



## Design Therapeutics Highlights Pipeline Progress and Reports Fourth Quarter and Full Year 2022 Financial Results

March 14, 2023

*Phase 1 Multiple-Ascending Dose Trial of DT-216 for Friedreich Ataxia Ongoing with Data Expected Mid-Year*

*Continued Progress Across Pipeline of GeneTAC™ Small Molecules with Three Programs Expected to be in Clinical Development in the Next Two Years*

*Strong Financial Position with \$330 Million in Cash and Securities Expected to Support Operating Runway through 2025*

CARLSBAD, Calif., March 14, 2023 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today highlighted recent progress and anticipated upcoming milestones across its clinical and research-stage pipeline of novel GeneTAC™ small molecules and reported fourth quarter and full year 2022 financial results.

"We are very proud of the important progress made last year with our novel GeneTAC™ small molecules, which represent a potential new class of therapies for patients suffering from devastating genetic diseases," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "The data from the Phase 1 single-ascending dose clinical trial of DT-216 in patients with Friedreich ataxia (FA) showed, for the first time, that our GeneTAC™ small molecule DT-216 could be dosed safely and is capable of overcoming the transcription block for frataxin in individuals with FA. This underscores the potential for DT-216 to address the root cause of the disease. We're underway with our Phase 1 multiple-ascending dose trial and look forward to evaluating the effects of three doses of DT-216 in people with FA."

Dr. Siffert continued, "Beyond FA, we further advanced and expanded our earlier-stage pipeline, selecting DT-168 as the development candidate for our second program in FECD and progressing our DM1 program through lead optimization and advancing our discovery portfolio, with the aim of bringing two additional programs into the clinic in the next two years. With a solid cash position to support operations through a steady cadence of milestones ahead, an expert team, and diligent execution, we are well-positioned to deliver on our near- and long-term goals."

### Pipeline Progress and Anticipated Upcoming Milestones

- **Data from Phase 1 Multiple-Ascending Dose (MAD) Trial of DT-216 for Friedreich Ataxia (FA) On-track for Mid-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. Friedreich ataxia 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. In December 2022, Design reported initial results from its single-ascending dose (SAD) Phase 1 clinical trial in patients with FA, which showed that DT-216 was generally well-tolerated and able to overcome the FXN transcription impairment that causes FA, with a greater than two-fold increase in FXN mRNA in the cohort with the highest response. Design is advancing a Phase 1 MAD trial, with initial results expected in mid-2023, and final data expected by year-end. A Phase 2 trial is expected to be initiated in the second half of 2023.
- **IND Submission On-track for DT-168 for Fuchs Endothelial Corneal Dystrophy (FECD) in the Second Half of 2023:** Design nominated its second GeneTAC™ development candidate, DT-168, an eye drop for the treatment of FECD, a genetic eye disease caused by a CTG repeat expansion in over 70% 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. Design plans to submit an Investigational New Drug application (IND) for DT-168 in the second half of 2023.
- **Myotonic Dystrophy Type-1 (DM1) Program Advancing toward IND in 2024:** Design has continued to advance its preclinical characterization of several lead DM1 GeneTAC™ molecules for the treatments of DM1, 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 breath independently. There are currently no approved treatment options for patients with DM1. Design has demonstrated the ability of its DM1 GeneTAC™ small molecules to potently and selectively block expression of the mutant DMPK gene in DM1 patient cells. Design is working toward selection of its development candidate and anticipates submitting an IND in 2024.

### Fourth Quarter and Full Year 2022 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were \$14.3 million for the quarter ended December 31, 2022, and \$48.6 million for the year ended December 31, 2022.
- **G&A Expenses:** General and administrative (G&A) expenses were \$5.1 million for the quarter ended December 31, 2022, and \$19.0 million for the year ended December 31, 2022.
- **Net Loss:** Net loss was \$17.3 million for the quarter ended December 31, 2022, and \$63.3 million for the year ended December 31, 2022.
- **Cash Position:** Cash, cash equivalents and marketable securities were \$330.4 million as of December 31, 2022.

## 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](http://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 aim to bring two additional programs into the clinic in the next two years; Design's ability to meet its stated milestones and advance the GeneTAC™ platform; Design's estimated financial runway and the sufficiency of its resources to support its planned operations; the ability of DT-216 to overcome the transcription block for frataxin in individuals with FA; the potential of DT-216 to address the root cause of FA; 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 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 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, as filed with the SEC on November 3, 2022, and under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, 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)

December 31, 2022	December 31, 2021
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**Assets**

## Current assets:

Cash, cash equivalents and investment securities	\$ 330,387	\$ 384,064
Prepaid expense and other current assets	4,732	1,371
Total current assets	335,119	385,435
Property and equipment, net	1,947	1,508
Right-of-use asset, related party	3,612	3,614
Other assets	459	—
Total assets	<u>\$ 341,137</u>	<u>\$ 390,557</u>

**Liabilities and Stockholders' Equity**

## Current liabilities:

Accounts payable	\$ 3,025	\$ 1,620
Accrued expenses and other current liabilities	7,751	3,663
Total current liabilities	10,776	5,283
Operating lease liability, net, related party	3,051	3,144
Total liabilities	13,827	8,427
Total stockholders' equity	327,310	382,130
Total liabilities and stockholders' equity	<u>\$ 341,137</u>	<u>\$ 390,557</u>

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

	<u>Quarter Ended December 31,</u>		<u>Year Ended December 31,</u>	
	<u>2022</u>	<u>2021</u>	<u>2022</u>	<u>2021</u>
	(unaudited)			
Operating expenses:				
Research and development	\$ 14,255	\$ 7,336	\$ 48,613	\$ 24,778
General and administrative	5,137	3,790	18,980	11,053
Total operating expenses	19,392	11,126	67,593	35,831
Loss from operations	(19,392)	(11,126)	(67,593)	(35,831)
Other income, net	2,052	61	4,285	298
Net loss	<u>\$ (17,340)</u>	<u>\$ (11,065)</u>	<u>\$ (63,308)</u>	<u>\$ (35,533)</u>
Net loss per share, basic and diluted	<u>\$ (0.31)</u>	<u>\$ (0.20)</u>	<u>\$ (1.14)</u>	<u>\$ (0.77)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>55,864,872</u>	<u>55,362,390</u>	<u>55,707,517</u>	<u>45,936,235</u>