



Design Therapeutics Outlines Progress Across GeneTAC™ Platform and Announces Fourth Quarter and Full Year 2023 Financial Results

March 19, 2024

New Drug Product for Friedreich Ataxia (FA) DT-216P2 with Favorable Nonclinical Pharmacokinetic and Injection Site Safety Profile; Complete GLP Studies by Year-end 2024 to Start Patient Trials in 2025

IND Cleared for DT-168 for Treatment of Fuchs Endothelial Corneal Dystrophy (FECD) with Phase 1 Development to Start in 2024; Observational Study Underway to Confirm Patient Characteristics and Evaluate Potential Endpoints

New Program Unveiled for Huntington's Disease (HD) Targeting Reduction of Mutant Huntingtin with a GeneTAC™ Small Molecule

Cash and Securities of \$281.8 Million at Year-end 2023 Support Five-Year Operating Runway and Advancement of Up to Four Programs to Clinical Proof-of-Concept

Conference Call and Webcast to be Held Today at 4:30pm ET

CARLSBAD, Calif., March 19, 2024 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for serious degenerative genetic diseases, today provided an update on the progress of its platform of GeneTAC™ portfolio assets and reported fourth quarter and full year 2023 financial results.

"At Design, our vision is to develop a portfolio of first or best-in-class small molecules capable of treating a host of degenerative diseases by working with a patient's natural genome to restore cellular health. In recent months we have undertaken important work to evaluate the scope of our GeneTAC™ platform, which we believe has the potential to deliver clinical proof-of-concept in up to four programs, subject to research and development results, under our current cash runway through the next five years," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics.

Dr. Shah continued, "Our lead program in FA has a new drug product, DT-216P2, that we have designed to have an improved pharmacokinetic and injection site safety profile, which positions us to resume clinical development for this debilitating, degenerative, neuro-muscular genetic disease. This work builds on encouraging data in FA patients from our previous clinical trial. In addition, our FECD program, DT-168, which has the potential to be the first effective treatment addressing the root cause of this degenerative corneal disease, now has an IND that was cleared by the FDA. Finally, we are highlighting an exciting new program for Huntington's Disease, an indication of tremendous unmet medical need, which further demonstrates the depth and versatility of our differentiated approach to genomic medicines."

Pipeline Updates and Anticipated Upcoming Milestones

- **Friedreich Ataxia (FA)** Design has developed a new drug product, DT-216P2, for patients with FA that demonstrates an improved pharmacokinetic (PK) profile and a favorable injection site safety profile in nonclinical studies. When compared to the prior formulation, DT-216P2 demonstrated greater than 10-fold exposures that are more sustained over time. DT-216P2 also appears suitable for intravenous or subcutaneous routes of administration.

Design previously reported Phase 1 data using its prior formulation that showed increased levels of frataxin (FXN) mRNA in peripheral blood cells and skeletal muscle, confirming activity in patients with FA. Based on these findings, Design is advancing DT-216P2 for FA, with plans to complete GLP studies by year-end 2024 to start patient trials in 2025.

- **Fuchs Endothelial Corneal Dystrophy (FECD)** The U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for DT-168 in FECD. To evaluate the potential impact of DT-168 on this degenerative corneal disease, the company is conducting an observational study designed to confirm disease characteristics and progression and evaluate potential endpoints prior to initiating an interventional treatment trial. The observational study is expected to enroll 200 patients with a follow-up of two years. Design expects to initiate Phase 1 development for DT-168 in 2024.
- **Huntington's Disease (HD)** Design announced a new program in HD that targets allele-selective reduction of mutant huntingtin (HTT) gene expression with a GeneTAC™ small molecule. In preclinical studies, the company's HD GeneTAC™ candidate molecules selectively dial-down the expression of the mutant HTT gene by over 50% in the brain striatum with systemic administration. The company is working toward selection of a development candidate in anticipation of a future IND submission.
- **Myotonic Dystrophy Type-1 (DM1)** Design is also advancing its preclinical characterization of several lead molecules for the treatment of DM1, a multi-system genetic disorder. The company's DM1 GeneTAC™ small molecules potently

dial-down the expression of the mutant DMPK gene in DM1 patient cells, eliminating foci and restoring normal splicing. Design is working toward selection of a development candidate in anticipation of a future IND submission.

