Design Therapeutics Highlights Pipeline Progress and Upcoming Milestones and Reports Third Quarter 2022 Financial Results

November 3, 2022

Initial Data from Single-Ascending Dose Phase 1 Trial of DT-216 for Friedreich Ataxia Expected to be Reported in December 2022

Dosing Initiated in the Multiple-Ascending Dose Phase 1 Trial of DT-216; Trial Completion Anticipated in Mid-2023

Strong Financial Position with $344.2 Million in Cash and Securities to Support Multi-Year Operating Runway and Further Advancement of GeneTAC™ Platform

CARLSBAD, Calif., Nov. 03, 2022 (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 third quarter 2022 financial results.

“At Design, we’re dedicated to bringing our pipeline of novel GeneTAC™ small molecules to patients suffering from devastating diseases, and are making meaningful strides toward achieving that goal,” said João Siffert, M.D., president and chief executive officer of Design Therapeutics. “Our Phase 1 clinical program evaluating DT-216, our lead GeneTAC™ molecule, as a treatment for patients with Friedreich ataxia (FA) is progressing well. We have enrolled patients in the final cohort of the single-ascending dose trial, and plan to assess and report initial data next month. In addition, we recently initiated dosing in the multiple-ascending dose trial, keeping us on-track to complete Phase 1 mid next year. We’ve also continued to advance our GeneTAC™ programs for myotonic dystrophy type-1 (DM1) and Fuchs endothelial corneal dystrophy (FECD), as well as multiple earlier-stage programs, all of which represent potential disease-modifying approaches to treating patients in need. With a solid cash position, expert team and strong science behind us, I’m confident in our ability to execute our milestones.”

DT-216 Progress and Upcoming Milestones

- **Initial Data from Single-Ascending Dose Phase 1 Trial for DT-216 On-track to be Reported in December 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. Design is evaluating DT-216 in a Phase 1 single-ascending dose (SAD) clinical trial in adult patients with FA, and plans to report initial SAD data, including safety, tolerability, pharmacokinetics and FXN levels in December 2022.
- **Dosing Initiated in Multiple-Ascending Dose Phase 1 Trial for DT-216:** Design has also initiated patient dosing in a multiple-ascending dose (MAD) Phase 1 clinical trial of DT-216 in patients with FA. The MAD trial is designed to evaluate the safety, tolerability, pharmacokinetic, and pharmacodynamic effects of three weekly doses of DT-216 in adult patients with FA. Design plans to complete the MAD trial in mid-2023.
- **Preclinical Data Supporting DT-216 for the Treatment of FA Presented at ICAR:** Preclinical data supporting DT-216 as a potential treatment for FA were presented during the plenary session on emerging therapeutics at the International Congress for Ataxia Research (ICAR) 2022. The data, which were also included in Design’s Investigational New Drug Application (IND) for DT-216, highlight the potential for DT-216 to restore FXN gene expression, improve mitochondrial function and address the root cause of FA.

Anticipated Pipeline Milestones

- **Clinical Development for DM1 Program Anticipated in 2023:** Design is advancing its GeneTAC™ program for the treatment of DM1 through preclinical research and anticipates beginning clinical development in 2023.
- **Continued Advancement in Research for FECD:** Design is continuing to advance its preclinical research in FECD, a common genetic eye disease characterized by progressive degeneration of the corneal endothelium, vision impairment and need for corneal transplant in advanced cases.

Third Quarter 2022 Financial Results

- **R&D Expenses**: Research and development (R&D) expenses were $14.3 million for the quarter ended September 30, 2022.
- **G&A Expenses**: General and administrative (G&A) expenses were $4.9 million for the quarter ended September 30, 2022.
- **Net Loss:** Net loss was $17.7 million for the quarter ended September 30, 2022.
- **Cash Position**: Cash, cash equivalents and marketable securities were $344.2 million as of September 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 and advance the GeneTAC™ platform; the anticipated sufficiency of Design's financial runway; the ability of DT-216 to restore FXN gene expression, improve mitochondrial function and address the root cause of FA; the expected initial data report for the SAD Phase 1 clinical trial for DT-216 in patients with FA and the timing thereof; the expected completion of the MAD Phase 1 clinical trial for DT-216 in patients with FA; Design's anticipated timeline to begin clinical development of its GeneTAC™ program for the treatment of DM1 in 2023; Design's FECD GeneTAC™ program and its potential therapeutic benefits and advantages; Design's belief that its approach paves the way for disease-modifying treatments for patients with inherited 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," "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 June 30, 2022, as filed with the SEC on August 8, 2022, and under the "Risk Factors" heading of Design's Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, being filed with the SEC on November 3, 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.
BALANCE SHEETS
(in thousands)

	September 30, 2022	December 31, 2021
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 344,233	$ 384,064
Prepaid expense and other current assets	4,255	1,371
Total current assets	348,488	385,435
Property and equipment, net	1,842	1,508
Right-of-use asset, related party	3,773	3,614
Other assets	466	—
Total assets	$ 354,569	$ 390,557
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,622	$ 1,620
Accrued expenses and other current liabilities	7,790	3,663

Total current liabilities	10,412	5,283
Operating lease liability, net, related party	3,219	3,144
Total liabilities	13,631	8,427
Convertible preferred stock	—	—
Total stockholders' equity	340,938	382,130
Total liabilities and stockholders' equity	$ 354,569	$ 390,557

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	14,304	8,539	34,358	17,441
General and administrative	4,888	2,798	13,843	7,263
Total operating expenses	19,192	11,337	48,201	24,704
Loss from operations	(19,192)	(11,337)	(48,201)	(24,704)
Other income, net	1,488	19	2,233	236
Net loss	$ (17,704)	$ (11,318)	$ (45,968)	$ (24,468)
Net loss per share, basic and diluted	$ (0.32)	$ (0.21)	$ (0.83)	$ (0.57)
Weighted-average shares of common stock outstanding, basic and diluted	55,782,329	55,155,030	55,654,490	42,759,656