Dyne Therapeutics Receives FDA Fast Track Designation for DYNE-101 for the Treatment of Myotonic Dystrophy Type 1

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- Company Anticipates Submitting for U.S. Accelerated Approval in H1 2026 -

WALTHAM, Mass., Jan. 21, 2025 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage neuromuscular disease company focused on advancing life-transforming therapeutics for people living with genetically driven diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for DYNE-101 for the treatment of myotonic dystrophy type 1 (DM1). DYNE-101 is currently being evaluated in the ongoing Phase 1/2 ACHIEVE global clinical trial.

"This Fast Track designation comes on the heels of robust clinical data from our ACHIEVE trial, which demonstrated substantial functional benefit for patients across a range of clinical measures and a compelling effect on the key disease biomarker of splicing correction," said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. "DM1 is a devastating disease with no approved therapies, and we are driven to deliver DYNE-101, a potentially transformative medicine, to patients as quickly as possible."

The FDA grants Fast Track designation to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need, with the goal of getting important new drugs to patients earlier. A drug that receives Fast Track designation may be eligible for more frequent meetings and communications with the FDA and rolling review of any application for marketing approval. Rolling review allows a company to submit completed sections of its Biologic License Application (BLA) for review by FDA as they are ready, rather than waiting until the entire application is complete for FDA review. This may lead to earlier drug approval and access by patients. A drug receiving Fast Track designation also may be eligible for Accelerated Approval and Priority Review if relevant criteria are met.

About DYNE-101

DYNE-101 is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE clinical trial for people living with DM1. DYNE-101 consists of an antisense oligonucleotide (ASO) conjugated to a fragment antibody (Fab) that binds to the transferrin receptor 1 (TfR1) which is highly expressed on muscle. It is designed to enable targeted muscle tissue delivery with the goal of reducing toxic *DMPK* RNA in the nucleus, releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins, and potentially stopping or reversing the disease progression. DYNE-101 has been granted orphan drug designation by the U.S. Food and Drug Administration and the European Medicines Agency for the treatment of DM1.

About Myotonic Dystrophy Type 1 (DM1)

DM1 is a rare, progressive, genetic disease that affects skeletal, cardiac and smooth muscle. It is a monogenic, autosomal dominant disease caused by an abnormal trinucleotide expansion in a region of the *DMPK* gene. This expansion of CTG repeats causes toxic RNA to cluster in the nucleus, forming nuclear foci and altering the splicing of multiple proteins essential for normal cellular function. This altered splicing, or spliceopathy, results in a wide range of symptoms. People living with DM1 typically experience myotonia and progressive weakness of major muscle groups, which can affect mobility, breathing, heart function, speech, digestion and vision as well as cognition. DM1 is estimated to affect more than 40,000 people in the United States and over 74,000 people in Europe, but there are currently no approved disease-modifying therapies.

About Dyne Therapeutics

Dyne Therapeutics is focused on discovering and advancing innovative life-transforming therapeutics for people living with genetically driven neuromuscular diseases. Leveraging the modularity of its FORCE™ platform, Dyne is developing targeted therapeutics that are designed to overcome limitations in delivery to muscle tissue and the central nervous system (CNS). Dyne has a broad pipeline for neuromuscular diseases, including clinical programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD) and preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease. For more information, please visit https://www.dyne-tx.com/, and follow us on X, LinkedIn and Facebook.

Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release, including statements regarding Dyne's strategy, future operations, prospects and plans, objectives of management, the potential of the FORCE platform, the anticipated timeline for reporting data from the DYNE-101 ACHIEVE clinical trial and expectations regarding the potential benefits of fast track designation, and expectations regarding the timing and outcome of interactions with global regulatory authorities and the availability of accelerated approval pathways for DYNE-101 and expectations regarding the timing of filing applications for U.S. Accelerated Approval constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Dyne may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; the timing of and Dyne's ability to enroll patients in clinical trials; whether results from preclinical studies and initial data from early clinical trials will be predictive of the final results of the clinical trials or future trials; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from Dyne's clinical trials and acceptance of Dyne's clinical programs and the regulatory approval process; fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process; whether Dyne's cash resources will be sufficient to fund its foreseeable and

unforeseeable operating expenses and capital expenditure requirements; as well as the risks and uncertainties identified in Dyne's filings with the Securities and Exchange Commission (SEC), including Dyne's most recent Form 10-Q and in subsequent filings Dyne may make with the SEC. In addition, the forward-looking statements included in this press release represent Dyne's views as of the date of this press release. Dyne anticipates that subsequent events and developments will cause its views to change. However, while Dyne may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Dyne's views as of any date subsequent to the date of this press release.

Contacts:

Investors
Mike Hencke
Kendall Investor Relations
ir@dyne-tx.com

Media
Stacy Nartker
Dyne Therapeutics
snartker@dyne-tx.com
781-317-1938