product candidate is no longer an issue

limiting continued development of DT-216. However, there can be no assurance that we will be able to successfully develop DT-216P2 with improved injection site tolerability and the absence of other unacceptable side effects on the timeframe we expect, or at all, or that we will be able to achieve our anticipated clinical development and data timelines for the RESTORE-FA trial. In addition, in June 2025, we received a clinical hold notice from the FDA regarding our IND for DT-216P2. The FDA's request pertains to the starting dose in the United States. Although we plan to address the clinical hold with clinical data and, if needed, nonclinical data, in order to initiate studies for DT-216P2 in the United States, there can be no assurance that we will be able to successfully resolve the FDA clinical hold with such anticipated data or in a timely or efficient manner, or at all, or that we will be able to resume U.S. clinical development for our DT-216 program on the timeframe anticipated, or at all.

We may experience delays in initiating our 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 nonclinical 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 a Data Safety Monitoring Board (DSMB) for such trial or by the FDA or other regulatory authorities (including foreign regulatory authorities comparable to the FDA). 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 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.

Additionally, the policies of the FDA and other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit, or delay regulatory approval of our product candidates. For example, the U.S. Supreme Court's June 2024 decision in *Loper Bright Enterprises v. Raimondo* overturned the longstanding Chevron doctrine, under which courts were

required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The *Loper* decision could result in additional legal challenges to regulations and decisions issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the *Loper* decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature, or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are unable to maintain regulatory compliance, we may be subject to enforcement action and our business, results of operations, and financial condition could be adversely affected.

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 initiating or completing our 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 GeneTAC® 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 GeneTAC® platform technologies in clinical trials or in obtaining marketing approval thereafter, and use of our GeneTAC® 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 clinical trials or commercializing any products on a timely or profitable basis, if at all. Further, because all of our product candidates and development programs are based on the same GeneTAC® platform technologies, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs.

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.

We are evaluating a biomarker-driven clinical development strategy, and such strategy carries increased risks. A proposal for new or emerging biomarker focused endpoints may result in data that is not accepted by the FDA or comparable foreign regulatory bodies or industry professionals, or if such endpoints are later found to be insufficient to establish clinical efficacy, may require us to change the design of our clinical trials.

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 GeneTAC® platform 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 clinical trials, our clinical development activities could be delayed or otherwise adversely affected.*

We may not be able to continue our ongoing clinical trials or initiate or continue clinical trials planned in the future 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 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 may 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. We may also experience enrollment challenges if patients that would otherwise enroll in our trial choose not to do so, or are not eligible to do so, as a result of treatment with another drug. For example, the FDA approved omaveloxolone for the treatment of FA in adults and adolescents aged 16 years and older in February 2023, and omaveloxolone was commercially launched in June 2023. The availability of omaveloxolone may impact future enrollment of our planned clinical trials. FA patients receiving omaveloxolone may be ineligible to enroll in a clinical trial for DT-216P2 or may choose not to do so due to the availability of an approved product. Furthermore, patients enrolled in our clinical trials may receive omaveloxolone and we cannot predict the impact of potential drug interactions on trial results. Likewise, patient enrollment for our planned Phase 1 MAD study of DT-818 may be adversely impacted by competition from clinical trials for other DM1 treatments. Delays in patient enrollment may result in increased costs or may affect the timing or outcome of our clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

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

We are conducting and 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, withholding and payroll taxes;
- administrative burdens of conducting clinical trials under multiple foreign regulatory schema;
- foreign exchange fluctuations;
- manufacturing, customs, shipment and storage requirements;

- impact of geopolitical events or a public health crisis 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, 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 nonclinical 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 our 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 ongoing or 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 clinical trials or nonclinical studies could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. For example, in December 2022, we reported initial data from the Phase 1 SAD clinical trial for the prior DT-216 product candidate, the first clinical trial for one of our product candidates. Initial safety results from Cohorts 1-5 showed that DT-216 was generally well-tolerated after a single dose of the prior DT-216 product candidate, but three patients on the prior DT-216 product candidate had injection site thrombophlebitis that were mild or moderate and resolved without treatment. DT-216 was generally well-tolerated in the subsequent Phase 1 MAD clinical trial. However, self-limited injection site thrombophlebitis was observed in five patients across all three dose levels (100 mg, 200 mg and 300 mg), whereas injection site thrombophlebitis was only observed at higher doses (the 400 mg and 600 mg cohorts) in the Phase 1 SAD clinical trial. Nonclinical studies showed that the injection site reactions were attributable to the formulation excipients in the prior DT-216 product candidate formulation, and that improving the formulation composition could enable higher doses and chronic administration. We have since shown that a potentially improved formulation using a novel and proprietary excipient with DT-216P2 had favorable injection site tolerability following multiple intravenous administrations and enabled dosing to increase tissue exposure. Given the injection site thrombophlebitis observed in the Phase 1 MAD clinical trial and concern for potential worsening of injection site thrombophlebitis at higher doses with multiple administration, the initial results from animal studies with DT-216P2 using a novel and proprietary excipient, instead of advancing the prior DT-216 product candidate with the current formulation through to Phase 2 in the second half of 2023 as originally planned, we decided to pursue development of DT-216P2 that we believe may better support the future clinical development and regulatory pathway for the drug substance DT-216. We withdrew our IND for the prior DT-216 product candidate in October 2023. Based on early clinical data and non-clinical studies of DT-216P2, we believe the injection site thrombophlebitis seen with the prior DT-216 product candidate is no longer an issue limiting continued development of DT-216. However, there can be no assurance that we will be able to successfully develop DT-216P2 with improved injection site tolerability and the absence of other unacceptable side effects on the timeframe we expect, or at all, or that we will be able to achieve our anticipated clinical development and data timelines.

