Design Therapeutics Highlights Clinical and Research Progress and Reports First Quarter 2022 Financial Results

May 9, 2022

Clinical Execution of Lead GeneTAC™ Molecule, DT-216, with Initiation of Phase 1 Trial for the Treatment of Friedreich Ataxia

Robust Research Pipeline of GeneTAC™ Candidates, with Promising New Data for Fuchs Endothelial Corneal Dystrophy

Strong Cash Position with $371.2 Million to Support Multi-Year Operating Runway

CARLSBAD, Calif., May 09, 2022 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for serious degenerative genetic diseases, today highlighted progress across its clinical and research-stage pipeline of novel GeneTAC™ small molecules and reported first quarter 2022 financial results.

"Design's vision is to become a fully integrated biotech company that is designing, developing, and delivering transformative treatments for patients with debilitating inherited degenerative diseases," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "So far in 2022, we've made meaningful progress toward that vision with the initiation of clinical development with DT-216, our lead GeneTAC™ molecule, as a potential disease-modifying treatment for patients with Friedreich ataxia (FA). In our interactions with the FA community, they express tremendous interest in the development of DT-216, underscoring the urgent need among FA patients. We've continued to deliver on our milestones for this program, recently completing dosing in the first single-ascending dose cohort of our Phase 1 study, and we look forward to reporting topline data in the second half of this year. Other significant advancements in our GeneTAC™ platform are exemplified by the exciting preclinical data we presented at ARVO. We showed that our GeneTAC™ molecules can correct the pathological hallmarks of the most common genetic cause of Fuchs endothelial corneal dystrophy – a disease that affects millions of people, leads to visual impairment and is the number one cause of corneal transplantation. Driven by our expert team, we believe we have developed an efficient operating model that supports a multi-year operating runway and enables us to execute our clinical and research milestones."

Recent Pipeline Progress

- **Significant Clinical and Regulatory Progress with DT-216 for FA; Topline Phase 1 Data On-track for Second Half of 2022:** Design completed dosing in the first cohort of its Phase 1 single-ascending dose (SAD) clinical trial of DT-216 in patients with FA. DT-216 is a novel GeneTAC™ molecule designed to specifically target the GAA repeat expansion mutation, the underlying cause of FA, and restore frataxin (FXN) gene expression. Design's Phase 1 clinical trial of DT-216 is a randomized, double-blind, placebo-controlled study to evaluate the safety, tolerability, pharmacokinetics and FXN levels from single ascending doses of IV-administered DT-216 in adult patients with FA. Preclinical data demonstrated that DT-216 was well tolerated at dose levels projected to achieve concentrations in the CNS, heart and skeletal muscle in excess of those required to restore FXN gene expression in FA patient derived cells in vitro.

  Additionally, DT-216 was recently granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of patients with FA. The Fast Track process is designed to facilitate the development and expedite the review of investigational treatments that demonstrate the potential to address unmet medical needs in serious or life-threatening conditions. With Fast Track designation, DT-216 is eligible for early and frequent communication with the FDA throughout the entire drug development and review process. In addition, it is now eligible for Accelerated Approval and Priority Review, if relevant criteria are met, and a Rolling Review for its New Drug Application.

- **Pipeline Expansion Underway with Novel GeneTAC™ Program Advancing for FECD:**Design presented preclinical data supporting the potential of the company's novel GeneTAC™ small molecules to correct the most common underlying genetic cause of Fuchs endothelial corneal dystrophy (FECD) during a poster session at the Association for Research in Vision and Ophthalmology 2022 Annual Meeting (ARVO 2022). FECD, a genetic eye disease characterized by progressive degeneration of the corneal endothelium and subsequent vision impairment, affects more than one million people in the U.S. and is the leading reason for tens of thousands of corneal transplants each year. When tested *in vitro,* FECD GeneTAC™ molecules led to robust reductions in toxic nuclear RNA foci of up to 99% in a time- and concentration-dependent manner. The FECD GeneTAC™ molecules also corrected key mis-spliced transcripts in FECD corneal endothelial cells to levels observed in unaffected corneal endothelial cells. Based on these findings, Design plans to advance this research program through additional preclinical studies.

Upcoming Investor Conference Presentations

- **2022 RBC Capital Markets Global Healthcare Conference:** João Siffert, M.D., president and chief executive officer, will participate in a fireside chat during the 2022 RBC Capital Markets Global Healthcare Conference on Wednesday, May 18, 2022, at 9:30 a.m. ET. The conference is being held May 17-18, 2022, in New York City. A live webcast will be available in the investor section of the company's website at www.designtx.com. The webcast will be archived for 30 days following the presentation.