Fourth Quarter and Full Year 2023 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were \$11.0 million for the quarter ended December 31, 2023, and \$57.1 million for the year ended December 31, 2023.
- **G&A Expenses:** General and administrative (G&A) expenses were \$4.1 million for the quarter ended December 31, 2023, and \$21.1 million for the year ended December 31, 2023.
- **Net Loss:** Net loss was \$11.8 million for the quarter ended December 31, 2023, and \$66.9 million for the year ended December 31, 2023.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were \$281.8 million as of December 31, 2023. Design expects its cash, cash equivalents and marketable securities as of December 31, 2023, to fund its planned operating expenses through the next five years.

Webcast and Conference Call Information

Design will host a live webcast and conference call today at 4:30 pm ET to discuss these updates. The event is accessible through the Events section of the Investors page of www.designtx.com. A replay of the webcast will be archived on the Design website for 30 days.

Dial-in information for conference participants may be obtained by [registering for the event here](#).

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 expectation that DT-216P2 will have an improved PK and safety profile; the progression or completion of certain development activities, including the selection of development candidates; the initiation of studies and clinical trials for DT-216P2 and DT-168 and the timing thereof; Design's ability to advance the GeneTAC™ platform; 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 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; risks associated with nonclinical development activities; risks associated with conducting a clinical trial and patient enrollment, which is 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; the risk that undesirable side effects or other properties could cause Design or regulatory authorities to suspend or discontinue clinical trials which could 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; the risk that promising early research or clinical trials do not demonstrate safety and/or efficacy in later nonclinical studies or clinical trials; changes in Design's plans to develop its product candidates; uncertainties associated with performing clinical trials, regulatory filings and applications; risks associated with reliance on third parties to successfully conduct clinical trials and nonclinical studies; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, as filed with the SEC on November 13, 2023, and under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, 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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CONDENSED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Quarter Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
	(unaudited)			
Operating expenses:				
Research and development	\$ 11,012	\$ 14,255	\$ 57,063	\$ 48,613
General and administrative	4,109	5,137	21,127	18,980
Total operating expenses	<u>15,121</u>	<u>19,392</u>	<u>78,190</u>	<u>67,593</u>
Loss from operations	(15,121)	(19,392)	(78,190)	(67,593)
Other income, net	3,279	2,052	11,328	4,285
Net loss	<u>\$ (11,842)</u>	<u>\$ (17,340)</u>	<u>\$ (66,862)</u>	<u>\$ (63,308)</u>
Net loss per share, basic and diluted	<u>\$ (0.21)</u>	<u>\$ (0.31)</u>	<u>\$ (1.19)</u>	<u>\$ (1.14)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>56,090,912</u>	<u>55,864,872</u>	<u>55,984,670</u>	<u>55,707,517</u>

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

(in thousands)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash, cash equivalents and investment securities	\$ 281,798	\$ 330,387
Prepaid expense and other current assets	2,786	4,732
Total current assets	<u>284,584</u>	<u>335,119</u>
Property and equipment, net	1,691	1,947
Right-of-use asset, related party	2,938	3,612
Other assets	430	459
Total assets	<u>\$ 289,643</u>	<u>\$ 341,137</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,940	\$ 3,025
Accrued expenses and other current liabilities	7,682	7,751
Total current liabilities	<u>9,622</u>	<u>10,776</u>
Operating lease liability, net, related party	2,334	3,051
Total liabilities	<u>11,956</u>	<u>13,827</u>
Total stockholders' equity	<u>277,687</u>	<u>327,310</u>
Total liabilities and stockholders' equity	<u>\$ 289,643</u>	<u>\$ 341,137</u>