Design Therapeutics Announces Plans to Initiate Patient Dosing of DT-818 in Myotonic Dystrophy Type-1 (DM1) in the First Half of 2026 and Reports Third Quarter 2025 Financial Results

November 5, 2025

Obtained ex-US Regulatory Clearance for DT-818, a Potentially Best-in-Disease Treatment for Myotonic Dystrophy Type-1 (DM1)

Trials of DT-216P2 in Friedreich Ataxia (FA) and DT-168 in Fuchs Endothelial Corneal Dystrophy (FECD) Ongoing

Cash and Securities of $206.0 Million as of Third Quarter 2025 Support Continued Pipeline Advancement

CARLSBAD, Calif., Nov. 05, 2025 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today announced progress and updated milestones across its portfolio of GeneTAC® candidates in addition to reporting financial results for the third quarter of 2025.

“The third quarter was marked by strong operational execution across our portfolio,” said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. “Today we are excited to unveil DT-818 as our development candidate for the treatment of DM1, a debilitating neuromuscular disease with significant unmet medical need. With broad tissue distribution, significant splicing correction, and selectivity for mutant DMPK, we believe DT-818 has best-in-disease potential. Our DT-216P2 and DT-168 trials also continue to progress toward anticipated second half 2026 data readouts in FA and FECD. With these milestones, we are entering an exciting phase for Design as we advance multiple programs toward clinical proof-of-concept.”

Corporate Highlights

- **Myotonic Dystrophy Type-1 (DM1):**
  - Today, Design announced the nomination of DT-818, a GeneTAC® small molecule, as a development candidate for the treatment of DM1. The underlying cause of DM1 is a CTG repeat expansion in the DMPK gene, which DT-818 is designed to address by selectively reducing transcription of the mutant expanded allele.
  - In preclinical studies, DT-818 has demonstrated a potential best-in-disease profile for DM1, including a greater than 90% reduction in toxic RNA foci in DM1 patient cells, corresponding splicing correction and selective targeting of mutant DMPK.
  - The company has obtained ex-US regulatory clearance to initiate clinical development and plans to begin dosing DM1 patients in a Phase 1 multiple-ascending dose (MAD) trial of DT-818 in Australia in the first half of 2026 to assess safety and correction of mis-splicing, with splicing data expected in 2027.
- **Friedreich Ataxia (FA):** Design continues to dose FA patients in its RESTORE-FA Phase 1/2 MAD trial of DT-216P2 outside the U.S. and anticipates reporting data, including levels of frataxin (FXN) expression based on 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.
- **Pipeline programs:** Design continues to advance preclinical characterization of several candidate molecules for its Huntington’s disease program.
- **Board of Directors appointment:** In September 2025, Design appointed Justin Gover to its Board of Directors. Mr. Gover brings over 25 years of leadership experience in the biotechnology industry and served as the founding Chief Executive Officer of GW Pharmaceuticals plc, guiding the company for over two decades, from inception through its strategic $7 billion acquisition by Jazz Pharmaceuticals in 2021.

Third Quarter 2025 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $14.6 million for the quarter ended September 30, 2025.
- **G&A Expenses:** General and administrative (G&A) expenses were $4.7 million for the quarter ended September 30, 2025.
- **Net Loss:** Net loss was $17.0 million for the quarter ended September 30, 2025.
- **Cash Position:** Cash, cash equivalents and investment securities were $206.0 million as of September 30, 2025.

About Myotonic Dystrophy Type-1 (DM1) and DT-818

DM1 is a monogenic, autosomal dominant, progressive neuromuscular disease that affects skeletal muscle, heart, brain and other organs. The cardinal features include muscle weakness, myotonia (slow muscle relaxation) and early cataracts. In addition, affected individuals often experience cardiac arrhythmias and changes in neuropsychological function. DM1 is caused by a mutation in the DMPK gene and is estimated to affect more than 70,000 people in the United States. DT-818 is a GeneTAC® small molecule designed to address the genetic cause of DM1 by preventing the expression of mutant gene product and consequently of pathogenic nuclear foci.

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 DT-818 for myotonic dystrophy type-1, and 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 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 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 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 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; 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 June 30, 2025, as filed with the SEC on August 7, 2025, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended September 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(unaudited)		(unaudited)	
Operating expenses:				
Research and development	$ 14,589	$ 11,876	$ 45,704	$ 32,193
General and administrative	4,722	4,370	15,594	13,496
Total operating expenses	19,311	16,246	61,298	45,689
Loss from operations	(19,311)	(16,246)	(61,298)	(45,689)
Other income, net	2,314	3,207	7,503	9,752
Net loss	$ (16,997)	$ (13,039)	$ (53,795)	$ (35,937)
Net loss per share, basic and diluted	$ (0.30)	$ (0.23)	$ (0.95)	$ (0.64)
Weighted-average shares of common stock outstanding, basic and diluted	56,950,999	56,620,731	56,856,779	56,555,312

DESIGN THERAPEUTICS, INC.

CONDENSED BALANCE SHEETS

(in thousands)

	September 30, 2025	December 31, 2024
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 205,970	$ 245,477
Prepaid expenses and other current assets	3,059	2,563
Total current assets	209,029	248,040
Property and equipment, net	1,104	1,410
Right-of-use asset, related party	1,637	2,216
Other assets	-	427
Total assets	$ 211,770	$ 252,093
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,913	$ 2,186
Accrued expenses and other current liabilities	9,260	6,276
Total current liabilities	11,173	8,462
Operating lease liability, net, related party	877	1,534
Total liabilities	12,050	9,996
Total stockholders' equity	199,720	242,097
Total liabilities and stockholders' equity	$ 211,770	$ 252,093