First Quarter 2022 Financial Results

- **R&D Expenses**: Research and development (R&D) expenses were $8.8 million for the quarter ended March 31, 2022.
- **G&A Expenses**: General and administrative (G&A) expenses were $4.6 million for the quarter ended March 31, 2022.
- **Net Loss:** Net loss was $13.3 million for the quarter ended March 31, 2022.
- **Cash Position**: Cash, cash equivalents and marketable securities were $371.2 million as of March 31, 2022.

About Design Therapeutics

Design Therapeutics is a clinical-stage biotechnology company developing a new class of therapies based on its platform of GeneTAC™ gene targeted chimera small molecules. The company's GeneTAC™ molecules are designed to either dial up or dial down the expression of a specific disease-causing gene to address the underlying cause of disease. Design's lead program is focused on the treatment of Friedreich ataxia, followed by a program in myotonic dystrophy type-1 and discovery efforts for multiple other serious degenerative disorders caused by nucleotide repeat expansions. For more information, please visit designtx.com.

Forward-Looking Statements

Statements in this press release that are not purely historical in nature are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements related to: Design's vision to become a fully integrated biotech company; projections from preclinical data; the expected timing for reporting topline data; Design's ability to meet its stated milestones; Design's anticipated cash runway; the potential benefits of FXN restoration; the potential benefits of Fast Track designation; Design's FECD GeneTAC™ program and its design and potential therapeutic benefits and advantages, including that Design's FECD GeneTAC™ molecules may be able to correct the pathological hallmarks of the underlying genetic cause of FECD; Design's ability to bring forward a new class of treatments for patients living with devastating genetic diseases; and the capabilities and potential advantages of GeneTAC™ molecules. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "believes," "vision," "designed to," "anticipates," "planned," "expects," "estimate," "intends," "will," "goal," "potential," "project" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Design's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks associated with conducting a clinical trial and patient enrollment, which is affected by many factors, and any difficulties or delays encountered with such clinical trial or patient enrollment may delay or otherwise adversely affect Design's ongoing Phase 1 clinical trial for DT-216; the process of discovering and developing therapies that are safe and effective for use as human therapeutics and operating as a development stage company; Design's ability to develop, initiate or complete preclinical studies and clinical trials for its product candidates; the risk that promising early research or clinical trials do not demonstrate safety and/or efficacy in later preclinical studies or clinical trials; changes in Design's plans to develop its product candidates; uncertainties associated with performing clinical trials, regulatory filings and applications; risks associated with reliance on third parties to successfully conduct clinical trials and preclinical studies; Design's ability to raise any additional funding it will need to continue to pursue its business and product development plans; regulatory developments in the United States and foreign countries; Design's reliance on key third parties, including contract manufacturers and contract research organizations; Design's ability to obtain and maintain intellectual property protection for its product candidates; Design's ability to recruit and retain key scientific or management personnel; competition in the industry in which Design operates, which may result in others discovering, developing or commercializing competitive products before or more successfully than Design; and market conditions. For a more detailed discussion of these and other factors, please refer to Design's filings with the Securities and Exchange Commission ("SEC"), including under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 31, 2021, as filed with the SEC on March 10, 2022, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended March 31, 2022, being filed with the SEC on May 9, 2022. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement and Design undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

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

	Three Months Ended March 31,

	2022	2021
Operating expenses:		
Research and development	8,759	3,875
General and administrative	4,611	1,805
Total operating expenses	13,370	5,680
Loss from operations	(13,370)	(5,680)
Other income, net	105	166
Net loss	$ (13,265)	$ (5,514)
Net loss per share, basic and diluted	$ (0.24)	$ (0.31)
Weighted-average shares of common stock outstanding, basic and diluted	55,507,338	17,630,178

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS
(in thousands)

	March 31, 2022	December 31, 2021
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 371,220	$ 384,064
Prepaid expense and other current assets	2,651	1,371
Total current assets	373,871	385,435
Property and equipment, net	1,448	1,508
Right-of-use asset, related party	3,482	3,614
Total assets	$ 378,801	$ 390,557
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,901	$ 1,620
Accrued expenses and other current liabilities	3,579	3,663
Total current liabilities	5,480	5,283
Operating lease liability, net, related party	3,012	3,144
Total liabilities	8,492	8,427
Convertible preferred stock	—	—
Total stockholders' equity	370,309	382,130
Total liabilities and stockholders' equity	$ 378,801	$ 390,557