If unacceptable side effects, such as severe injection site reactions associated with intravenous administration (including injection site thrombophlebitis), arise in the development of our product candidates, we, the FDA or comparable foreign regulatory authorities, the IRBs at the institutions in which our studies are conducted, or the DSMB (or applicable safety oversight committee) could recommend a protocol amendment, suspend or terminate our clinical trials or the FDA or comparable foreign regulatory

authorities may 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 from 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 be 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.

Early, interim, topline and preliminary data from our nonclinical studies or 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 early, interim, preliminary or topline data from our nonclinical studies or 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 early, 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. Early, 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 early or interim data from our clinical trials. Early, 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 early, preliminary, interim or topline data and final data could significantly harm our business prospects.

For example, a Phase 1 SAD clinical trial of DT-216P2 in normal healthy volunteers in Australia to evaluate single doses using multiple routes of administration, specifically IV infusion and SC injection and infusion routes, has shown that human plasma pharmacokinetics (PK) profiles of DT-216P2 were consistent with NHP data following both IV and SC single-dose administration. Early human PK data demonstrated that DT-216P2 exhibited improved exposure and PK parameters compared to the prior DT-216 product candidate, including higher AUC and sustained plasma levels at comparable doses. It is possible that our exposure, PK and other observations will materially change as additional data becomes available, including from the RESTORE-FA Phase 1/2 MAD clinical trial.

In addition, 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 early, 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 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 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 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 ongoing and 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 may also 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.

International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.*

We operate in a global economy, which includes utilizing third-party suppliers in certain countries outside the United States. There is inherent risk, based on the complex relationships among the U.S. and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty. The U.S. government has recently announced substantial new tariffs affecting a wide range of products and jurisdictions and has indicated an intention to continue developing new trade policies, including with respect to the pharmaceutical industry. In response, certain foreign governments have announced or implemented retaliatory tariffs and other protectionist measures. These developments have created a dynamic and unpredictable trade landscape, which may adversely impact our business, results of operations, financial condition and prospects.

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates for clinical testing and the manufacture of our materials for our product candidates, including our active pharmaceutical ingredients (APIs) and certain excipients, as well as for manufacture of any products that we may commercialize, if approved. Such materials for our product candidates are currently manufactured in multiple foreign countries, including China.

Current or future tariffs will result in increased research and development expenses, including with respect to increased costs associated with APIs. In addition, such tariffs will increase our supply chain complexity and could also potentially disrupt our existing supply chain. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence, negatively impacting our ability to secure additional financing on favorable terms or at all. In addition, as we advance toward commercialization in the future, tariffs and trade restrictions could hinder our ability to establish cost-effective production capabilities, negatively impacting our growth prospects.

The complexity of announced or future tariffs may also increase the risk that we or our suppliers may be subject to civil or criminal enforcement actions in the United States or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In addition, the United States and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report.

A health epidemic or 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.

Our business could be adversely affected by the effects of health epidemics or pandemics in regions where we have business operations, and could cause significant disruption in the operations of third parties upon whom we rely including our suppliers, CROs and other contract service providers. Measures imposed by governments in affected regions may impact commercial activities and businesses in an effort to reduce the spread of the disease. We may experience disruptions that could severely impact our business and clinical trials, including:

- delays or difficulties in enrolling and retaining patients in our clinical trials, particularly subjects who are at a higher risk of severe illness or death;
- 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 an infectious disease on patients;
- changes in local regulations as part of a response to a public health crisis 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;
- diversion of healthcare resources away from the conduct of 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 nonclinical 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 operations due to impact from the public health crisis;
- changes in regulations as part of a response to an epidemic or 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 an infectious disease 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.

In addition, to the extent a health epidemic or 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 (EU), 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 EU, 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 EU 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 active moiety in a new 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.

A Fast Track designation by the FDA may not actually lead to a faster development or regulatory review or approval process.

We may seek Fast Track designation for some of our product candidates. If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this condition, the product candidate may be eligible for Fast Track designation. The benefits of Fast Track designation include more frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval, more frequent written communication from the FDA about such things as the design of the proposed clinical trials and use of biomarkers, eligibility for Accelerated Approval and Priority Review, if relevant criteria are met, and rolling review for its NDA. Even with the Fast Track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

Any Breakthrough Therapy designation that we may receive from the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek Breakthrough Therapy designation for some of our product candidates. A Breakthrough Therapy is defined as a drug that 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. For drugs that have been designated as a Breakthrough Therapy, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as a Breakthrough Therapy by the FDA are also eligible for accelerated approval. Designation as a Breakthrough Therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a Breakthrough Therapy, the FDA may disagree and instead determine not to make such designation. We cannot be sure that any evaluation we may make of our product candidates as qualifying for Breakthrough Therapy designation will meet the FDA's expectations. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as a Breakthrough Therapy, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

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, HD, FECD 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.

In February 2023, the FDA approved omaveloxolone, a Nrf2 activator, for the treatment of FA in adults and adolescents aged 16 years and older and omaveloxolone was commercially launched by Reata Pharmaceuticals in June 2023. Reata Pharmaceuticals was acquired by Biogen in September 2023. We are also aware of a number of companies with active clinical-stage FA programs including (i) Larimar Therapeutics evaluating CTI-1601, a cell penetrating peptide FXN recombinant fusion protein, (ii) Lexeo Therapeutics evaluating a cardiac targeted FXN gene therapy, (iii) Minoryx Therapeutics evaluating leriglitazone, a PPAR-gamma agonist, (iv) PTC Therapeutics evaluating vatiquinone, a 15-lipoxygenase inhibitor, and (v) Solid Biosciences evaluating a recombinant AAV-based gene replacement therapy. In addition, several companies have stated that they have preclinical gene therapy programs for FA including Capsida Biotherapeutics, Papillon Therapeutics and Voyager Therapeutics.

