



Design Therapeutics Provides Pipeline Updates and Reports First Quarter 2023 Financial Results

May 9, 2023

Initial Data from Phase 1 Multiple-Ascending Dose Trial of DT-216 for Friedreich Ataxia Expected in the Third Quarter of 2023

Progress Across GeneTAC™ Small Molecule Pipeline with IND Submissions for FECD and DM1 Programs On-track for the Second Half of 2023 and 2024, Respectively

\$315.4 Million in Cash and Securities Expected to Support Operating Runway through 2025

CARLSBAD, Calif., May 09, 2023 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today provided updates and anticipated upcoming milestones across its clinical and research-stage pipeline of novel GeneTAC™ small molecules and reported first quarter 2023 financial results.

"2023 is poised to be an important year for Design as we work to bring our novel GeneTAC™ small molecules closer to patients suffering from devastating genetic diseases," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "We have meaningfully progressed DT-216 development and expect to report initial data from the Phase 1 multiple-ascending dose (MAD) trial in the third quarter of this year. As part of this update, we plan to summarize the effects of three weekly doses of DT-216 in people with Friedreich ataxia (FA), including clinical safety and levels of DT-216 in muscle, which will be important as we plan for Phase 2 development. In parallel, we are characterizing and validating FA biomarker assays in blood and skeletal muscle samples from individuals with FA, FA carriers and healthy controls, to determine if they can be used to reliably assess treatment response. Separately, we are preparing to submit a second Investigational New Drug application (IND) in the second half of this year to support development of our novel DT-168 eye drop for patients with Fuchs endothelial corneal dystrophy (FECD) and are further advancing our myotonic dystrophy type-1 (DM1) and research programs, all of which underscore the immense potential of our platform and approach. This is an exciting time for our company, and with a sharp focus on execution of near-term catalysts and a strong balance sheet, I am confident in our ability to deliver on the promise of our potentially transformational therapeutics."

Pipeline Updates and Anticipated Upcoming Milestones

- **Initial Data from Ongoing Phase 1 MAD Trial of DT-216 for FA Expected in the Third Quarter of 2023:** Design is evaluating its lead GeneTAC™ small molecule, DT-216, in an ongoing Phase 1 MAD clinical trial designed to evaluate the safety, tolerability, pharmacokinetic, biodistribution, and pharmacodynamic effects of three weekly doses of DT-216 in adults with FA. FA is a multisystem degenerative disease caused by a GAA nucleotide repeat expansion in the frataxin (FXN) gene that impairs transcription and reduces FXN mRNA. DT-216 is designed to specifically target the GAA repeat expansion mutation and restore FXN gene expression. An unanticipated vendor issue related to the study drug vial stopper caused a short delay in product supply, which has been resolved. Design now anticipates presenting initial results from the MAD trial in the third quarter of 2023. The company plans to initiate a Phase 2 trial in the second half of 2023.
- **IND Submission On-track for DT-168 for FECD in the Second Half of 2023:** Design is progressing its second GeneTAC™ development candidate, DT-168, an eye drop treatment for FECD, toward an IND in the second half of 2023. FECD is a genetic eye disease caused by a CTG repeat expansion in approximately 75% of cases and is characterized by progressive degeneration of the corneal endothelium and subsequent loss of vision that affects millions of people. There is currently no effective therapeutic intervention that addresses the root causes of the disease.

Design recently [presented preclinical data](#) at the Association for Research in Vision and Ophthalmology 2023 Annual Meeting (ARVO 2023), which showed that DT-168 reduced foci in patient-derived primary corneal endothelial cells (CECs) to levels seen in cells from healthy individuals with low nanomolar IC50 values. Treatment with DT-168 also significantly improved mis-splicing in patient-derived CECs across a panel of genes. Additionally, in animal studies DT-168 eye drops were well-tolerated after multiple doses and distributed throughout the cornea with micromolar levels of DT-168 observed in the cornea 24 hours after dosing.

