



Design Therapeutics Announces First Quarter 2024 Financial Results and Highlights Upcoming Program Milestones

May 8, 2024

Advancing DT-216P2, New Drug Product for Friedreich Ataxia (FA), Toward Clinical Trials

Starting Phase 1 Development for Fuchs Endothelial Corneal Dystrophy (FECD) in 2024; Observational Study Currently Enrolling Patients

Progressing GeneTAC™ Pipeline Programs in Huntington's Disease (HD) and Myotonic Dystrophy Type-1 (DM1) to Development Candidates

Cash and Securities of \$270.7 Million Support Multi-Year Operating Runway and Advancement of Up to Four Programs to Clinical Proof-of-Concept

CARLSBAD, Calif., May 08, 2024 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for serious degenerative genetic diseases, today announced first quarter 2024 financial results along with recent business highlights and upcoming milestones.

"At Design, we began 2024 with a clear vision to advance our portfolio of first- or best-in-class therapies to treat major genetic disorders, using novel small molecules that work with a patient's natural genome," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. Dr. Shah continued, "Our work in FA is differentiated by the fact that we have been able to increase levels of endogenous frataxin and we look forward to beginning patient trials with our new drug product, DT-216P2, next year. We have also made solid progress enrolling patients in our FECD observational study and remain on track to initiate Phase 1 development for DT-168 later this year. DT-168 has the potential to be the first effective treatment addressing the root cause of this degenerative corneal disease. Behind these clinical-stage programs, we have an exciting pipeline in HD and DM1, resulting in four programs with the potential to deliver clinical proof-of-concept, depending on R&D results, over the next several years under our current cash runway. We believe success in any one of these programs has the potential to generate enormous value for patients and shareholders."

Business Highlights and Anticipated Upcoming Milestones

- **Friedreich Ataxia (FA)** Design's new drug product for FA, DT-216P2, demonstrates an improved pharmacokinetic (PK) profile, injection site profile and sustained drug exposure in nonclinical studies compared to the prior formulation. Design is on track to complete GLP studies for DT-216P2 by year-end 2024 to start patient trials in 2025.
- **Fuchs Endothelial Corneal Dystrophy (FECD)** Design is conducting an observational study designed to confirm FECD disease characteristics and evaluate potential endpoints and progression prior to initiating an interventional treatment trial. The observational study is expected to enroll 200 patients with a planned follow-up of two years. Design expects to initiate Phase 1 development for DT-168 in 2024.
- **Pipeline programs** Design is advancing 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. In preclinical studies for HD, GeneTAC™ candidate molecules selectively dialed-down the expression of the mutant HTT gene by over 50% in the brain striatum with systemic administration. In the DM1 program, GeneTAC™ small molecules potently dialed-down the expression of the mutant DMPK gene in DM1 patient cells, eliminating foci and restoring normal splicing.

First Quarter 2024 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were \$9.8 million for the quarter ended March 31, 2024.
- **G&A Expenses:** General and administrative (G&A) expenses were \$4.6 million for the quarter ended March 31, 2024.
- **Net Loss:** Net loss was \$11.1 million for the quarter ended March 31, 2024.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were \$270.7 million as of March 31, 2024, which the company expects to fund its planned operating expenses into 2029.

About Design Therapeutics

Design Therapeutics is a 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; 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; the potential of each of Design's programs to generate enormous value for patients and shareholders; Design's estimated financial 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," "aims," "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 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; performing clinical trials, regulatory filings and applications; reliance on third parties to successfully conduct clinical trials and nonclinical studies; competitive products, which may make any products we 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; competition in the industry in which Design operates, which may result in others discovering, developing or commercializing competitive products before or more successfully than Design; 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2023, as filed with the SEC on March 19, 2024, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended March 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)

	Three Months Ended March 31,	
	2024	2023
	(unaudited)	
Operating expenses:		
Research and development	\$ 9,801	\$ 15,730
General and administrative	4,599	5,921
Total operating expenses	14,400	21,651
Loss from operations	(14,400)	(21,651)
Other income, net	3,295	2,357
Net loss	\$ (11,105)	\$ (19,294)
Net loss per share, basic and diluted	\$ (0.20)	\$ (0.35)
Weighted-average shares of common stock outstanding, basic and diluted	56,488,527	55,908,033

**DESIGN THERAPEUTICS, INC.
 CONDENSED BALANCE SHEETS**

(in thousands)

	March 31,	December 31,
	2024	2023
	(unaudited)	

Assets

Current assets:		
Cash, cash equivalents and investment securities	\$ 270,686	\$ 281,798
Prepaid expense and other current assets	3,200	2,786
Total current assets	<u>273,886</u>	<u>284,584</u>
Property and equipment, net	1,718	1,691
Right-of-use asset, related party	2,762	2,938
Other assets	427	430
Total assets	<u>\$ 278,793</u>	<u>\$ 289,643</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,372	\$ 1,940
Accrued expenses and other current liabilities	5,722	7,682
Total current liabilities	<u>7,094</u>	<u>9,622</u>
Operating lease liability, net, related party	2,142	2,334
Total liabilities	<u>9,236</u>	<u>11,956</u>
Total stockholders' equity	<u>269,557</u>	<u>277,687</u>
Total liabilities and stockholders' equity	<u>\$ 278,793</u>	<u>\$ 289,643</u>