Design Therapeutics Reports Fourth Quarter and Full Year 2025 Financial Results and Recent Business Updates

March 9, 2026

Trials for DT-216P2 (RESTORE-FA) and DT-168 (FECD) Ongoing; DT-818 (DM1) Dosing in Patients Expected in the First Half of 2026

Cash and Securities of $219.8 Million as of Year-End Supports Ongoing Clinical Execution

CARLSBAD, Calif., March 09, 2026 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today reported fourth quarter and full year 2025 financial results and highlighted business updates and upcoming milestones across its GeneTAC® portfolio.

"The fourth quarter capped a year of strong execution and meaningful progress across our GeneTAC ® portfolio," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. "We enter 2026 with three clinical programs advancing toward important milestones, including the planned initiation of patient dosing in our Phase 1 trial of DT-818 in DM1 in the first half of the year and anticipated data from DT-216P2 in FA and DT-168 in FECD in the second half of 2026. Together, these anticipated milestones position us for continued execution of our strategy to build a diversified set of clinical proof-of-concept opportunities for genetic diseases with significant unmet needs."

Corporate Highlights

- **Friedreich Ataxia (FA):**
  - Design continues to dose FA patients in its RESTORE-FA Phase 1/2 MAD trial and anticipates providing an update on the effect of DT-216P2 on endogenous frataxin levels following 12 weeks of dosing in the second half of 2026.
- **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.
  - In preclinical studies, DT-818 demonstrated a potential best-in-disease profile for investigational therapies targeting mutant DMPK.
- **Pipeline:**
  - Design continues to advance preclinical characterization of several candidate molecules for its Huntington's disease program.

Fourth Quarter and Full Year 2025 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $13.4 million for the quarter ended December 31, 2025, and $59.1 million for the year ended December 31, 2025.
- **G&A Expenses:** General and administrative (G&A) expenses were $4.7 million for the quarter ended December 31, 2025, and $20.3 million for the year ended December 31, 2025.
- **Net Loss:** Net loss was $16.0 million for the quarter ended December 31, 2025, and $69.8 million for the year ended December 31, 2025.
- **Cash Position and Operating Runway:** Cash, cash equivalents and investment securities were $219.8 million as of December 31, 2025, 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, including the potential to have multiple 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," "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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, as filed with the SEC on November 5, 2025, and under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 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.

Contact:
Renee Leck, THRUST
renee@thrustsc.com

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Quarter Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
	(unaudited)			
Operating expenses:				
Research and development	$ 13,425	$ 12,157	$ 59,129	$ 44,350
General and administrative	4,744	4,537	20,338	18,033
Total operating expenses	18,169	16,694	79,467	62,383
Loss from operations	(18,169)	(16,694)	(79,467)	(62,383)
Interest income	2,172	3,043	9,675	12,795
Net loss	$ (15,997)	$ (13,651)	$ (69,792)	$ (49,588)
Net loss per share, basic and diluted	$ (0.27)	$ (0.24)	$ (1.22)	$ (0.88)
Weighted-average shares of common stock outstanding, basic and diluted	58,734,995	56,681,940	57,330,192	56,587,142

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 219,845	$ 245,477
Prepaid expenses and other current assets	3,939	2,563

Total current assets	223,784	248,040
Property and equipment, net	981	1,410
Right-of-use asset, related party	1,438	2,216
Other assets	—	427
Total assets	$ 226,203	$ 252,093
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,312	$ 2,186
Accrued expenses and other current liabilities	10,743	6,276
Total current liabilities	13,055	8,462
Operating lease liability, net, related party	645	1,534
Total liabilities	13,700	9,996
Total stockholders' equity	212,503	242,097
Total liabilities and stockholders' equity	$ 226,203	$ 252,093