Design Therapeutics Provides RESTORE-FA Clinical Development Update and Reports Second Quarter 2026 Financial Results

August 3, 2026

RESTORE-FA trial modifications build on positive four-week data; expect to share data based on 12 weeks of dosing in the first quarter of 2027

Patient dosing initiated in Phase 1 multiple-ascending dose trial of DT-818 in myotonic dystrophy type 1

Cash and securities of $207.4 million at quarter-end provide strong financial runway

CARLSBAD, Calif., Aug. 03, 2026 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today reported second quarter 2026 financial results and highlighted business updates and upcoming milestones across its GeneTAC® portfolio.

“Design continued its strong operational execution in the second quarter building on the positive RESTORE-FA data reported in May. Those four-week data demonstrated the ability of DT-216P2 to increase endogenous frataxin and its potential to deliver a differentiated, best-in-disease therapy for Friedreich ataxia,” said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. “Today's updates reflect continued progress across our pipeline, from the evolution of the DT-216P2 clinical program to the recent initiation of DT-818 dosing in DM1 patients, demonstrating the momentum of our GeneTAC ® platform across multiple serious genetic diseases.”

RESTORE-FA Progress

- **Positive RESTORE-FA Four-Week Data Support Advancement of DT-216P2.** As reported in May 2026, DT-216P2 was generally well-tolerated and demonstrated dose-dependent increases in endogenous frataxin mRNA and protein levels, together with improvements across multiple clinical measures following four weeks of intravenous dosing in patients with Friedreich ataxia.
- **Modifications to RESTORE-FA.** Based on the four-week data, Design is modifying the ongoing cohorts in the RESTORE-FA trial to support the next stage of clinical development. The study will continue to evaluate 1 mpk as the planned go-forward dose, with the intention of enrolling 10 patients in the 12-week cohort. In addition, modifications include specifying endogenous blood FXN protein percent change from baseline as the primary efficacy endpoint and exploring a dose level above 1 mpk.
- **Next Steps and Expected Milestones:** Design expects to provide an update on its registrational plans in the fourth quarter of 2026, with data following 12 weeks of treatment expected in the first quarter of 2027.

GeneTAC ® Pipeline Progress:

- **Myotonic Dystrophy Type-1 (DM1):** Design has initiated dosing patients with DM1 in its Phase 1 multiple-ascending dose (MAD) trial of DT-818, a GeneTAC® small molecule designed to selectively reduce transcription of the mutant DMPK allele. Design anticipates reporting data from this study in 2027.
- **Fuchs Endothelial Corneal Dystrophy (FECD):** A Phase 2 biomarker trial of DT-168 is ongoing to evaluate safety, tolerability and corneal endothelium biomarkers in FECD patients who are scheduled for corneal transplant surgery. Data is now expected in 2027 due to a delay in the anticipated supply of DT-168 blow-fill-seal eye droppers.
- **Huntington’s disease (HD):** Design continues to advance preclinical characterization of several candidate molecules for its Huntington’s disease program.

Second Quarter 2026 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $16.4 million for the quarter ended June 30, 2026.
- **G&A Expenses:** General and administrative (G&A) expenses were $5.8 million for the quarter ended June 30, 2026.
- **Net Loss:** Net loss was $20.2 million for the quarter ended June 30, 2026.
- **Cash Position:** Cash, cash equivalents and investment securities were $207.4 million as of June 30, 2026.

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, DT-168, for Fuchs endothelial corneal dystrophy, and DT-818, for myotonic dystrophy type-1, the company is advancing a program in 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, DT-168 and DT-818 and the timing thereof; the anticipated timing for data readouts and other program updates; planned modifications to the RESTORE-FA trial; the potential attributes and potential best-in-disease profile of DT-818; establishing clinical proof of concept for any product candidate; 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," "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 data we observe from early 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 and retention, which are affected by many factors, and any difficulties or delays encountered with such clinical trial or patient enrollment or retention 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 on the timeframe anticipated, or at all; whether promising early research or clinical trials will result in demonstrated safety and/or efficacy in later 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 third parties, including contract manufacturers and contract research organizations; interactions with regulatory authorities; 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; and Design's ability to recruit and retain key scientific or management personnel. 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, 2026, as filed with the SEC on April 28, 2026, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended June 30, 2026, 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.

Contact:
Renee Leck, THRUST
renee@thrustsc.com

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,	
	2026	2025	2026	2025
	(unaudited)		(unaudited)	
Operating expenses:				
Research and development	$ 16,388	$ 15,738	$ 30,767	$ 31,115
General and administrative	5,789	5,831	11,116	10,872
Total operating expenses	22,177	21,569	41,883	41,987
Loss from operations	(22,177)	(21,569)	(41,883)	(41,987)
Other income, net	2,012	2,486	4,082	5,189
Net loss	$ (20,165)	$ (19,083)	$ (37,801)	$ (36,798)
Net loss per share, basic and diluted	$ (0.32)	$ (0.34)	$ (0.61)	$ (0.65)
Weighted-average shares of common stock outstanding, basic and diluted	62,505,340	56,859,388	61,972,857	56,808,888

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

	June 30, 2026	December 31, 2025
	(unaudited)	

Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 207,396	$ 219,845
Prepaid expenses and other current assets	4,617	3,939
Total current assets	212,013	223,784
Property and equipment, net	694	981
Right-of-use asset	2,422	1,438
Total assets	$ 215,129	$ 226,203
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,141	$ 2,312
Accrued expenses and other current liabilities	8,099	10,743
Total current liabilities	11,240	13,055
Operating lease liability	2,031	645
Total liabilities	13,271	13,700
Total stockholders' equity	201,858	212,503
Total liabilities and stockholders' equity	$ 215,129	$ 226,203