Design Therapeutics Completes Dosing in First Patient Cohort of Phase 1 Trial of DT-216 GeneTAC™ Molecule for the Treatment of Friedreich Ataxia

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Topline Data from Phase 1 Trial Expected in the Second Half of 2022

DT-216 Granted FDA Fast Track Designation for Patients with Friedreich Ataxia

CARLSBAD, Calif., March 30, 2022 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for degenerative genetic disorders, today announced that it has completed dosing in the first single ascending dose (SAD) cohort of its Phase 1 clinical trial of DT-216 in patients with Friedreich ataxia (FA). DT-216 is a novel GeneTAC™ gene targeted chimera small molecule designed to specifically target the GAA repeat expansion mutation, the underlying cause of FA, and restore frataxin (FXN) gene expression. Additionally, Design announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to DT-216 for the treatment of patients with FA.

“The rapid advancement of our Phase 1 trial of DT-216 – from initiation to dosing completion in the first SAD cohort – marks both our transition to a clinical-stage company and underscores the urgent need for effective treatments for patients with degenerative genetic disorders,” said João Siffert, M.D., president and chief executive officer of Design Therapeutics. “The FDA’s decision to grant Fast Track designation to DT-216 reflects the tremendous need for an effective treatment for people with FA. Our GeneTAC™ molecules are thoughtfully designed to dial up or dial down the expression of disease-causing genes, thereby addressing the root cause of the disease without the need for irreversible genetic modification. In a disease like FA, where deficiency of FXN causes its clinical manifestations, we believe the potential FXN restoration by DT-216 could have a meaningful clinical impact. Preclinical data demonstrated that DT-216 was well tolerated at dose levels projected to achieve concentrations in the CNS, heart and skeletal muscle in excess of those required to restore FXN gene expression in FA patient derived cells *in vitro.* We look forward to assessing the potential of DT-216 in the clinic with hopes of offering a disease modifying treatment for people living with FA.”

“FA is a devastating and life-shortening degenerative condition for which there are no effective treatments or cures available today,” said Jennifer Farmer, MS, Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA). “At FARA, we fund and facilitate research for FA, so that we can help bring forward treatments to improve the quality and length of life for those diagnosed with the condition. We are grateful for the progress that Design has made in advancing DT-216 development and initiating the Phase 1 clinical trial, as this is an important milestone for the entire FA community.”

Design’s Phase 1 clinical trial of DT-216 is a randomized, double-blind, placebo-controlled study to evaluate the safety, tolerability, pharmacokinetics and FXN levels from single ascending doses of IV-administered DT-216 in adult patients with FA. FA patients are expected to receive a single dose of either DT-216 or placebo across five ascending dose cohorts. Additional information may be found at ClinicalTrials.gov, using Identifier NCT: NCT05285540. The company anticipates reporting topline data from the Phase 1 trial in the second half of 2022.

The Fast Track process is designed to facilitate the development and expedite the review of investigational treatments that demonstrate the potential to address unmet medical needs in serious or life-threatening conditions. With Fast Track designation, DT-216 is eligible for early and frequent communication with the FDA throughout the entire drug development and review process. In addition, it is now eligible for Accelerated Approval and Priority Review, if relevant criteria are met, and a Rolling Review for its New Drug Application (NDA).

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, statements related to: projections from preclinical data; the expected timing for reporting topline data; the potential benefits of FXN restoration; the potential benefits of Fast Track designation; Design’s ability to bring forward a new class of treatments for patients living with devastating genetic diseases; and the capabilities and potential advantages 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,” "anticipates," “planned,” "expects," “estimate,” "intends," "will," "goal," "potential", “project” 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 trial 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2021, as 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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