Design Therapeutics Highlights Progress Across Lead GeneTAC® Programs and Reports Second Quarter 2025 Financial Results

August 7, 2025

Early Human Pharmacokinetics Data for DT-216P2 Demonstrates Favorable Translation from Non-Human Primates (NHPs) to Humans and Improved DT-216 Product Profile for Friedreich Ataxia (FA) Program

RESTORE-FA Phase 1/2 Multiple-Ascending Dose Trial of DT-216P2 in FA Patients Underway

Phase 2 Biomarker Study for DT-168 Initiated in Patients with Fuchs Endothelial Corneal Dystrophy (FECD)

Cash and Securities of $216.3 Million Support Continued Pipeline Advancement

CARLSBAD, Calif., Aug. 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 second quarter 2025.

"We've made meaningful progress across our pipeline this quarter," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. "Early human PK data for DT-216P2 demonstrate the consistency of human plasma exposure profiles with NHP data across both IV and subcutaneous routes. We're also pleased to have initiated our Phase 2 biomarker trial in patients with FECD, a disease with no approved disease-modifying therapies. Our preclinical programs also continue to advance as we work to deliver a new class of genomic medicines for patients with serious degenerative diseases."

Corporate Highlights

- **Friedreich Ataxia (FA)**
  - Today, Design announced early pharmacokinetics (PK) data for DT-216P2 demonstrating favorable translation from NHPs to humans with both intravenous (IV) and subcutaneous (SC) administration and an improved product profile compared to the prior DT-216 formulation (DT-216P1).
    - Human plasma PK profiles of DT-216P2 were consistent with NHP data following both IV and SC single-dose administration.
    - DT-216P2 exhibited improved exposure and PK parameters compared to DT-216P1, including higher AUC and sustained plasma levels at comparable doses.
  - DT-216P2 has been generally well-tolerated, and based on clinical and non-clinical data, Design believes the injection site thrombophlebitis seen with DT-216P1 is no longer an issue limiting continued development of DT-216.
  - In June, Design announced that it had received a clinical hold notice from the U.S. Food and Drug Administration (FDA) regarding its Investigational New Drug (IND) application for DT-216P2. FDA's request pertains to the starting dose in the U.S., which the company plans to address with clinical data and, if needed, nonclinical data, in order to initiate studies for DT-216P2 in the U.S. Design continues to dose patients in its RESTORE-FA Phase 1/2 MAD trial of DT-216P2 outside the U.S.
- **Fuchs Endothelial Corneal Dystrophy (FECD):** Design has initiated a Phase 2 biomarker trial of DT-168 to evaluate safety, tolerability and corneal endothelium biomarkers in FECD patients who are scheduled for corneal transplant surgery.
- **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.

Second Quarter 2025 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $15.7 million for the quarter ended June 30, 2025.
- **G&A Expenses:** General and administrative (G&A) expenses were $5.8 million for the quarter ended June 30, 2025.
- **Net Loss:** Net loss was $19.1 million for the quarter ended June 30, 2025.
- **Cash Position:** Cash, cash equivalents and investment securities were $216.3 million as of June 30, 2025.

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; Design’s pipeline; Design’s plan to address the FDA clinical hold with clinical data and, if needed, nonclinical data, in order to initiate studies for DT-216P2 in the U.S.; Design’s belief that the injection site thrombophlebitis seen with DT-216P1 is no longer an issue limiting continued development of DT-216; Design's ability to advance the GeneTAC® platform 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,” “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 intend to provide the FDA to resolve the clinical hold may not be sufficient, which could further delay our ability to commence U.S. clinical trials of DT-216P2; 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, as filed with the SEC on May 7, 2025, and under the “Risk Factors” heading of Design’s Quarterly Report on Form 10-Q for the quarter ended June 30, 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 June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
	(unaudited)			
Operating expenses:				
Research and development	$ 15,738	$ 10,516	$ 31,115	$ 20,317
General and administrative	5,831	4,527	10,872	9,126
Total operating expenses	21,569	15,043	41,987	29,443
Loss from operations	(21,569)	(15,043)	(41,987)	(29,443)
Other income, net	2,486	3,250	5,189	6,545
Net loss	$ (19,083)	$ (11,793)	$ (36,798)	$ (22,898)
Net loss per share, basic and diluted	$ (0.34)	$ (0.21)	$ (0.65)	$ (0.41)
Weighted-average shares of common stock outstanding, basic and diluted	56,859,388	56,555,960	56,808,888	56,522,244

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	June 30, 2025	December 31, 2024
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 216,276	$ 245,477
Prepaid expenses and other current assets	3,518	2,563
Total current assets	219,794	248,040
Property and equipment, net	1,258	1,410
Right-of-use asset, related party	1,834	2,216
Other assets	—	427
Total assets	$ 222,886	$ 252,093
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,013	$ 2,186
Accrued expenses and other current liabilities	7,734	6,276
Total current liabilities	8,747	8,462
Operating lease liability, net, related party	1,102	1,534
Total liabilities	9,849	9,996
Total stockholders' equity	213,037	242,097
Total liabilities and stockholders' equity	$ 222,886	$ 252,093