Design Therapeutics Highlights Upcoming Milestones and Reports Second Quarter 2022 Financial Results

August 8, 2022

Initial Data from Friedreich Ataxia Phase 1 Trial of DT-216 Expected in the Fourth Quarter of 2022

Strong Financial Position with $359.4 Million in Cash and Securities to Support Multi-Year Operating Runway

CARLSBAD, Calif., Aug. 08, 2022 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today highlighted anticipated upcoming milestones across its clinical and research-stage pipeline of novel GeneTAC™ small molecules and reported second quarter 2022 financial results.

"Throughout the first half of 2022, we have successfully advanced our pipeline of novel GeneTAC™ small molecules designed to address the root cause of disease, without the need for irreversible gene therapy or gene editing. We believe this approach paves the way for disease-modifying treatments for many more patients with inherited genetic diseases," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "Our Phase 1 clinical trial evaluating DT-216, our lead GeneTAC™ molecule, as a treatment for patients with Friedreich ataxia (FA) is progressing well and we look forward to sharing initial data in the fourth quarter of this year. In parallel, we've advanced our GeneTAC™ program for myotonic dystrophy type-1 (DM1), announced compelling preclinical data in Fuchs endothelial corneal dystrophy (FECD), and made meaningful strides across multiple earlier-stage research programs, all of which represent potentially transformative opportunities. With a financial runway to support our current multi-year operating plan, we continue to be well-positioned to execute our milestones."

Upcoming Pipeline Milestones

- **Initial Data from Single-Ascending Dose Portion of Phase 1 trial for DT-216 Expected in the Fourth Quarter of 2022:** DT-216, Design's lead GeneTAC™ molecule, is designed to treat FA by specifically targeting the GAA repeat expansion mutation, the underlying cause of disease, and restore frataxin (FXN) gene expression. DT-216 is being evaluated in a Phase 1 clinical trial in adult patients with FA. The company plans to report initial data, including safety, tolerability, pharmacokinetics and FXN levels from the single-ascending dose portion of the trial in the fourth quarter of 2022. Design expects to initiate dosing of DT-216 in the multiple-ascending dose portion of the Phase 1 study in the second half of 2022.

- **Clinical Development for DM1 Program Anticipated in 2023:** Design's GeneTAC™ program for the treatment of DM1 is progressing through preclinical research. The company anticipates beginning clinical development in 2023.

- **Advancing Research in FECD Throughout the Second Half of 2022:** The company is continuing to advance its preclinical research in FECD, a genetic eye disease characterized by progressive degeneration of the corneal endothelium and subsequent vision impairment. FECD affects millions of people worldwide and is the leading reason for tens of thousands of corneal transplants each year. When tested *in vitro* in FECD patient-derived corneal endothelial cells, Design's FECD GeneTAC™ molecules led to nearly complete reductions in toxic nuclear RNA foci in a time- and concentration-dependent manner. The data were presented at the Association for Research in Vision and Ophthalmology (ARVO) 2022 Annual Meeting in May 2022.

Second Quarter 2022 Financial Results

- **R&D Expenses**: Research and development (R&D) expenses were $11.3 million for the quarter ended June 30, 2022.
- **G&A Expenses**: General and administrative (G&A) expenses were $4.3 million for the quarter ended June 30, 2022.
- **Net Loss:** Net loss was $15.0 million for the quarter ended June 30, 2022.
- **Cash Position**: Cash, cash equivalents and marketable securities were $359.4 million as of June 30, 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's lead program is focused on the treatment of Friedreich ataxia, followed by a program in myotonic dystrophy type-1 and discovery efforts for multiple other serious degenerative disorders caused by nucleotide repeat expansions. 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 and preclinical data; potential transformative opportunities; expectations for reporting data and the timing thereof; Design's ability to meet its stated milestones; the anticipated sufficiency of Design's financial runway; the potential benefits of FXN restoration; the expected initiation of Design's multiple-ascending dose Phase 1 clinical trial for DT-216 in patients with FA and the timing thereof; Design's anticipated timeline to begin clinical development of its GeneTAC™ program for the treatment of DM1 in 2023; Design's FECD GeneTAC ^{TM} program and its potential therapeutic benefits and advantages; Design's belief that its approach paves the way for disease-modifying treatments for many more patients with inherited genetic diseases; Design's ability to bring forward a new class of treatments for patients living with 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,” “planned,” “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 trial 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 March 31, 2022, as filed with the SEC on May 9, 2022, and under the “Risk Factors” heading of Design’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, being filed with the SEC on August 8, 2022. 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)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	11,295	5,027	20,054	8,902
General and administrative	4,344	2,660	8,955	4,465
Total operating expenses	15,639	7,687	29,009	13,367
Loss from operations	(15,639)	(7,687)	(29,009)	(13,367)
Other income, net	640	51	745	217
Net loss	$ (14,999)	$ (7,636)	$ (28,264)	$ (13,150)
Net loss per share, basic and diluted	$ (0.27)	$ (0.14)	$ (0.51)	$ (0.36)
Weighted-average shares of common stock outstanding, basic and diluted	55,670,780	55,081,397	55,589,510	36,459,244

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	June 30, 2022	December 31, 2021
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 359,377	$ 384,064
Prepaid expense and other current assets	2,345	1,371
Total current assets	361,722	385,435
Property and equipment, net	1,804	1,508
Right-of-use asset, related party	3,932	3,614
Other assets	474	—
Total assets	$ 367,932	$ 390,557

Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,071	$ 1,620
Accrued expenses and other current liabilities	5,317	3,663
Total current liabilities	7,388	5,283
Operating lease liability, net, related party	3,381	3,144
Total liabilities	10,769	8,427
Convertible preferred stock	—	—
Total stockholders' equity	357,163	382,130
Total liabilities and stockholders' equity	$ 367,932	$ 390,557