We are aware of a number of companies with active clinical-stage FECD programs including (i) Aurion Biotech evaluating a combination cell therapy comprised of donor cells and a Rho kinase inhibitor, (ii) Emmecell evaluating donor cornea endothelial cells delivered through magnetic nanoparticles injected into the anterior chamber, (iii) Kowa Pharmaceutical evaluating Ripasudil, a Rho kinase inhibitor, for use in conjunction with corneal surgery, (iv) Santen Pharmaceutical evaluating STN1010904 (licensed from ActualEyes Inc.), an mTOR inhibitor, and (v) Trefoil Therapeutics evaluating TTHX1114, an engineered FGF1 delivered via intracameral injection, for use in conjunction with corneal surgery.

We are aware of a number of programs for DM1 including (i) AMO Pharma evaluating tideglusib, a GSK3-ß inhibitor, (ii) Arrowhead Pharmaceuticals evaluating an RNA interference (RNAi) conjugate, (iii) Arthex Biotech evaluating anti-miRNA oligonucleotides, (iv) Avidity Biosciences evaluating an antibody linked siRNA, (v) Dyne Therapeutics evaluating an antibody linked oligonucleotide, (vi) EditForce evaluating an RNA editing technology, (vii) Harmony Biosciences evaluating a histamine 3 receptor for the treatment of excessive daytime sleepiness in DM1, (viii) Juvena Therapeutics evaluating JUV-161, a stem cell-secreted protein, (ix) Modalis Therapeutics evaluating MDL-202, a gene therapy candidate, (x) PepGen evaluating a peptide conjugated antisense oligonucleotide, (xi) Transition Bio evaluating condensate therapeutics, and (xii) Vertex Pharmaceuticals evaluating a peptide conjugated oligonucleotide (licensed from Entrada Therapeutics).

We are aware of a number of companies with active clinical-stage HD programs including (i) Alnylam Pharmaceuticals evaluating an RNAi therapeutic, (ii) Annexon Biosciences evaluating a monoclonal antibody, (iii) Hoffmann-La Roche AG evaluating an antisense oligonucleotide candidate and a gene therapy candidate, (iv) Prilenia Therapeutics evaluating a sigma-1 receptor agonist, (v) PTC Therapeutics evaluating a splicing modifier, (vi) Skyhawk Therapeutics evaluating a splicing modifier, (vii) uniQure evaluating an AAV delivered miRNA, (viii) Vaccinex evaluating a monoclonal antibody, (ix) VICO Therapeutics evaluating an antisense oligonucleotide, and (x) Wave Life Sciences evaluating an antisense oligonucleotide.

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, Rocket Pharmaceuticals, Sangamo Biosciences and Verve Therapeutics (a wholly owned subsidiary of Eli Lilly and Company).

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.

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 clinical trials and perform some of our research and nonclinical 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 nonclinical testing or clinical trials ourselves. As a result, we are and expect to remain dependent on third parties to conduct our nonclinical studies, our ongoing clinical trials 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 nonclinical studies and 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 Good Laboratory Practice (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 nonclinical 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

nonclinical studies and clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional nonclinical 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. The 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, provide services in a timely manner or perform as contractually required. These risks may be heightened as a result of the efforts of government agencies and the CROs themselves to limit the spread of an infectious disease, including quarantines and shelter-in-place orders, which have adversely impacted the supply chain for many research and clinical supplies, including animals for nonclinical testing. In addition, demand for CROs and their resources and services has increased in recent years, which has impacted performance timelines. Furthermore, there are shortages in the supply of materials and animal availability for nonclinical testing, which are required to conduct nonclinical studies. This has led us to experience increased competition for CRO services, including, without limitation, scheduling nonclinical studies and delays in study reporting, which could impact development timelines. 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 nonclinical testing and 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 nonclinical 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. For example, as previously announced in May 2023, an unanticipated issue related to the vial stopper used by our vendor for manufacturing the prior DT-216 product candidate caused a short delay in product supply for the Phase 1 MAD clinical trial. In addition, we currently have only one supplier for one of the excipient components of DT-216P2. We may not be able to establish additional sources of supply for this excipient component of DT-216P2 or our other product candidates on a timely basis, or at all, or may be unable to do so on acceptable terms.

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 global supply chain challenges, geopolitical events impacting trade with global partners, labor shortages, or a health epidemic or 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, including for one of the excipient components of DT-216P2, 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.

Certain Chinese biotechnology companies, CROs and contract development and manufacturing organizations may become subject to trade restrictions, sanctions, other regulatory requirements, or proposed legislation by the U.S. government, which could potentially impact services available for our research and development or our ability to secure the materials we need for our product candidates. For example, the House of Representatives of the prior Congress (the 118th Congress) passed the BIOSECURE Act, which proposed prohibiting U.S. government contracts, grants, and loans to entities that use equipment and services from certain named Chinese biotech companies, and authorized the U.S. government to name additional Chinese biotechnology companies of concern. The BIOSECURE Act did not become law in the 118th Congress. It is unclear whether the current Congress (the 119th Congress) will introduce the BIOSECURE Act or similar legislation in this congressional session and, if so, how the scope, prohibitions, or designated biotechnology companies of concern may differ from the version of the BIOSECURE Act passed by the House in the prior 118th Congress.

Our or a third party's failure to execute on our manufacturing requirements, or to 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 nonclinical 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 any such products.

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

As product candidates progress through nonclinical 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, manufacture 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 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 nonclinical studies 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. For instance, while DT-216 was generally well-tolerated and exhibited the ability to overcome the FXN transcription impairment that causes FA in the Phase 1 SAD clinical trial and the Phase 1 MAD clinical trial, injection site thrombophlebitis was observed in patients at lower dose levels in the Phase 1 MAD clinical trial. Instead of advancing the prior DT-216 product candidate through to Phase 2 in the second half of 2023 as originally planned, due to concern for potential worsening of injection site thrombophlebitis at higher doses with multiple administration, we decided to pursue development of a potentially improved formulation using a novel and proprietary excipient with DT-216P2 which has shown favorable injection site tolerability that we believe may better support the future clinical development and regulatory pathway for the drug substance, DT-216. It is possible that our product candidates may require additional formulation or drug substance changes in the future to support continued clinical development. In such event, our development timelines may be materially adversely impacted.

