Design Therapeutics Reports Pipeline and Business Progress and Fourth Quarter and Full Year 2021 Financial Results

March 10, 2022

Phase 1 Trial of DT-216, a Novel FA GeneTAC™ Molecule, in Patients with Friedreich Ataxia On Track to Begin Soon

Preclinical Data Supporting Development of Novel GeneTAC™ Small Molecules for the Treatment of Fuchs Endothelial Corneal Dystrophy (FECD) to be Presented at ARVO 2022

Well-Capitalized with $384.1 Million in Cash and Investments at the End of 2021 to Support Upcoming Milestones

CARLSBAD, Calif., March 10, 2022 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for degenerative genetic disorders, today announced pipeline and business progress and reported fourth quarter and full year 2021 financial results.

“This is an exciting time for Design as we continue to progress our pipeline of novel GeneTAC™ molecules toward patients, particularly those with limited therapeutic options or no disease modifying treatments available today,” said João Siffert, M.D., president and chief executive officer of Design Therapeutics. “The recent clearance of our IND for DT-216 is a significant milestone for the company, and we’re excited to begin our clinical trial in patients with Friedreich ataxia, a genetic, progressive disease that has a life-altering impact. In parallel, we continue to advance our myotonic dystrophy type-1 GeneTAC™ molecules toward clinical development, and have recently generated encouraging new data showing that our GeneTAC™ molecules may be able to correct the most common underlying genetic cause of Fuchs endothelial corneal dystrophy, a prevalent genetic condition affecting over 1 million people in the United States that can lead to vision loss. 2022 is set to be a meaningful year for Design, with key upcoming milestones and a pipeline comprised of potentially transformative new genomic medicines.”

“Design was founded to address the known monogenic causes of a range of inherited degenerative diseases and now, just two years from launch, is on the cusp of initiating its first clinical trial,” added Pratik Shah, Ph.D., executive chair of Design Therapeutics. “With a highly productive platform, an experienced team and a strong balance sheet in place, the company is well-positioned to advance its robust pipeline to bring forward a new class of treatments for patients living with devastating genetic diseases.”

Recent Pipeline Progress

- **IND Application for FA Development Candidate, DT-216, Cleared by the FDA:** The Investigational New Drug Application (IND) for DT-216, a GeneTAC™ small molecule being developed for the treatment of Friedreich ataxia (FA), was recently cleared by the U.S. Food and Drug Administration (FDA). Data from multidose IND-enabling studies in rodents and non-human primates showed that systemic administration of DT-216 was well-tolerated at dose levels that achieved concentrations in the CNS, heart, and skeletal muscle well in excess of those required to restore frataxin (FXN) gene expression in FA cells *in vitro*. Design is preparing to initiate a Phase 1 clinical trial of DT-216 to assess the safety, tolerability, pharmacokinetics and frataxin levels in patients with FA.

Upcoming Medical Meeting Presentations

- **Preclinical Data Highlighting Novel GeneTAC™ Therapy for the Treatment of FECD to be Presented at ARVO 2022:** Design will present preclinical data supporting the potential of the company’s novel GeneTAC™ small molecules to correct the most common underlying genetic cause of Fuchs endothelial corneal dystrophy (FECD) during a poster session at the Association for Research in Vision and Ophthalmology 2022 Annual Meeting (ARVO 2022), which is being held in Denver, Colorado from May 1- 4, 2022 and virtually from May 11-12, 2022. Details of the presentation are as follows:

  Title: GeneTAC™ small molecules reduce toxic nuclear foci and restore normal splicing in corneal endothelial cells derived from patients with Fuchs endothelial corneal dystrophy (FECD) harboring repeat expansions in transcription factor 4 (*TCF4*)
  Session Title: Corneal Endothelium
  Session Abbreviation: 334
  Date/Time: Tuesday, May 3, 2022 at 1:00 p.m. MST

Recent Business Progress

- **Strengthened Leadership Team with Appointment of Chief Medical Officer:** Design appointed drug development expert, Jae Kim, M.D., FACC, as chief medical officer.

Fourth Quarter and Full Year 2021 Financial Results

- **R&D Expenses:** Research and development (R&D) expenses were $7.3 million for the quarter ended December 31, 2021, and $24.8 million for the year ended December 31, 2021.
- **G&A Expenses:** General and administrative (G&A) expenses were $3.8 million for the quarter ended December 31, 2021, and $11.1 million for the year ended December 31, 2021.
- **Net Loss:** Net loss was $11.1 million for the quarter ended December 31, 2021, and $35.5 million for the year ended December 31, 2021.
- **Cash Position:** Cash, cash equivalents and marketable securities were $384.1 million as of December 31, 2021.

About Design Therapeutics

Design Therapeutics is a 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, statements related to: the expected initiation of Design's Phase 1 clinical trial of DT-216 in patients with Friedreich ataxia and the timing thereof; the potential initiation of a clinical trial in patients with myotonic dystrophy type-1 and the timing thereof; the ability of Design's GeneTAC™ molecules to correct the most common underlying genetic cause of Fuchs endothelial corneal dystrophy; the potential advancement of Design's pipeline and ability to bring forward a new class of treatments for patients living with devastating genetic diseases; and the potential advantages of these GeneTAC™ programs. 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," "goal," "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 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; 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, 2021, as filed with the SEC on November 9, 2021, and under the "Risk Factors" heading of Design's Annual Report on Form 10-K for the fiscal year ended December 31, 2021, which is being filed with the SEC on March 10, 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)

	Quarter Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
	(unaudited)			
Revenue:				
Grant revenue	$ —	$ 33	$ —	$ 226
Operating expenses:				
Research and development	7,336	2,708	24,778	6,060

General and administrative	3,790	1,186	11,053	2,496
Total operating expenses	11,126	3,894	35,831	8,556
Loss from operations	(11,126)	(3,861)	(35,831)	(8,330)
Other income, net	61	6	298	50
Net loss	$ (11,065)	$ (3,855)	$ (35,533)	$ (8,280)
Net loss per share, basic and diluted	$ (0.20)	$ (0.24)	$ (0.77)	$ (0.52)
Weighted-average shares of common stock outstanding, basic and diluted	55,362,390	15,925,284	45,936,235	15,796,674

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS

(in thousands)

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash, cash equivalents and investment securities	$ 384,064	$ 36,091
Prepaid expense and other current assets	1,371	142
Total current assets	385,435	36,233
Property and equipment, net	1,508	71
Right-of-use asset, related party	3,614	—
Deferred offering costs	—	212
Total assets	$ 390,557	$ 36,516
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 1,620	$ 1,399
Accrued expenses and other current liabilities	3,663	931
Total current liabilities	5,283	2,330
Operating lease liability, net, related party	3,144	—
Other long-term liabilities	—	145
Total liabilities	8,427	2,475
Convertible preferred stock	—	45,356
Total stockholders' equity (deficit)	382,130	(11,315)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 390,557	$ 36,516