



## Design Therapeutics Announces Business Highlights and Reports First Quarter 2021 Financial Results

May 10, 2021

*Successful \$276 Million IPO Completed to Advance GeneTAC™ Programs for Friedreich Ataxia and Myotonic Dystrophy Type-1 Toward Clinical Development*

*Company On-track to Initiate Phase 1 Clinical Trial for the Treatment of Friedreich Ataxia in the First Half of 2022*

CARLSBAD, Calif., May 10, 2021 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a biotechnology company developing treatments for degenerative genetic disorders, today reported business highlights and first quarter 2021 financial results.

"2021 has been a meaningful year so far for Design, with marked progress across our proprietary gene targeted chimera (GeneTAC™) platform, our small molecule pipeline, and our business operations," said João Siffert, M.D., president and chief executive officer of Design Therapeutics. "We transitioned to a public company and stand well-capitalized to fuel the continued advancement of our platform, led by the development of a potentially disease-modifying treatment for people living with Friedreich ataxia, a debilitating autosomal-recessive genetic disease. Following a recent successful pre-IND meeting with the FDA, we remain on track to initiate clinical development for our FA program in the first half of 2022 and make the important transformation to a clinical-stage organization."

### Business Highlights

- **On-track with Clinical Development Plans for Friedreich Ataxia Program Following Pre-IND Meeting with FDA:** Design Therapeutics successfully completed a pre-Investigational New Drug (IND) meeting with the U.S. Food and Drug Administration (FDA) regarding the preclinical and clinical development plans for its lead GeneTAC program for the treatment of Friedreich ataxia (FA). As part of the meeting, Design gained alignment with the FDA on its development plans and, pending regulatory approval, is preparing to initiate a Phase 1 clinical trial in patients with FA in the first half of 2022. FA is a devastating disease for which more than 95% of cases are caused by homozygous guanine-adenine-adenine (GAA) triplet repeat expansions in the first intron of the FXN gene.
- **Myotonic Dystrophy Type-1 Program Advancing as Planned:** Design's second GeneTAC program is focused on the development of a potentially disease-modifying treatment for myotonic dystrophy type-1 (DM1). The company is advancing its DM1 GeneTAC program, with plans to seek regulatory clearance for a first-in-human trial in 2023.
- **\$276 Million Initial Public Offering (IPO) Successfully Completed:** In March 2021, Design sold 13,800,000 shares of its common stock, which included 1,800,000 shares sold pursuant to the exercise in full by the underwriters of their option to purchase additional shares, at a public offering price of \$20.00 per share. Including the option exercise, the aggregate gross proceeds to Design from the offering were \$276.0 million, before deducting the underwriting discounts and commissions and offering expenses.

### First Quarter 2021 Financial Results

For the first quarter ended March 31, 2021, Design reported a net loss of \$5.5 million, compared to a net loss of \$0.7 million for the comparable period in 2020.

Research and development expenses for the first quarter of 2021 were \$3.9 million, compared to \$0.4 million for the comparable period in 2020. The increase in the company's research and development expenses in 2021 was primarily attributable to the advancement of its FA program and related activities, including chemistry and manufacturing development costs, and costs incurred on its DM1 program in 2021 that were not incurred in 2020. Further the company incurred higher personnel costs to support its development programs, including an additional \$0.2 million of non-cash stock-based compensation costs.

General and administrative expenses for the first quarter of 2021, were \$1.8 million, compared to \$0.4 million for the comparable period in 2020. The increase in general and administrative expenses in 2021 was primarily attributable to increased personnel and related costs as the company expanded its general and administrative team to support its operations, including an additional \$0.5 million of non-cash stock-based compensation costs. Further, the company incurred increased professional fees for legal and accounting services during the first quarter of 2021 as compared to same period in 2020.

As of March 31, 2021, Design reported cash, cash equivalents and investment securities of \$411.3 million, an increase of \$375.2 million from the \$36.1 million reported as of December 31, 2020. The increase during the first quarter of 2021 was attributed to the net proceeds from the company's IPO in March 2021 and its Series B convertible preferred stock financing in January 2021.

## 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. Our GeneTAC molecules are designed to either turn on or turn off a specific disease-causing gene to address the underlying cause of disease. The company'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](http://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; 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; 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; the loss of 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.

## Contact:

Alicia Davis  
THRUST Strategic Communications  
(910) 620-3302  
[alicia@thrustsc.com](mailto:alicia@thrustsc.com)

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

	Three Months Ended March 31,	
	2021	2020
Revenue:		
Grant revenue	\$ —	\$ 142
Operating expenses:		
Research and development	3,875	377
General and administrative	1,805	388
Total operating expenses	5,680	765
Loss from operations	(5,680)	(623)
Other income (expense), net	166	(40)
Net loss	\$ (5,514)	\$ (663)
Net loss per share, basic and diluted	\$ (0.31)	\$ (0.04)
Weighted-average shares of common stock outstanding, basic and diluted	17,630,178	15,667,115

**DESIGN THERAPEUTICS, INC.**  
**BALANCE SHEETS**  
(in thousands)  
(unaudited)

March 31,

December 31,

	<u>2021</u>	<u>2020</u>
	(unaudited)	
<b>Assets</b>		
Current assets:		
Cash, cash equivalents and investment securities	\$ 411,339	\$ 36,091
Prepaid expense and other current assets	415	142
Total current assets	<u>411,754</u>	<u>36,233</u>
Property and equipment, net	100	71
Deferred offering costs	—	212
Total assets	<u>\$ 411,854</u>	<u>\$ 36,516</u>
<b>Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)</b>		
Current liabilities:		
Accounts payable	\$ 2,033	\$ 1,399
Accrued expenses	1,673	931
Total current liabilities	<u>3,706</u>	<u>2,330</u>
Other long-term liabilities	142	145
Total liabilities	<u>3,848</u>	<u>2,475</u>
Convertible preferred stock	—	45,356
Total stockholders' equity (deficit)	<u>408,006</u>	<u>(11,315)</u>
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 411,854</u>	<u>\$ 36,516</u>