Design Therapeutics Highlights Momentum Across Lead GeneTAC® Programs and Reports First Quarter 2025 Financial Results

May 7, 2025

Phase 1 Single Ascending Dose Trial of DT-216P2 for Friedreich Ataxia (FA) Program Ongoing

Reported Favorable Phase 1 Data for DT-168 for Fuchs Endothelial Corneal Dystrophy (FECD) Program

Well-Capitalized with Cash and Securities of $229.7 Million to Fund Operations Through up to Four Potential Clinical Proof-of-Concept Data Sets

CARLSBAD, Calif., May 07, 2025 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today announced progress across its portfolio of GeneTAC® candidates and reported financial results for the first quarter 2025.

“Design continued its progress through the first quarter of 2025, marked by the favorable results from our Phase 1 trial in FECD which, in combination with our biomarker studies, support advancing DT-168 into a Phase 2 biomarker trial in patients later this year,” said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. “We are also conducting our Phase 1 SAD trial in healthy volunteers for FA, where favorable results would position us to begin a Phase 1/2 trial of DT-216P2 in patients. These programs anchor a differentiated GeneTAC® pipeline that we believe could deliver transformative value in genomic medicine, with the potential for multiple clinical proof-of-concept readouts over the next few years.”

Corporate Highlights and Anticipated Upcoming Milestones

- **Friedreich Ataxia (FA):** A Phase 1 clinical trial in healthy volunteers is ongoing to evaluate the safety and pharmacokinetics (PK) of single ascending doses (SAD) of DT-216P2. The results will inform plans for a Phase 1/2 multiple ascending dose (MAD) trial to assess safety, PK and pharmacodynamics of DT-216P2 in FA patients. Design anticipates initiating the DT-216P2 MAD patient study in mid-2025.
- **Fuchs Endothelial Corneal Dystrophy (FECD):** Design reported favorable results from the Phase 1 SAD/MAD clinical trial of DT-168 in healthy volunteers at Eyecelerator @ Park City 2025 in May. The results demonstrated that DT-168 was well-tolerated with no treatment-emergent adverse events and, as expected, systemic exposure was below the limit of quantitation in all participants. In parallel, Design conducted reference range studies that support the potential for corneal endothelium RNA biomarkers as a clinical proof-of-concept measure of drug activity. Based on these findings, Design plans to conduct a Phase 2 biomarker trial of DT-168 to evaluate safety, tolerability and corneal endothelium biomarkers in FECD patients with the TCF4 mutation who are scheduled for corneal transplant surgery. Design plans to initiate the Phase 2 biomarker trial in the second half of 2025.
- **Pipeline programs:** Design continues to progress preclinical activities for its myotonic dystrophy type-1 (DM1) program toward the selection of a development candidate later in 2025. In Huntington’s disease, the company also continues to advance preclinical characterization of several candidate molecules.
- **Leadership appointment:** In April 2025, Design appointed Chris Storgard, M.D., as Chief Medical Officer. Dr. Storgard brings over two decades of leadership and hands-on drug development experience and has successfully advanced multiple assets from preclinical stages through global regulatory approvals.

First Quarter 2025 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $15.4 million for the quarter ended March 31, 2025.
- **G&A Expenses:** General and administrative (G&A) expenses were $5.0 million for the quarter ended March 31, 2025.
- **Net Loss:** Net loss was $17.7 million for the quarter ended March 31, 2025.
- **Cash Position and Operating Runway:** Cash, cash equivalents and investment securities were $229.7 million as of March 31, 2025, which the company expects to fund its planned operating expenses into 2029.

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. In addition to its clinical-stage GeneTAC® programs, DT-216P2, in development for patients with Friedreich ataxia, and DT-168, for Fuchs endothelial corneal dystrophy, the company is advancing programs in myotonic dystrophy type-1 and Huntington’s disease. Discovery efforts are underway for multiple genomic medicines. 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: projections from early-stage programs, nonclinical data and early-stage clinical data; the progression or completion of certain development activities, including the selection of development candidates; the initiation and progression of studies and clinical trials for DT-216P2 and DT-168 and the timing thereof; the expected timing for data readouts; Design’s pipeline,

including the potential to have four programs with clinical proof-of-concept with Design's current cash runway; Design's ability to advance the GeneTAC® platform; Design's estimated cash runway and the sufficiency of its resources to support its planned operations; and the capabilities and potential advantages of Design's pipeline 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," "designed to," "anticipates," "capable of," "on track to," "plans to," "expects," "estimate," "intends," "will," "potential" 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 and uncertainties associated with: the acceptance of INDs by the FDA or similar applications by foreign regulatory agencies for the conduct of planned clinical trials of our product candidates and our proposed design of future clinical trials; the data we observe from earlier clinical and nonclinical studies may impact our clinical development plans; pursuing a biomarker-driven clinical development strategy carries increased risks as there are currently a limited number of approved biomarker-specific therapies; nonclinical development activities and results of nonclinical studies; conducting a clinical trial and patient enrollment, which are 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 clinical development plans; the process of discovering and developing therapies that are safe and effective for use as human therapeutics and operating as a development stage company; undesirable side effects or other undesirable properties, which could cause Design or regulatory authorities to suspend or discontinue clinical trials and thereby delay or prevent Design's product candidates' development or regulatory approval; Design's ability to develop, initiate or complete nonclinical studies and clinical trials for its product candidates; whether promising early research or clinical trials will demonstrate safety and/or efficacy in later nonclinical studies or clinical trials; changes in Design's plans to develop its product candidates; reliance on third parties to successfully conduct clinical trials and nonclinical studies; competitive products, which may make any products we develop or seek to develop obsolete or noncompetitive; Design's reliance on key third parties, including contract manufacturers and contract research organizations; 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 ability to obtain and maintain intellectual property protection for its product candidates; Design's ability to recruit and retain key scientific or management personnel; 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, 2024, as filed with the SEC on March 10, 2025, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, being filed with the SEC later today. 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)

	Three Months Ended March 31,	
	2025	**2024**
	(unaudited)	
Operating expenses:		
Research and development	$ 15,377	$ 9,801
General and administrative	5,041	4,599
Total operating expenses	20,418	14,400
Loss from operations	(20,418)	(14,400)
Other income, net	2,703	3,295
Net loss	$ (17,715)	$ (11,105)
Net loss per share, basic and diluted	$ (0.31)	$ (0.20)
Weighted-average shares of common stock outstanding, basic and diluted	56,757,827	56,488,527

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	March 31, 2025	**December 31, 2024**
	(unaudited)	
Assets		
Current assets:		

Cash, cash equivalents and investment securities	$ 229,674	$ 245,477
Prepaid expenses and other current assets	3,970	2,563
Total current assets	233,644	248,040
Property and equipment, net	1,377	1,410
Right-of-use asset, related party	2,027	2,216
Other assets	427	427
Total assets	$ 237,475	$ 252,093
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,180	$ 2,186
Accrued expenses and other current liabilities	5,056	6,276
Total current liabilities	8,236	8,462
Operating lease liability, net, related party	1,320	1,534
Total liabilities	9,556	9,996
Total stockholders' equity	227,919	242,097
Total liabilities and stockholders' equity	$ 237,475	$ 252,093