Design Therapeutics Reports Pipeline Progress and Third Quarter 2021 Results

November 9, 2021

Clinical Initiation of Lead GeneTAC™ Program for Friedreich Ataxia On-track for the First Half of 2022

CARLSBAD, Calif., Nov. 09, 2021 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for degenerative genetic disorders, today reported recent pipeline progress and third quarter 2021 financial results.

"At Design, our mission is to bring a new class of small-molecule genomic medicines to patients with inherited degenerative diseases. We've taken major strides toward achieving our goals, with advancements across our pipeline and GeneTAC™ platform, and expansion of our leadership team to support our expected near-term transition to a clinical-stage organization," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "We expect 2022 will be a milestone-rich and transformational year for the company, including initiation of the clinical program in Friedreich ataxia, progress with our myotonic dystrophy type-1 GeneTAC™ program, and expansion of our pipeline with several earlier stage programs that represent exciting opportunities to treat additional diseases caused by nucleotide repeat expansions."

Recent Pipeline Highlights

- **Friedreich Ataxia (FA) GeneTAC™ Program On-track for Clinical Initiation in First Half of 2022:** In ongoing IND-enabling studies, Design's FA GeneTAC ™ clinical candidate has been shown to be well tolerated in repeat dose GLP toxicity studies in rats and non-human primates at doses that exceed what we estimate to be biologically active in the clinic. Design remains on track to initiate a Phase 1 clinical trial in patients with FA in the first half of 2022, with initial topline clinical data expected in 2022.
- **Positive Preclinical Data Reported Highlighting Disease-Modifying Potential of DM1 GeneTAC™ Molecules for Myotonic Dystrophy Type-1 (DM1)**: Design reported new preclinical data from its novel DM1 GeneTAC™ program at the 2021 Virtual Myotonic Dystrophy Foundation Annual Conference in September 2021, including demonstration of near-complete resolution of disease-causing foci and correction of splicing defects in DM1 patient cells. Preclinical *in vivo* studies demonstrated distribution of DM1 GeneTAC™ molecules to key target tissues including skeletal muscle and heart, achieving tissue concentrations that reduced nuclear foci and corrected mRNA splicing in our *in vitro* experiments. These data support the continued advancement of the DM1 program and underscore the broader potential of GeneTAC™ molecules to treat multiple additional nucleotide repeat expansion diseases. The company remains on track to initiate its clinical program in DM1 in 2023.

Upcoming Investor Conference Presentations

- **33rd Annual Virtual Piper Healthcare Conference:** João Siffert, M.D., president and chief executive officer and Sean Jeffries, Ph.D., chief operating officer, will participate in a fireside chat during the 33rd Annual Virtual Piper Healthcare Conference, being held November 30 – December 2, 2021. The pre-recorded fireside chat will be available to registered participants beginning on November 22, 2021.

The webcast will be available in the investor section of the company's website at www.designtx.com and will be archived for 30 days following the presentation.

Third Quarter 2021 Financial Results

- Net loss for the third quarter ended September 30, 2021, was $11.3 million.
- Research and development expenses for the third quarter of 2021 were $8.5 million.
- General and administrative expenses for the third quarter of 2021 were $2.8 million.
- As of September 30, 2021, the company had cash, cash equivalents and investment securities of $393.7 million.

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 turn on or turn off 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 progress and expected timing of Design's development programs and any clinical trials; expected timing for initial topline data; the effectiveness of Design's GeneTAC ™ program in the treatment of Friedreich ataxia and myotonic dystrophy type-1; the potential advantages of these GeneTAC™ programs; Design's continued growth, including the expected expansion of our pipeline and the timing thereof; and the strength of Design's balance sheet and the adequacy of cash on hand. 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," "on track," "plans," "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, developing and commercializing 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, obtain approvals for and commercialize any of 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; preliminary or expected results; changes in Design's plans to develop and commercialize its product candidates; the risk that Design may not obtain approval to market 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; changes in Design's plans to develop and commercialize its product candidates; 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. 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 September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Revenue:				
Grant revenue	$ -	$ 20	$ -	$ 193
Operating expenses:				
Research and development	8,539	1,914	17,441	3,352
General and administrative	2,798	489	7,263	1,310
Total operating expenses	11,337	2,403	24,704	4,662
Loss from operations	(11,337)	(2,383)	(24,704)	(4,469)
Other income, net	19	63	236	44
Net loss	$ (11,318)	$ (2,320)	$ (24,468)	$ (4,425)
Net loss per share, basic and diluted	$ (0.21)	$ (0.09)	$ (0.57)	$ (0.17)
Weighted-average shares of common stock outstanding, basic and diluted	55,155,030	25,701,737	42,759,656	25,606,779

DESIGN THERAPEUTICS, INC.
BALANCE SHEETS
(in thousands)

	September 30, 2021	December 31, 2020
	(unaudited)	
Assets		
Current assets:		

Cash, cash equivalents and investment securities	$ 393,718	$ 36,091
Prepaid expense and other current assets	1,572	142
Total current assets	395,290	36,233
Property and equipment, net	1,320	71
Right-of-use asset	3,743	—
Deferred offering costs	—	212
Total assets	$ 400,353	$ 36,516
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,216	$ 1,399
Accrued expenses and other current liabilities	3,289	931
Total current liabilities	5,505	2,330
Operating lease liability, net	3,273	—
Other long-term liabilities	11	145
Total liabilities	8,789	2,475
Convertible preferred stock	—	45,356
Total stockholders' equity (deficit)	391,564	(11,315)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 400,353	$ 36,516