Design Therapeutics Announces Third Quarter 2024 Financial Results and Reviews Near-term Milestones for GeneTACTM Portfolio

November 7, 2024

Fuchs Endothelial Corneal Dystrophy (FECD) Phase 1 Trial Initiated with Data Expected in the First Half of 2025

Friedreich Ataxia (FA) Program on Track to Initiate Phase 1 Single Ascending Dose, Healthy Volunteer Study in the First Half of 2025

Active Research Pipeline Advancing with Programs in Myotonic Dystrophy Type-1 (DM1) and Huntington's Disease (HD)

Well-Capitalized with Cash and Securities of $254.1 Million to Fund Operations Through Up to Four Potential Clinical Proof-of-Concept Data Sets

CARLSBAD, Calif., Nov. 07, 2024 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today announced its third quarter 2024 financial results and reviewed anticipated upcoming program milestones for its portfolio of GeneTAC™ candidates.

"Thanks to our progress so far this year, the first half of 2025 will be a busy one, with data expected from our ongoing Phase 1 trial in FECD and the start of clinical activities for 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 is on track to initiate the Phase 1 single ascending dose, normal healthy volunteer trial for DT-216P2 in the first half of 2025. The company anticipates beginning FA patient dosing later in 2025.
- **Fuchs Endothelial Corneal Dystrophy (FECD)** The company has initiated Phase 1 development for DT-168 in normal healthy volunteers, with initial data expected in the first half of 2025. In parallel, Design continues its ongoing FECD observational study.
- **Pipeline programs** Design also continues to advance preclinical characterization of several lead molecules toward the selection of development candidates for Huntington's disease (HD) and myotonic dystrophy type-1 (DM1) in anticipation of future IND submissions.

Third Quarter 2024 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $11.9 million for the quarter ended September 30, 2024.
- **G&A Expenses:** General and administrative (G&A) expenses were $4.4 million for the quarter ended September 30, 2024.
- **Net Loss:** Net loss was $13.0 million for the quarter ended September 30, 2024.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were $254.1 million as of September 30, 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 lead GeneTAC™ small molecule, DT-216, in development for patients with Friedreich ataxia, the company is advancing programs in Fuchs endothelial corneal dystrophy, Huntington's disease and myotonic dystrophy type-1. 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 June 30, 2024, as filed with the SEC on August 5, 2024, and under the “Risk Factors” heading of Design’s Quarterly Report on Form 10-Q for the quarter ended September 30, 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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
	(unaudited)			
Operating expenses:				
Research and development	$ 11,876	$ 13,257	$ 32,193	$ 46,051
General and administrative	4,370	5,565	13,496	17,018
Total operating expenses	16,246	18,822	45,689	63,069
Loss from operations	(16,246)	(18,822)	(45,689)	(63,069)
Other income, net	3,207	3,033	9,752	8,049
Net loss	$ (13,039)	$ (15,789)	$ (35,937)	$ (55,020)
Net loss per share, basic and diluted	$ (0.23)	$ (0.28)	$ (0.64)	$ (0.98)
Weighted-average shares of common stock outstanding, basic and diluted	56,620,731	55,988,691	56,555,312	55,948,867

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	September 30, 2024	December 31, 2023
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 254,074	$ 281,798
Prepaid expenses and other current assets	3,168	2,786
Total current assets	257,242	284,584

Property and equipment, net	1,559	1,691
Right-of-use asset, related party	2,401	2,938
Other assets	427	430
Total assets	$ 261,629	$ 289,643
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,470	$ 1,940
Accrued expenses and other current liabilities	5,962	7,682
Total current liabilities	7,432	9,622
Operating lease liability, net, related party	1,744	2,334
Total liabilities	9,176	11,956
Total stockholders' equity	252,453	277,687
Total liabilities and stockholders' equity	$ 261,629	$ 289,643