Design Therapeutics Provides Pipeline Update and Reports Second Quarter 2023 Financial Results

August 14, 2023

Initial Data from Phase 1 Multiple-Ascending Dose Trial of DT-216 for Friedreich Ataxia Showed Significant Increase in FXN mRNA Levels in Skeletal Muscle

Company Expects to Resume a Multiple Dose Phase 1 Clinical Study with DT-216 with an Improved Formulation in the Second Half of 2024 and Report Data in the First Half of 2025

IND Submission for FECD GeneTAC™ Program On-track for the Second Half of 2023

$303.1 Million in Cash and Securities Expected to Support Operating Runway through 2026

CARLSBAD, Calif., Aug. 14, 2023 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today provided business updates and anticipated upcoming milestones across its clinical and research-stage pipeline of novel GeneTAC™ small molecules and reported second quarter 2023 financial results.

“Throughout the first half of 2023, our team has been focused on executing our programs to bring our novel GeneTAC™ small molecules to patients who need them,” said João Siffert, M.D., president and chief executive officer of Design Therapeutics. “Today, we presented initial results from our Phase 1 multiple-ascending dose trial of DT-216 for the treatment of Friedreich ataxia, which show proof-of-concept for clinical activity and are in-line with our founding hypothesis that GeneTAC™ molecules are capable of restoring FXN transcription in patients with FA. Additionally, we are preparing to submit an IND later this year to enable clinical development of our novel DT-168 eye drop for patients with FECD, a degenerative genetic eye disease that affects millions of people. With an expert team and strong financial position afforded by efficient cost and program management, I am confident in our ability to execute our goals. We look forward to providing an update on our progress across our GeneTAC™ pipeline in the months to come.”

Pipeline Updates and Anticipated Upcoming Milestones

- **Reported Initial Data from Ongoing Phase 1 MAD Trial Demonstrating Proof-of-Concept for DT-216 for FA:** Design reported initial results, based upon a data cutoff date of August 7, 2023, from the company’s Phase 1 multiple-ascending dose (MAD) clinical trial of its lead GeneTAC™ small molecule, DT-216, in patients with FA. Exploratory analyses demonstrated that DT-216 treatment achieved a significant and dose-related increase in frataxin (FXN) mRNA levels in skeletal muscle ($p < 0.05$), confirming clinical activity in patients with FA in an affected tissue. DT-216 was generally well-tolerated. Injection site reactions associated with the formulation excipients, were observed across dose cohorts.

  The Phase 1 findings support the continued development of DT-216 and Design plans to explore the full potential of FXN restoration with an improved formulation containing excipients with improved injection site tolerability. The company is now conducting bridging nonclinical studies to resume clinical development and expects to begin a multiple-dose Phase 1 clinical trial in the second half of 2024 with initial data expected in the first half of 2025.

- **Investigational New Drug (IND) Submission On-track for DT-168 for Fuchs Endothelial Corneal Dystrophy (FECD) in the Second Half of 2023:** Design is progressing its second GeneTAC™ candidate DT-168 as an eye drop treatment for patients suffering from FECD, which is caused by a CTG repeat expansion in approximately 75% of cases. FECD 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. The company plans to submit an IND for DT-168 in the second half of 2023.

- **IND Submission for Myotonic Dystrophy Type-1 (DM1) Program On-track for the Second Half of 2024:** Design is advancing its preclinical characterization of several lead GeneTAC™ molecules for the treatment of DM1—a multi-system genetic disorder. 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.

Second Quarter 2023 Financial Results

- **R&D Expenses**: Research and development (R&D) expenses were $17.1 million for the quarter ended June 30, 2023.
- **G&A Expenses**: General and administrative (G&A) expenses were $5.5 million for the quarter ended June 30, 2023.
- **Net Loss:** Net loss was $19.9 million for the quarter ended June 30, 2023.

- **Cash Position and Operating Runway**: Cash, cash equivalents and marketable securities were $303.1 million as of June 30, 2023. As a result of a strategic review of program spend and clinical prioritization, Design now expects its cash, cash equivalents and marketable securities as of June 30, 2023 to fund its planned operating expenses through 2026.

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 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, nonclinical data and early-stage clinical data; the potential benefits of restoring FXN in FA patients; expectations for resuming clinical development in FA and announcing date therefrom and the timing thereof; Design's ability to improve the formulation of DT-216 to prevent injection site thrombophlebitis or other limiting side effects; Design's ability to meet its stated milestones, near-term catalysts and advance the GeneTAC ^{TM} platform; the potential of Design's platform and approach; 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," "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 designing and implementing investigational drug product formulation improvements; 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 Phase 1 clinical development of 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; 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 March 31, 2023, as filed with the SEC on May 9, 2023, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended June 30, 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.
STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022

Operating expenses:				
Research and development	17,064	11,295	32,794	20,054
General and administrative	5,532	4,344	11,453	8,955
Total operating expenses	22,596	15,639	44,247	29,009
Loss from operations	(22,596)	(15,639)	(44,247)	(29,009)
Other income, net	2,659	640	5,016	745
Net loss	$ (19,937)	$ (14,999)	$ (39,231)	$ (28,264)
Net loss per share, basic and diluted	$ (0.36)	$ (0.27)	$ (0.70)	$ (0.51)
Weighted-average shares of common stock outstanding, basic and diluted	55,948,990	55,670,780	55,928,625	55,589,510

DESIGN THERAPEUTICS, INC.
BALANCE SHEETS
(in thousands)

	June 30, 2023	December 31, 2022
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 303,088	$ 330,387
Prepaid expense and other current assets	2,957	4,732
Total current assets	306,045	335,119
Property and equipment, net	1,861	1,947
Right-of-use asset, related party	3,280	3,612
Other assets	444	459
Total assets	$ 311,630	$ 341,137
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,922	$ 3,025
Accrued expenses and other current liabilities	10,211	7,751
Total current liabilities	12,133	10,776
Operating lease liability, net, related party	2,702	3,051
Total liabilities	14,835	13,827
Total stockholders' equity	296,795	327,310
Total liabilities and stockholders' equity	$ 311,630	$ 341,137