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, a 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, the Centers for Medicare & Medicaid Services (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 this 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 nonclinical 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 global supply chain challenges, labor shortages or a health

epidemic or pandemic, 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 Business Operations, Employee Matters and Managing Growth

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 claim, 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 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 at our leased laboratory and office space in Carlsbad, California and remotely. 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 September 30, 2025, we had 55 employees. In addition, we also utilize specialized contract research organizations for additional research and development personnel. Together with our employees, our team comprised approximately 123 full-time equivalents as of September 30, 2025. 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 when needed, we may not be able to successfully implement the tasks necessary to further develop and eventually 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. As of December 31, 2024, we had U.S. federal and state net operating loss (NOL) carryforwards of $86.4 million and $11.1 million, respectively. U.S. federal and state NOL carryforwards totaling $0.1 million and $10.2 million respectively, begin to expire in 2037, unless previously utilized. The U.S. federal and certain state NOL carryforwards of $86.3 million and $0.9 million, respectively, generated after 2017, may be carried forward indefinitely but the deductibility of such net operating loss carryforwards in a year is limited to 80% of taxable income in such year. In addition, we have U.S. federal and state research and development (R&D) credit carryforwards totaling $9.0 million and $3.2 million, respectively. The U.S. federal R&D credit carryforwards will 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, 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. 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 completed a Section 382 study to assess whether an ownership change had occurred from our formation through December 31, 2021. Based upon the study, we determined that we experienced multiple ownership changes during 2020, causing the annual utilization of NOLs, R&D credits, and other applicable tax attributes generated before then to be limited. We may have experienced additional ownership changes since December 31, 2021, and may experience additional 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 further limitations, which could potentially result in increased future tax liability to us.

Risks Related to Government Regulation

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 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively, 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 CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, as well as information regarding ownership and investment interests held by the physicians described above and their immediate family members.
- 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 amendments to and executive, judicial and congressional challenges to certain aspects of the Affordable Care Act. For example, on August 16, 2022, the Inflation Reduction Act of 2022 (Inflation Reduction Act) was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in Affordable Care Act marketplaces through plan year 2025. The Inflation Reduction Act also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is unclear how any additional healthcare reform measures of the current administration will impact the Affordable Care Act or our business.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, on July 4, 2025, the annual reconciliation bill, the "One Big Beautiful Bill Act" (OBBBA), was signed into law which is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. OBBBA also narrows access to ACA marketplace exchange enrollment and declines to extend the ACA enhanced advanced premium tax credits, set to expire in 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Further, 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, which, due to subsequent legislative amendments, including the Infrastructure Investment and Jobs Act and the Consolidated Appropriations Act of 2023, will stay in effect through 2032.

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 Inflation Reduction Act, among other things, (i) directs the Department of Health and Human Services (HHS) to negotiate the price of certain high-expenditure, single source drugs that have been on the market for at least 7 years covered under Medicare (the Medicare Drug Price Negotiation Program), and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law, and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. The Inflation Reduction Act permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. These provisions took effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon prices of the first 10 drugs that were subject to price negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected 15 additional drugs covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework.

The current administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, CMS and related agencies. These actions and proposals, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions include, for example, (1) directives to reduce agency workforce; (2) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending; (3) eliminating the Biden administration's executive order that directed HHS to establishing an AI task force and developing a strategic plan; (4) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (5) imposing tariffs of imported pharmaceutical products; (6) directing certain federal agencies to enforce existing law regarding hospital and price plan transparency and by standardizing prices across hospitals and health plans; and (7) as part of the Make America Healthy Again (MAHA) Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program created under the IRA. We expect additional health reform measures may be implemented in the future, particularly given the recent change in administration.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control costs of 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.

We and the third parties with whom we work are subject to stringent and changing U.S. and foreign laws, regulations and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Our, or our third parties', actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions, litigation (including class claims) and mass arbitration demands, fines and penalties, disruptions of our business operations, reputational harm and other adverse business impacts.*

In the ordinary course of business, we and our collaborators and third-party providers collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, such as proprietary and confidential business data, trade secrets, intellectual property, and data we collect about trial participants in connection with clinical trials. Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts, and other obligations that govern the processing of sensitive information by us and on our behalf. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including federal health information

privacy laws, state data breach notification laws, state health information privacy laws and consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act) and other similar laws (e.g., wiretapping laws). In addition, we obtain health information from certain third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA, as amended by HITECH, which imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Depending on the facts and circumstances, we could be subject to significant penalties if we violate HIPAA.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services to the extent we become subject to these laws in the future. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018 (CCPA) applies to personal data of consumers, business representatives, employees and other individuals who are California residents, and requires businesses subject to the CCPA to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines and allows private litigants affected by certain data breaches to recover significant statutory damages. While these states, like the CCPA, also exempt some data processed in the context of clinical trials, these developments further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely, should we become subject to them in the future.

Outside the United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation (EU GDPR) and the United Kingdom’s GDPR (UK GDPR) (collectively, GDPR) impose strict requirements for processing the personal data of individuals located, respectively, within the European Economic Area (EEA) and the United Kingdom (UK). For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines up to 20 million Euros under the EU GDPR / 17.5 million pounds sterling under the UK GDPR, or 4% of the annual global revenue, whichever is greater in either case; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. 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 or change our business practices to ensure compliance with European and other foreign data protection rules.

In Canada, the Personal Information Protection and Electronic Documents Act (PIPEDA) and various related provincial laws, as well as Canada’s Anti-Spam Legislation (CASL), may apply to our operations as we expand our clinical trials. Australia’s Privacy Act may also apply to our operations as we expand our clinical trials.

