Design Therapeutics Announces Initiation of Patient Dosing in Phase 1 Multiple Ascending Dose Trial of DT-818 for Myotonic Dystrophy Type-1

June 30, 2026

GeneTAC® small molecule designed to selectively reduce transcription of the mutant DMPK allele and address the underlying cause of DM1

CARLSBAD, Calif., June 30, 2026 (GLOBE NEWSWIRE) -- Design Therapeutics, Inc. (Nasdaq: DSGN), a clinical-stage biotechnology company developing treatments for serious degenerative genetic diseases, today announced the initiation of patient dosing in its Phase 1 multiple-ascending dose (MAD) clinical trial evaluating DT-818 in adults with myotonic dystrophy type-1 (DM1). DT-818 is a GeneTAC® small molecule designed to selectively reduce transcription of the mutant DMPK allele and address the underlying cause of DM1.

"Initiation of patient dosing with DT-818 marks an important milestone for Design and for the DM1 community," said Pratik Shah, Ph.D., chairperson and chief executive officer of Design Therapeutics. "DT-818 is designed to address the underlying genetic cause of DM1 by selectively reducing transcription of the mutant DMPK allele that gives rise to toxic RNA foci and downstream spliceopathy. We believe DT-818 has the potential to be a best-in-disease treatment for people living with DM1, supported by its differentiated preclinical profile, and we look forward to advancing its clinical development."

The Phase 1 trial is an open-label, MAD study designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamic effects of DT-818 in adults with DM1. Design anticipates reporting data from this study in 2027.

About Myotonic Dystrophy Type-1 and DT-818
DM1 is a monogenic, autosomal dominant, progressive neuromuscular disease that affects skeletal muscle, heart, brain and other organs. The cardinal features include muscle weakness, myotonia (slow muscle relaxation) and early cataracts. In addition, affected individuals often experience cardiac arrhythmias and changes in neuropsychological function. DM1 is caused by a mutation in the DMPK gene and is estimated to affect more than 70,000 people in the United States. DT-818 is a GeneTAC® small molecule designed to address the genetic cause of DM1 by preventing the expression of mutant gene product and consequently of pathogenic nuclear foci. In preclinical studies, DT-818 demonstrated broad tissue distribution, selective targeting of mutant DMPK and robust pharmacodynamic activity, supporting its potential as a best-in-disease treatment approach for people living with DM1.

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: projections from early-stage programs, nonclinical data and early-stage clinical data; the progression or completion of certain development activities; the initiation and progression of studies and clinical trials for DT-216P2, DT-168 and DT-818 and the timing thereof; the anticipated timing for data readouts; the potential attributes and potential best-in-disease profile of DT-818; establishing clinical proof of concept for any product candidate; Design's ability to advance the GeneTAC® platform; and the capabilities and potential advantages of Design's pipeline 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," "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, preclinical data or early clinical results will result in demonstrated safety and/or efficacy in later clinical trials; changes in Design's plans to develop its product candidates; 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 ended 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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