Design Therapeutics Announces Four-Week IV Data from the RESTORE-FA Trial of DT-216P2 Demonstrating Clinical Improvements and Comprehensive Biomarker Activity in Friedreich Ataxia

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DT-216P2 demonstrated dose-dependent improvement in multiple clinical measures and increases in endogenous frataxin mRNA and protein after four weeks of intravenous dosing

DT-216P2 generally well-tolerated

Data support advancement toward registrational development

Management to host conference call and webcast today at 8:00 am ET

CARLSBAD, Calif., May 18, 2026 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today announced positive biomarker and clinical data from the ongoing Phase 1/2 RESTORE-FA trial evaluating DT-216P2 in patients with Friedreich ataxia (FA). DT-216P2 is a GeneTAC® small-molecule therapeutic candidate designed to increase frataxin (FXN) expression by targeting the GAA repeat expansion in the FXN gene, the genetic root cause of FA.

“These data represent an advancement for Friedreich ataxia treatment, demonstrating that DT-216P2 increased endogenous frataxin and translated biomarker observations into clinical improvements after only four weeks of treatment,” said Pratik Shah, Chief Executive Officer of Design Therapeutics. “To our knowledge, this is the first time increases in endogenous FXN have been observed alongside clinical improvements of this magnitude in patients with FA, overcoming the presence of the underlying GAA repeat expansion. We observed both dose-dependent increases in FXN levels and dose-dependent improvements across multiple clinical measures, including mFARS, upright stability score and patient-reported fatigue. Based on these findings, we believe DT-216P2 has the potential to be a best-in-disease treatment for patients with FA and look forward to advancing the program toward registrational development.”

RESTORE-FA Key Data Highlights
RESTORE-FA is a Phase 1/2 clinical trial evaluating DT-216P2 in patients with FA, designed to assess safety, pharmacokinetics, pharmacodynamics, and exploratory clinical endpoints. As of May 17, 2026, 16 patients had completed treatment with weekly intravenous DT-216P2 across dose cohorts of 0.1, 0.3, 0.6, and 1 mpk (n=4 per cohort) for four weeks.

Clinical Outcomes
After four weeks of DT-216P2 treatment at the 1 mpk dose cohort, patients demonstrated mean improvements from baseline of 6.4 points in the modified Friedreich’s Ataxia Rating Scale (mFARS) and 2.7 points in the Upright Stability Score. Further, DT-216P2 demonstrated changes of greater than five points in patient-reported fatigue, as measured by the PROMIS Fatigue Scale, both at the end of four weeks of treatment and two weeks following the last dose. These data exceeded the three-point threshold generally considered to be a minimal important change in fatigue.

Biomarker and Safety Results
Dose-dependent increases in endogenous FXN were observed following treatment with DT-216P2 across FXN mRNA and protein assays in whole blood, as well as FXN mRNA measurements in affected muscle tissue, demonstrating activity in both blood and muscle.

Following four weeks of treatment at 1 mpk, whole blood FXN mRNA levels increased by 65% from baseline ($p < 0.001$). Whole blood FXN-M and FXN-E protein levels increased by 22-27% from baseline two weeks following the last dose ($p < 0.001$). Muscle FXN mRNA levels increased by 42% from baseline ($p = 0.015$). Together, these findings demonstrate comprehensive biomarker activity with meaningful increases in FXN mRNA and protein, as well as activity in both blood and muscle caused by DT-216P2 treatment. The biomarker data provide mechanistic support for the observed clinical improvements in FA patients.

DT-216P2 was generally well-tolerated, with no serious adverse events or treatment discontinuations reported. All adverse events were mild or moderate. Adverse events considered possibly or probably related to DT-216P2 occurring in more than one patient included mild to moderate transient alanine transaminase (ALT) elevations observed in three patients, all of which were asymptomatic with no associated increases in bilirubin and on background omaveloxolone. Transient ALT elevations are anticipated with enhanced mitochondrial activity, a downstream consequence of FXN restoration.

Next Steps for DT-216P2
Based on these data, the company intends to pursue a registrational path and provide an update on its plans in the fourth quarter of 2026.

Webcast and Conference Call Information
Design will host a live webcast and conference call today at 8:00 am ET to discuss the RESTORE-FA data findings and updates. The event is accessible through the Events section of the Investors page of www.designtx.com. A replay of the webcast will be archived on the Design website for 30 days.

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. In addition to its clinical-stage GeneTAC® programs, DT-216P2, in development for patients with Friedreich ataxia, DT-168, for Fuchs endothelial corneal dystrophy, and DT-818, for myotonic dystrophy type-1, the company is advancing a program in Huntington's disease. Discovery efforts are underway for multiple genomic medicines. 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: the progression of studies and clinical trials for DT216P2, including the potential for DT-216P2 to emerge as a best-in-disease treatment for patients with FA; plans to advance DT-216P2 towards registrational development with an update to be provided in the fourth quarter of 2026; and projections and expectations arising from early-stage programs, nonclinical data and interim and early-stage clinical data. 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," "capable of," "plans to," "expects," "estimate," "intends," "will," "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 and uncertainties associated with: the data we observe from early clinical and nonclinical studies may impact our clinical development plans; pursuing a biomarker-driven clinical development strategy carries increased risks as there are currently a limited number of approved biomarker-specific therapies; nonclinical development activities and results of nonclinical studies; conducting a clinical trial and patient enrollment and retention, which are affected by many factors, and any difficulties or delays encountered with such clinical trial or patient enrollment or retention may delay or otherwise adversely affect Design's clinical development plans; the process of discovering and developing therapies that are safe and effective for use as human therapeutics and operating as a development stage company; undesirable side effects or other undesirable properties, which could cause Design or regulatory authorities to suspend or discontinue clinical trials and thereby delay or prevent Design's product candidates' development or regulatory approval; Design's ability to develop, initiate or complete nonclinical studies and clinical trials for its product candidates on the timeframe anticipated, or at all; whether promising early research or clinical trials will result in demonstrated safety and/or efficacy in later clinical trials; changes in Design's plans to develop its product candidates; the data results described herein are based on a preliminary analysis of key efficacy and safety data, and such data may change following a more comprehensive review of the data and such data may not accurately reflect the complete results of the trial; reliance on third parties to successfully conduct clinical trials and nonclinical studies; competitive products, which may make any products we develop or seek to develop obsolete or noncompetitive; Design's reliance on third parties, including contract manufacturers and contract research organizations; 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 ability to obtain and maintain intellectual property protection for its product candidates; and Design's ability to recruit and retain key scientific or management personnel. 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 March 31, 2026, as filed with the SEC on April 28, 2026. 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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