Certain jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the EEA and the UK have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions have adopted similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EU and UK to the United States in compliance with law, such as the EEA standard contractual clauses and the EU-U.S. Data Privacy Framework and UK extension thereto (which allows for transfers to relevant organizations based in the United States who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR’s cross-border data transfer limitations.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities located in or controlled by individuals or entities located in those jurisdictions) that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to engage in transactions or agreements with certain third parties in the future.

Our employees and personnel use generative artificial intelligence (AI) and/or automated decision-making technologies to perform certain tasks at work, and the disclosure and use of personal data in AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating AI and/or automated decision-making technologies. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and consumer lawsuits. If we are unable to use generative AI and/or automated decision-making technologies, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. For example, certain privacy laws, such as the GDPR and the CCPA, require customers to impose specific contractual restrictions on their service providers. We may publish privacy policies and other statements concerning data privacy, and security. Regulators in the United States are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

Obligations related to data privacy and security (and consumer data privacy expectations) are quickly changing in an increasingly stringent fashion, creating some uncertainty as to the effective future legal framework. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and has, in some instances, prompted changes to our information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. In addition, these obligations may even require us to change our business model. Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third parties upon whom we rely may fail to comply with such obligations which could negatively impact our business operations and compliance posture. If we fail, or are perceived to have failed, to address or comply with data privacy and security obligations, we could face significant consequences. These consequences include, but are not limited to, government enforcement actions (e.g., investigations, fines, penalties, audits, inspections and similar); litigation (including class-related claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal data; orders to destroy or not use personal data; and imprisonment of company officials. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for significant statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: interruptions or stoppages in our business operations (including, as relevant, clinical trials); inability to process personal data or operate in certain jurisdictions; limited ability to develop or commercialize our product candidates; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or revision or restructuring our operations.

Disruptions to the operations of the FDA, the SEC, other U.S. governmental agencies or comparable foreign regulatory authorities caused by funding shortages, leadership changes, staffing cuts or other staffing shortages, along with uncertainty regarding the potential for new initiatives, laws, regulations, policies and guidance affecting our product candidates or other aspects of our business, could materially and adversely affect our business.*

The ability of the FDA or other comparable foreign regulatory authorities to review and approve new products or take action with respect to other regulatory matters can be affected by a variety of factors, including government budget and funding levels, leadership changes, the ability to hire and retain key personnel and accept payment of user fees, the availability of personnel and other resources, changes in statutes, regulations and policies that affect the FDA's or comparable foreign regulatory authorities' ability to perform routine functions, and other business disruptions. Average review times at the FDA and comparable foreign regulatory authorities have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. In addition, there have recently been terminations of large numbers of federal employees at various federal agencies, including the FDA. Changes and cuts in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review IND submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion, or at all. A prolonged government shutdown occurs and/or employee terminations or resignations could significantly impact the ability of the FDA or other federal agencies to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns and/or employee terminations or resignations at the SEC could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

There is substantial uncertainty as to whether and how the current administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates and any products for which we obtain approval. This uncertainty could present new challenges as we navigate development and approval of our product candidates. Some of these efforts have manifested to date in the form of personnel cuts and measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development and obtain the requisite regulatory approvals in the future. There is uncertainty as to whether we will be materially and negatively impacted by governmental orders, regulations, policies or guidance, or disruptions to the normal operations of government agencies.

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, trade secrets, 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 product candidates and their uses, platform technologies, 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 provide assurance 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 will 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. Recent reforms and changes at government agencies of the United States and those of non-U.S. jurisdictions could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications, and the maintenance, enforcement, or defense of our issued patents. For example, the ability of the USPTO and other applicable patent authorities to properly administer their functions is highly dependent on the levels of funding available to the agency and their ability to retain key personnel and fill key leadership appointments, among various factors. Termination of employees or delays in replacing or hiring for key positions could significantly impact the ability of the USPTO and other applicable patent authorities to fulfill their functions and could greatly impact our ability to timely and adequately prosecute or maintain our patent applications, and our ability to timely and adequately maintain, enforce, or defend our issued patents. 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 pharmaceutical and biological 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 until issuance of the patent. 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 that 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 we may experience patent office interruption or delays to our ability to timely secure patent coverage to our product candidates as a result of factors outside our control.

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 issued patents 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. In Europe, beginning June 1, 2023, European applications and patents may be subjected to the jurisdiction of the Unified Patent Court (UPC). Also, European applications will have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the UPC. This will be a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty. As a single court system can invalidate a European patent, we, where applicable, may opt out of the UPC and as such, each European patent would need to be challenged in each individual country.

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.

Geopolitical actions in the United States and in foreign countries (such as retaliatory measures by foreign countries in response to actions by the United States, in particular, tariffs) could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, the United States and foreign government actions related to Russia's invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. Further, many foreign countries could threaten to impose retaliatory measures that may adversely impact our intellectual property rights in those countries. For example, on March 14, 2025, Brazil enacted Law No. 15. 122/2025 (known as the "Economic Reciprocity Law"), which provides a framework that allows for the suspension of obligations related to foreign entities' intellectual property rights. Additionally, changes in US trade policy, including the imposition of new or increased tariffs as well as retaliatory measures by other countries, could adversely affect our patent strategy, such as where we choose to file, maintain, or enforce our patents. Also, if we are required to move our research or

manufacturing activities to new regions, this may expose us to jurisdictions with weaker intellectual property enforcement, differing patent eligibility standards, or greater risk of compulsory licensing. These factors could compromise the protection or value of our proprietary technologies, including our core patents and related know-how.

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.

