Design Therapeutics Highlights Progress Across Lead GeneTAC® Programs and Reports Fourth Quarter and Full Year 2024 Financial Results

March 10, 2025

Initiated Phase 1 Single Ascending Dose Trial of DT-216P2 in Healthy Volunteers; Friedreich Ataxia (FA) Patient Dosing to Begin in mid-2025

Dosing Complete in DT-168 Phase 1 Healthy Volunteer Trial, with Data on Track for the First Half of 2025; Enrollment Target Achieved in Fuchs Endothelial Corneal Dystrophy (FECD) Observational Study

Selection of Development Candidate for Myotonic Dystrophy Type-1 (DM1) Expected in 2025

Well-Capitalized with Cash and Securities of $245.5 Million to Fund Operations Through Up to Four Potential Clinical Proof-of-Concept Data Sets

CARLSBAD, Calif., March 10, 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 fourth quarter and full year 2024 financial results.

“Thanks to the progress we have achieved so far this year, the first half of 2025 will be a busy one for Design, with data expected from our Phase 1 trial in FECD and the advancement of clinical activities in our FA program,” said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. “We believe these programs lead a pipeline of GeneTAC ® small molecules capable of transforming the status quo in genomic medicines, with the potential for multiple clinical proof-of-concept data sets over the next few years.”

Corporate Highlights and Anticipated Upcoming Milestones

- **Friedreich Ataxia (FA)** Design has initiated dosing in a Phase 1 clinical trial in healthy volunteers in Australia to evaluate the safety and pharmacokinetics (PK) of single ascending doses of DT-216P2 via multiple routes of administration (intravenous infusion, subcutaneous infusion and subcutaneous injection). A Phase 1/2 multiple ascending dose (MAD) clinical trial to assess safety, PK and pharmacodynamics (PD) in FA patients is anticipated to begin in mid-2025. Data based on twelve weeks of DT-216P2 dosing in patients is anticipated in 2026.
- **Fuchs Endothelial Corneal Dystrophy (FECD)** Design has completed dosing in a Phase 1 MAD clinical trial of DT-168 in healthy volunteers and expects to report data in the first half of 2025. The company achieved its enrollment goal for the observational study by recruiting and completing baseline assessments on approximately 250 FECD patients. Based on the baseline characteristics data, Design has chosen approximately 100 patients for future follow-up visits.
- **Pipeline programs** Design continues to advance preclinical work toward the selection of a development candidate for myotonic dystrophy type-1 (DM1) later in 2025. Preclinical characterization of several candidate molecules also continues in Huntington’s disease (HD).

Fourth Quarter and Full Year 2024 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $12.2 million for the quarter ended December 31, 2024, and $44.4 million for the year ended December 31, 2024.
- **G&A Expenses:** General and administrative (G&A) expenses were $4.5 million for the quarter ended December 31, 2024, and $18.0 million for the year ended December 31, 2024.
- **Net Loss:** Net loss was $13.7 million for the quarter ended December 31, 2024, and $49.6 million for the year ended December 31, 2024.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were $245.5 million as of December 31, 2024, which the company expects to fund its planned operating expenses 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, 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; the expected timing for data readouts; Design's pipeline, including the potential to have four 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," "on track to," "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; 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 September 30, 2024, as filed with the SEC on November 7, 2024, and under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 31, 2024, 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)

	Quarter Ended December 31,		Year Ended December 31,	
	2024	2023	2024	2023
	(unaudited)			
Operating expenses:				
Research and development	$ 12,157	$ 11,012	$ 44,350	$ 57,063
General and administrative	4,537	4,109	18,033	21,127
Total operating expenses	16,694	15,121	62,383	78,190
Loss from operations	(16,694)	(15,121)	(62,383)	(78,190)
Interest income	3,043	3,279	12,795	11,328
Net loss	$ (13,651)	$ (11,842)	$ (49,588)	$ (66,862)
Net loss per share, basic and diluted	$ (0.24)	$ (0.21)	$ (0.88)	$ (1.19)
Weighted-average shares of common stock outstanding, basic and diluted	56,681,940	56,090,912	56,587,142	55,984,670

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	December 31,	December 31,

	2024	2023
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 245,477	$ 281,798
Prepaid expenses and other current assets	2,563	2,786
Total current assets	248,040	284,584
Property and equipment, net	1,410	1,691
Right-of-use asset, related party	2,216	2,938
Other assets	427	430
Total assets	$ 252,093	$ 289,643
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,186	$ 1,940
Accrued expenses and other current liabilities	6,276	7,682
Total current liabilities	8,462	9,622
Operating lease liability, net, related party	1,534	2,334
Total liabilities	9,996	11,956
Total stockholders' equity	242,097	277,687
Total liabilities and stockholders' equity	$ 252,093	$ 289,643