



Design Therapeutics Reports GeneTAC™ Portfolio Progress and Second Quarter 2021 Results

August 9, 2021

Preclinical Data Support Initiation of Clinical Development of Lead GeneTAC Program for Friedreich Ataxia in the First Half of 2022

Leadership Team Strengthened with Appointment of Elizabeth Gordon, Ph.D., as Senior Vice President of Regulatory Affairs

CARLSBAD, Calif., Aug. 09, 2021 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for degenerative genetic disorders, today reported recent progress with its portfolio of novel small molecule gene targeted chimeras (GeneTACs™), as well as business highlights and second quarter 2021 financial results.

“So far in 2021, we’ve made substantial progress as a company, highlighted by the compelling new data from ongoing IND-enabling studies with our lead GeneTAC program for Friedreich ataxia. Importantly, we’ve observed well-tolerated GeneTAC doses in rodents and non-human primates that produced ample biodistribution into key tissues affected by the disease, including the brain, increasing our confidence in the potential of this program as a disease-modifying treatment for patients,” said João Siffert, M.D., president and chief executive officer of Design Therapeutics. “With plans to begin clinical development in the first half of 2022, we are pleased to welcome Dr. Elizabeth Gordon to the team, who brings decades of valuable experience in overseeing US and ex-US regulatory affairs and successful regulatory submissions.”

“Design is well positioned to advance our research and development activities targeting a number of nucleotide repeat expansion diseases, enabled by a talented team that includes multiple new additions in our R&D organization and a strong balance sheet to fuel our pipeline of novel GeneTAC programs,” added Pratik Shah, Ph.D., co-founder and executive chair of Design Therapeutics. “We are now preparing for the important transition to a clinical-stage organization, bringing us another step closer to delivering a new class of genomic medicines for a range of serious disorders currently without approved treatments.”

Pipeline Progress

- **New Data from IND-enabling Studies with GeneTAC Product Candidate for Friedreich Ataxia (FA) Support Initiation of Clinical Trial in First Half of 2022:** Data from IND-enabling studies in rodents and non-human primates showed that multidose systemic administration of the company’s FA GeneTAC was well tolerated and achieved higher concentrations in the CNS (cerebrum, cerebellum, brainstem and spinal cord), heart, and skeletal muscle than needed to restore frataxin (FXN) gene expression. In addition, the company observed that exposure to low nanomolar (nM) concentrations of its FA GeneTAC in neurons and cardiomyocytes derived from FA patient stem cells in *in vitro* experiments led to robust and durable increases in FXN mRNA, as well as an increase in endogenous protein reaching levels comparable to unaffected individuals.
- **On-track for FA GeneTAC Initiation of Clinical Trial in First Half of 2022:** Design has received scientific advice from the European Medicines Agency consistent with the favorable feedback previously received from the U.S. Food and Drug Administration (FDA), supporting the development plan for its FA GeneTAC. In addition, the company has successfully manufactured both drug substance and product at a scale sufficient for clinical use. Design anticipates initiating a Phase 1 clinical trial in patients with Friedreich ataxia in the first half of 2022, with initial topline clinical data expected in the second half of 2022.
- **Continued Progress across GeneTAC Portfolio with Myotonic Dystrophy with Preclinical Data to be Reported in Second Half of 2021:** Design has continued to advance its second GeneTAC program focused on treating myotonic dystrophy type-1 (DM1), a genetic disorder that causes progressive muscle weakness and for which there are currently no approved treatment options. The company plans to report preclinical data supporting its potential to develop a disease-modifying treatment for patients with DM1 at a medical meeting in the second half of 2021. In addition, leveraging its GeneTAC platform, Design is progressing several earlier-stage programs targeting diseases caused by inherited nucleotide repeat expansions, which represent significant pipeline opportunities to address serious unmet medical needs.

Business Highlights

- **Leadership Team Strengthened with Appointment of Elizabeth Gordon, Ph.D. as Senior Vice President of Regulatory Affairs:** Dr. Gordon is a recognized expert in regulatory affairs with over 30 years of experience in the pharmaceutical industry and at the FDA. Dr. Gordon most recently served as senior vice president of regulatory affairs at Amlyx Pharmaceuticals, and before that as vice president, regulatory affairs at Shire Pharmaceuticals and vice president of regulatory affairs at Lumena Pharmaceuticals. Earlier in her career, Dr. Gordon served in the Center for Biologics Evaluation and Research and the Center for Drug Evaluation and Research at the FDA, where she was instrumental in developing policy for the regulation of biological products. Dr. Gordon earned her Ph.D. in microbiology from the University of Rhode Island.
- **Board of Directors Expanded with Key Appointments:** In June, Design further enhanced its board with the appointments of industry veterans, Heather Behanna, Ph.D., principal of SR One, and Deepa Prasad, managing director of WestRiver Group, as directors of the company.

Second Quarter 2021 Financial Results

- Net loss for the second quarter ended June 30, 2021 was \$7.6 million.

- Research and development expenses for the second quarter of 2021 were \$5.0 million.
- General and administrative expenses for the second quarter of 2021 were \$2.7 million.
- As of June 30, 2021, the company had cash, cash equivalents and investment securities of \$402.8 million.

About Design Therapeutics

Design Therapeutics is a biotechnology company developing a new class of therapies based on a platform of gene targeted chimera (GeneTAC™) 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; 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; 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," "anticipates," "plans," "expects," "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; 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; our 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. 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,	
	2021	2020	2021	2020
Revenue:				
Grant revenue	\$ —	\$ 31	\$ —	\$ 173
Operating expenses:				
Research and development	5,027	1,061	8,902	1,438
General and administrative	2,660	433	4,465	821
Total operating expenses	<u>7,687</u>	<u>1,494</u>	<u>13,367</u>	<u>2,259</u>
Loss from operations	(7,687)	(1,463)	(13,367)	(2,086)
Other income (expense), net	51	21	217	(19)
Net loss	<u>\$ (7,636)</u>	<u>\$ (1,442)</u>	<u>\$ (13,150)</u>	<u>\$ (2,105)</u>
Net loss per share, basic and diluted	<u>\$ (0.14)</u>	<u>\$ (0.06)</u>	<u>\$ (0.36)</u>	<u>\$ (0.08)</u>
Weighted-average shares of common stock outstanding, basic and diluted	<u>55,081,397</u>	<u>25,597,154</u>	<u>36,459,244</u>	<u>25,558,779</u>

DESIGN THERAPEUTICS, INC.
CONDENSED BALANCE SHEETS
 (in thousands)

(unaudited)

	<u>June 30,</u> <u>2021</u>	<u>December 31,</u> <u>2020</u>
	(unaudited)	
Assets		
Current assets:		
Cash, cash equivalents and investment securities	\$ 402,836	\$ 36,091
Prepaid expense and other current assets	2,039	142
Total current assets	<u>404,875</u>	<u>36,233</u>
Property and equipment, net	271	71
Deferred offering costs	—	212
Total assets	<u>\$ 405,146</u>	<u>\$ 36,516</u>
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	\$ 1,585	\$ 1,399
Accrued expenses	1,820	931
Total current liabilities	<u>3,405</u>	<u>2,330</u>
Other long-term liabilities	140	145
Total liabilities	<u>3,545</u>	<u>2,475</u>
Convertible preferred stock	—	45,356
Total stockholders' equity (deficit)	<u>401,601</u>	<u>(11,315)</u>
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 405,146</u>	<u>\$ 36,516</u>