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 Leahy-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 included 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 16, 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 this inventor was the first to invent the claimed invention. As a result, a third party that files a patent application in the USPTO on or after March 16, 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 until publication or 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. We cannot predict how decisions by the federal courts, the U.S. Congress or the USPTO may impact the value of our patent rights. For example, the Supreme Court of the United States held in *Amgen v. Sanofi* (2023) that a functionally claimed genus was invalid for failing to comply with the enablement requirement of the Patent Act. In addition, the Federal circuit recently issued a decision, *In re Cellect, LLC* (2023) involving the interaction of patent term adjustment (PTA), terminal disclaimers, and obvious-type double patenting which may affect the patent term of any issued patents that rely on any PTA. 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. In the 2013 case Assoc. for Molecular Pathology v. Myriad Genetics, Inc., for instance, 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. For example, the Inflation Reduction Act (IRA) passed by Congress authorizes the Secretary of the Department of HHS to negotiate prices directly with participating manufacturers for selected medicines covered by Medicare even if these medicines are protected by an existing patent. For small molecule medicines, the process begins seven years after initial approval by the FDA. While we do not believe that the IRA or its effects will impact our ability to obtain patents in the near future, we cannot be certain whether it will affect our patent strategy in the long run. Additionally, in July 2025, the FDA announced its intent to increase transparency by publicly releasing portions of Complete Response Letters (CRLs) issued to drug and biologic sponsors. While the FDA has stated that confidential information will be protected, it remains unclear how such disclosures will be implemented. Because CRLs often contain specific observations about study design, clinical endpoints, chemistry, manufacturing, and controls (CMC) data, or other proprietary information, any public release could unintentionally disclose information that competitors may use to infer proprietary aspects of our product candidates or platform technologies. This could compromise the confidentiality of our trade secrets and know-how or facilitate third-party efforts to design around or challenge the validity, enforceability, or scope of our patents, or accelerate the development of generics or biosimilars. If we are required to modify or limit the information shared with the FDA to mitigate such risks, it could increase costs, slow our regulatory interactions, or delay product approval timelines.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, 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 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.

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 are 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, copyrights, 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.

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

We are currently party to a license agreement with 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 nonclinical 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.

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 currently or 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 current or future licensors or collaboration partners. If any of our current or 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 current or future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

We have entered into and may in the future enter into license agreements with others that may 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 current or 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 product candidates that are subject of such licensed rights could be adversely affected.

Our current or future licensors may rely on third-party consultants or collaborators or on funds from third parties such that our current or future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to our current or 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 current or 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 current or 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 current or 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;
- 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 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 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.

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. Additionally, generative AI resources that are publicly available also present a risk that a company may inadvertently obtain, incorporate or use a third party's intellectual property.

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. As a result, we may be unable to identify such patents or patent applications despite our best efforts. 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.

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 product 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 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 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 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-examination, 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 European Patent Office (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, or 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.

We may rely on trade secrets 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 security incident) 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, apply prior to expiration of relevant patents or otherwise satisfy applicable requirements. Administrative changes at the USPTO or other applicable patent authorities, such as reduced hiring and/or funding, may result in delays in issuance of a patent or in accrual of PTE, thereby reducing the amount of PTE that could otherwise be received. Administrative changes (e.g., at the FDA or USPTO) may also lead to delays in review and analysis of requests for PTE, which could result in a PTE not being timely granted (e.g., before the expiration of the patent). If we are unable to obtain PTE or other patent term 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 nonclinical 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, we 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 the Securities Market and Ownership of Our Common Stock

An active trading market for our common stock may not be sustained, which may make it difficult for you to sell your shares.

Prior to our initial public offering in March 2021, there had been no public market for our common stock. The trading market for our common stock on The Nasdaq Global Select Market has been limited and an active trading market for our shares may not be sustained. If an active market for our common stock is not sustained, it may be difficult for you to sell your shares at a price that is attractive to you, or at all.

The price of our common stock could be subject to volatility related or unrelated to our operations.*

Our stock price may be volatile. The stock market in general and the market for biotechnology and pharmaceutical companies, in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your shares at a price that is attractive to you, or at all. The market price for our common stock may be influenced by 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 clinical trials with our current and future product candidates or of our competitors;
- adverse results or delays in nonclinical studies or 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 our 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;
- macroeconomic conditions, such as inflation, tariffs, other fiscal and trade policy changes and market conditions in our industry;
- trading activity by our principal stockholders;
- the size of our market float;
- political uncertainty and/or instability in the United States;
- wars, military conflicts and other geopolitical events;
- global supply chain and/or labor shortage challenges;
- a health epidemic or pandemic and actions taken to slow its spread; and
- any other factors discussed in this report.

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 recently. 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.

Certain of our executive officers, directors and large stockholders own a significant percentage of our outstanding capital stock. As a result of their share ownership, 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.

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 automatically increases on January 1 of each calendar year 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. In addition, our 2021 Employee Stock Purchase Plan (ESPP) authorizes the issuance of 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 under the ESPP automatically increases on January 1 of each calendar year by the lesser of 1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year and 1,200,000 shares, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. Increases in the number of shares available for future grant or purchase may result in 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 we intend to take advantage of reduced reporting requirements.

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:

- 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.

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) December 31, 2026 (the last day of the fiscal year following the fifth anniversary of the completion of our initial public offering), (ii) the last day of the fiscal year in which we have total annual gross revenue of at least $1.235 billion, (iii) the date upon which we are deemed to be a "large accelerated filer", which means we have been subject to the reporting requirements of the Securities Exchange Act of 1934, as amended (Exchange Act), for at least 12 months as of December 31st, and the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th and (iv) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to public company reporting and compliance initiatives.

As a public company listed on the Nasdaq Global Select Market, we 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 Select 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 required the SEC to adopt 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 our initial public offering. We intend to continue to take advantage of this 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 increase our legal and financial compliance costs and make some activities more time-consuming and costlier. For example, these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance and we are required to incur substantial costs to maintain our current levels of such coverage.

If we fail to maintain an effective system of disclosure controls and internal control over financial reporting, our ability to produce timely and accurate financial statements or comply with applicable regulations could be impaired.

