Design Therapeutics Announces First Quarter 2026 Financial Results and Recent Business Updates

April 28, 2026

Additional Detail Provided for RESTORE-FA (DT-216P2) Trial Design, Dosing and Endpoints

David Shapiro, M.D., Appointed to Board of Directors, Strengthening Clinical and Regulatory Expertise

Cash and Securities of $222.8 Million at Quarter-End Provide Runway to Support Ongoing Clinical Execution

CARLSBAD, Calif., April 28, 2026 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today reported first quarter 2026 financial results and highlighted business updates and upcoming milestones across its GeneTAC® portfolio.

“The first quarter was marked by continued operational execution across our portfolio as we progress our clinical programs, including our ongoing RESTORE-FA multiple ascending dose trial evaluating DT-216P2,” said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. “DT-216P2 is designed to restore endogenous frataxin, with the potential to address the underlying cause of Friedreich ataxia and deliver a differentiated therapeutic approach. We believe our GeneTAC® platform represents a novel way to modulate gene expression, with the potential to unlock new therapeutic opportunities across a broad range of rare genetic diseases. We are also pleased to welcome David Shapiro, M.D., to our Board, where his experience will support the continued advancement of our clinical programs.”

Corporate Highlights

- **Friedreich Ataxia (FA):**
  - Design continues to dose FA patients in its RESTORE-FA trial, a Phase 1/2 multiple ascending dose study of DT-216P2 over four- or 12-week treatment periods to evaluate safety, pharmacokinetics and biomarker endpoints assessing changes in endogenous frataxin (FXN) mRNA and protein levels in whole blood and muscle biopsy samples. Exploratory clinical endpoints include the modified Friedreich Ataxia Rating Scale (mFARS), Upright Stability Score, and PROMIS Fatigue Scale.
  - Design anticipates providing an update on the effect of DT-216P2 on endogenous frataxin levels in the second half of 2026.
- **Pipeline:**
  - **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, with data anticipated in the second half of 2026.
  - **Myotonic Dystrophy Type-1 (DM1):** Design expects to begin dosing DM1 patients 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, in the first half of 2026. The study, with results anticipated in 2027, is expected to assess safety and correction of mis-splicing.
  - **Huntington’s disease (HD):** Design continues to advance preclinical characterization of several candidate molecules for its Huntington’s disease program.
- **Board of Directors:** In March 2026, Design appointed David Shapiro, M.D., to its board of directors. Dr. Shapiro has extensive biopharmaceutical experience, including serving as Chief Medical Officer and Head of R&D at Intercept Pharmaceuticals, where he advanced therapies through clinical development and regulatory approval, and as a member of multiple boards of directors.

First Quarter 2026 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $14.4 million for the quarter ended March 31, 2026.
- **G&A Expenses:** General and administrative (G&A) expenses were $5.3 million for the quarter ended March 31, 2026.
- **Net Loss:** Net loss was $17.6 million for the quarter ended March 31, 2026.
- **Cash Position and Operating Runway:** Cash, cash equivalents and investment securities were $222.8 million as of March 31, 2026, which the company expects to fund its planned operations 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, 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; 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; 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2025, as filed with the SEC on March 9, 2026, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended March 31, 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.

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DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2026	**2025**
	(unaudited)	
Operating expenses:		
Research and development	$ 14,379	$ 15,377
General and administrative	5,327	5,041
Total operating expenses	19,706	20,418
Loss from operations	(19,706)	(20,418)
Other income, net	2,070	2,703
Net loss	$ (17,636)	$ (17,715)
Net loss per share, basic and diluted	$ (0.29)	$ (0.31)
Weighted-average shares of common stock outstanding, basic and diluted	61,434,457	56,757,827

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	March 31, 2026	**December 31, 2025**

	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 222,823	$ 219,845
Prepaid expenses and other current assets	4,226	3,939
Total current assets	227,049	223,784
Property and equipment, net	824	981
Right-of-use asset	2,569	1,438
Total assets	$ 230,442	$ 226,203
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,276	$ 2,312
Accrued expenses and other current liabilities	7,914	10,743
Total current liabilities	10,190	13,055
Operating lease liability	2,198	645
Total liabilities	12,388	13,700
Total stockholders' equity	218,054	212,503
Total liabilities and stockholders' equity	$ 230,442	$ 226,203