- **IND Submission On-track for DM1 Program in the Second Half of 2024:** Design is advancing its preclinical characterization of several lead GeneTAC™ molecules for the treatment of DM1. DM1 is a multi-system genetic disorder caused by a nucleotide repeat expansion in the DMPK gene that leads to progressive muscle weakness, heart disease, and gastrointestinal and endocrine dysfunctions. Progressive weakness ultimately impairs the ability to breathe independently. There are currently no approved treatment options for patients with DM1. Design's DM1 GeneTAC™ small molecules potently and selectively block expression of the mutant DMPK gene in DM1 patient cells. The company is working toward selection of its development candidate and anticipates submitting an IND in the second half of 2024.

First Quarter 2023 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were \$15.7 million for the quarter ended March 31, 2023.
- **G&A Expenses:** General and administrative (G&A) expenses were \$5.9 million for the quarter ended March 31, 2023.
- **Net Loss:** Net loss was \$19.3 million for the quarter ended March 31, 2023.
- **Cash Position and Operating Runway:** Cash, cash equivalents and marketable securities were \$315.4 million as of March 31, 2023, which the company expects is sufficient to fund its current operating plan through 2025.

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. Design is currently evaluating its lead GeneTAC™ small molecule, DT-216, in an ongoing Phase 1 clinical trial in patients with Friedreich ataxia. The company is also advancing programs in Fuchs endothelial corneal dystrophy and myotonic dystrophy type-1. Discovery efforts for multiple other serious degenerative disorders caused by nucleotide repeat expansions are also underway, including for fragile X syndrome, spinocerebellar ataxias, Huntington disease, spinobulbar muscular atrophy, and C9orf72-amyotrophic lateral sclerosis/frontotemporal dementia. 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, preclinical data and early-stage clinical data; the potential benefits of restoring FXN in FA patients; expectations for reporting data for the MAD Phase 1 clinical trial and the timing thereof; the expected initiation of Design's Phase 2 clinical trial for DT-216 in patients with FA and the timing thereof; Design's ability to meet its stated milestones, near-term catalysts and advance the GeneTAC™ platform; the potential of Design's platform and approach; Design's ability to deliver on the promise of our potentially transformational therapeutics; Design's estimated financial runway and the sufficiency of its resources to support its planned operations; Design's anticipated timeline to submit an IND for DT-168 in the second half of 2023; Design's anticipated timeline to select a development candidate and submit an IND for its GeneTAC™ program for the treatment of DM1 in the second half of 2024; the potential of Design's GeneTAC™ small molecules to be a new class of therapies for patients suffering from devastating genetic diseases; 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,” “on-track 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 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 ongoing Phase 1 clinical trials for DT-216; the process of discovering and developing therapies that are safe and effective for use as human therapeutics and operating as a development stage company; Design's ability to develop, initiate or complete preclinical 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 preclinical 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 preclinical studies; 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 reliance on key third parties, including contract manufacturers and contract research organizations; 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, 2022, as filed with the SEC on March 14, 2023, and under the “Risk Factors” heading of Design's Quarterly Report on Form 10-Q for the quarter ended March 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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DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS
(in thousands)

March 31,

December 31,

	2023	2022
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	\$ 315,392	\$ 330,387
Prepaid expense and other current assets	3,547	4,732
Total current assets	318,939	335,119
Property and equipment, net	1,905	1,947
Right-of-use asset, related party	3,447	3,612
Other assets	452	459
Total assets	<u>\$ 324,743</u>	<u>\$ 341,137</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 2,744	\$ 3,025
Accrued expenses and other current liabilities	6,303	7,751
Total current liabilities	9,047	10,776
Operating lease liability, net, related party	2,878	3,051
Total liabilities	11,925	13,827
Total stockholders' equity	312,818	327,310
Total liabilities and stockholders' equity	<u>\$ 324,743</u>	<u>\$ 341,137</u>

DESIGN THERAPEUTICS, INC.
CONDENSED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2023	2022
	(unaudited)	
Operating expenses:		
Research and development	\$ 15,730	\$ 8,759
General and administrative	5,921	4,611
Total operating expenses	21,651	13,370
Loss from operations	(21,651)	(13,370)
Other income, net	2,357	105
Net loss	<u>\$ (19,294)</u>	<u>\$ (13,265)</u>
Net loss per share, basic and diluted	<u>\$ (0.35)</u>	<u>\$ (0.24)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>55,908,033</u>	<u>55,507,338</u>