As a public company, we are subject to requirements of the Sarbanes-Oxley Act, the regulations of the Nasdaq Global Select Market, the rules and regulations of the SEC, expanded disclosure requirements, accelerated reporting requirements and more complex accounting rules. We expect that the requirements of these rules and regulations will continue to increase our legal, accounting and financial compliance costs, make some activities more difficult, time-consuming and costly and place significant strain on our personnel, systems and resources. Company responsibilities required by the Sarbanes-Oxley Act include, among other things, that we maintain corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. We are continuing to develop and refine our disclosure controls and other procedures that are designed to ensure that information required to be disclosed by us in the reports that we file with the SEC is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms and that information required to be disclosed in reports under the Exchange Act is accumulated and communicated to our principal executive and financial officers. We are also continuing to improve our internal control over financial reporting. In order to develop, maintain, and improve the effectiveness of our internal controls and procedures, and internal control over financial reporting, we have expended, and anticipate that we will continue to expend, significant resources, including accounting-related costs and significant management oversight.

Our current controls and any new controls that we develop may become inadequate because of changes in conditions in our business. Further, weaknesses in our disclosure controls and internal control over financial reporting may be discovered in the future. Any failure to develop or maintain effective controls or any difficulties encountered in their implementation or improvement could harm our results of operations or cause us to fail to meet our reporting obligations and may result in a restatement of our financial statements for prior periods. Any failure to implement and maintain effective internal control over financial reporting could also adversely affect the results of periodic management evaluations and annual independent registered public accounting firm attestation reports regarding the effectiveness of our internal control over financial reporting that we will eventually be required to include in our periodic reports that will be filed with the SEC. Ineffective disclosure controls and procedures and internal control over financial reporting could also cause investors to lose confidence in our reported financial and other information, which would likely have a negative effect on the trading price of our common stock. In addition, if we are unable to continue to meet these requirements, we may not be able to remain listed on the Nasdaq Global Select Market.

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. Any failure to maintain effective disclosure controls and internal control over financial reporting could have a material and adverse effect on our business, results of operations and financial condition and could cause a decline in the trading price of our common stock.

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 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 U.S. government recently enacted legislation commonly referred to as the One Big Beautiful Bill Act, that (along with other recent U.S. federal tax reform) has resulted in significant changes to the taxation of business entities including, among other changes, changes to the taxation of income derived from international operations, changes in the deduction and amortization of research and development expenditures, and limitations on the deductibility of business interest. Future guidance from the Internal Revenue Service and other tax authorities with respect to any legislation may affect us, and certain aspects of such legislation could be repealed or modified or sunset in future years. The current administration and Congress could also enact other tax law changes that could have an adverse effect on our operations, cash flows and results from operations and contribute to overall market volatility. In addition, it is uncertain if and to what extent various states

will conform to 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 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.

Our information technology systems or sensitive data, or those of our third-party CROs or other contractors, consultants, or third parties with whom we work, may fail or suffer security incidents, loss or leakage of data, and other disruptions, which could result in a material disruption of our product candidates' development programs, compromise sensitive data related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.*

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we or our third-party CROs or other contractors or consultants acting on our behalf process proprietary, confidential, and sensitive data (including but not limited to intellectual property, proprietary business information, health-related data, and personal data) (collectively, sensitive information).

Cyberattacks, malicious internet-based activity, and online and offline fraud are prevalent and continue to increase. These threats are becoming increasingly difficult to detect. These threats come from a variety of sources, including traditional computer "hackers," threat actors, personnel misconduct or error (such as theft or misuse), sophisticated nation-state and nation-state supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including, without limitation, nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including cyber-attacks, that could materially disrupt our systems and operations. We and our third-party CROs or other contractors or consultants, are subject to a variety of evolving threats, including but not limited to social engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks, credential stuffing or harvesting, ransomware attacks, supply-chain attacks, software bugs, server malfunction, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, attacks enhanced or facilitated by AI, earthquakes, fire, flood, and other similar threats. Ransomware attacks, including those perpetrated by organized criminal threat actors, nation-states, and nation-state supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions in our operations, loss of data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting payments. Similarly, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain have not been compromised or that they do not contain exploitable defects or bugs that could result in a breach of or disruption to our information technology systems or the third-party information technology systems that support us and our services. It may be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems.

Remote work has increased risks to our information technology systems and data, as our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations. Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

While we have implemented measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps designed to detect, mitigate and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties upon which we rely) and security incidents. We have not and may not in the future, however, be able to detect and remediate all such vulnerabilities and security incidents, including on a timely basis. Further, we have and may in the future experience delays in developing and deploying remedial measures and patches designed to address any such identified vulnerabilities and security incidents. Vulnerabilities could be exploited and result in security incidents.

We have outsourced elements of our operations to third parties, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email, and other functions, and as a result we rely on a number of third-party contractors who have access to our sensitive information. We share or receive sensitive information with or from third parties. Our ability to monitor these third parties' cybersecurity practices is limited, and these third parties may not have adequate information security measures in place. If the third parties with whom we work experience a security incident or other interruption, which has occurred in the past, we could experience adverse consequences. While we may be entitled to damages if the third parties

with whom we work fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

Any of the previously identified or similar threats could cause a security incident or other interruption, and has in the past. For example, we frequently experience minor phishing attempts and may experience more significant phishing attempts in the future. A security incident or other interruption could result in unauthorized, unlawful or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of or access to data. A security incident or other interruption could disrupt our (and third parties upon whom we rely) ability to develop or provide our products or conduct clinical trials. We may expend significant resources or modify our business activities (including our clinical trial activities) in an effort to protect against security incidents. Additionally, certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and data.

Applicable data privacy and security obligations as well as public company disclosure obligations may require us, or we may choose, to notify relevant stakeholders, including affected individuals, regulators and investors, of certain security incidents or to take other actions, such as providing credit monitoring and identity protection services. Such disclosures and related actions can be costly, and the disclosures or the failure to comply with applicable requirements could lead to adverse impacts. If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, which has occurred in the past, we may experience adverse consequences. These consequences may include the following: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing data (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may impact our ability to conduct clinical trials or bring any approved products to market, and negatively impact our ability to grow and operate our business. Whether a cybersecurity incident is reportable to our investors may not be straightforward, may take considerable time to determine, and may be subject to change as the investigation of the incident progresses, including changes that may significantly alter any initial disclosure that we provide. Moreover, experiencing a material cybersecurity incident and any mandatory disclosures could lead to negative publicity, loss of investor or partner confidence in the effectiveness of our cybersecurity measures, diversion of management's attention, governmental investigations, lawsuits, and the expenditure of significant capital and other resources.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, our sensitive information could be leaked, disclosed, or revealed as a result of or in connection with our employees', personnel's, or vendors' use of generative AI technologies.

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, nonclinical 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. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. 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 or contract with, 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 may also result in substantial fines, penalties or other sanctions.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Our status as a Delaware corporation and the anti-takeover provisions of the Delaware General Corporation Law (DGCL) 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 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 DGCL. 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.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the 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 asserting a claim of breach of a fiduciary duty; (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 DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; and (iv) 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.

Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such 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 further provides that 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.

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 against us and our directors, officers, and other employees. While the Delaware courts have determined that such choice of forum provisions are facially valid, and several state trial courts have enforced such provisions and required that suits asserting Securities Act claims be filed in federal court, there is no guarantee that courts of appeal will affirm the enforceability of such provisions and 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. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with litigating Securities Act claims in state court, both state and federal court, or other jurisdictions which could seriously harm our business, financial condition, results of operations, and prospects.

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.

General Risk Factors

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

The global credit and financial markets have experienced extreme volatility and disruptions in the past. These disruptions have resulted or can result in severely diminished liquidity and credit availability, high inflation, rising interest rates, declines in consumer confidence, disruptions in access to bank deposits or lending commitments due to bank failures and uncertainty about economic stability, 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, higher inflation, 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. Our portfolio of investment securities could also be adversely impacted. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our operations, growth strategy, financial performance and stock price and could require us to delay or abandon clinical or research 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 or rising inflation, which could directly affect our ability to attain our operating goals on schedule and on budget.

Other international and geo-political events could also have a serious adverse impact on our business, including the geopolitical tensions between the U.S. and China, the Russia/Ukraine conflict, and the conflict in the Middle East which has recently included actions by Iran, Hamas, Israel and the United States. While we cannot predict the broader consequences, the conflict and retaliatory and counter-retaliatory actions has affected and could further affect, potentially materially and adversely, global trade, currency exchange rates, inflation, regional economies, and the global economy, which in turn may increase our costs, disrupt our supply chain, impair our ability to raise or access additional capital when needed on acceptable terms, if at all, or otherwise adversely affect our business, financial condition, and results of operations.

If there are substantial sales of shares of our common stock, the market 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.

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

We are 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.

If securities or industry analysts 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. If 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Use of Proceeds

On March 25, 2021, we commenced our initial public offering pursuant to a registration statement on Form S-1 (File No. 333-253954) that was declared effective by the SEC on March 25, 2021, for 12,000,000 shares of our common stock for sale to the public at a price of $20.00 per share. In addition, in March 2021, the underwriters exercised their over-allotment option to purchase 1,800,000 additional shares of our common stock in the initial public offering at the public offering price of $20.00 per share, such that the aggregate offering price of our initial public offering was $276.0 million. The net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, were $254.3 million. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10% or more of any class of our equity securities or to any other affiliates. The underwriters for our initial public offering were Goldman Sachs & Co. LLC, SVB Leerink LLC and Piper Sandler & Co.

The net proceeds from our initial public offering are being held in cash, cash equivalents and investment securities, primarily in money market funds invested in U.S. government agency securities and U.S. Treasury securities. These investments are made pursuant to our investment policy and we may further invest these funds in high-quality marketable debt instruments of corporations and government sponsored enterprises with contractual maturity dates of generally less than two years until needed to fund our operations. There has been no material change in the use of proceeds from our initial public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on March 26, 2021 and through September 30, 2025, we had used approximately $126.9 million of the net proceeds received from our initial public offering to support our operations.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

There are no disclosures required by this Item 5, including those relating to “Rule 10b5-1 trading arrangements” and “non-Rule 10b5-1 trading arrangements” as those terms are defined in Item 408 of Regulation S-K.

Item 6. Exhibits.

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed March 30, 2021).
3.2	Amended and Restated Bylaws (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed March 30, 2021).
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended, filed March 22, 2021).
4.2	Amended and Restated Investors' Rights Agreement, by and between the Registrant and certain of its stockholders, dated January 25, 2021 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, filed March 5, 2021).
31.1	Certification of Principal Executive and Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Principal Executive and Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Design Therapeutics, Inc.

Date: November 5, 2025

By: /s/ Pratik Shah, Ph.D.
Pratik Shah, Ph.D.
President, Chief Executive Officer and Chairperson
(Principal Executive and Financial Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Pratik Shah, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Design Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. I am responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under my supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to me by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under my supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report my conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. I have disclosed, based on my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 5, 2025

By: /s/ Pratik Shah, Ph.D.

Pratik Shah, Ph.D.
President, Chief Executive Officer and Chairperson
(Principal Executive and Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Design Therapeutics, Inc. (the "Company") for the quarterly period ended September 30, 2025, to which this Certification is attached, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

The undersigned has executed this Certification effective as of November 5, 2025.

By: /s/ Pratik Shah, Ph.D.
Pratik Shah, Ph.D.
President, Chief Executive Officer and Chairperson
(Principal Executive and Financial Officer)

The foregoing certification is being furnished solely to accompany the Report pursuant to 18 U.S.C. § 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Company, whether made before or after the date hereof, regardless of any general incorporation language in such filing. A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.