Dyne Therapeutics Announces U.S. FDA Clearance of Investigational New Drug (IND) Application for DYNE-302 in Facioscapulohumeral Muscular Dystrophy (FSHD)

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- Company's third program to enter clinical development leveraging FORCE platform with the goal of delivering functional improvement in rare neuromuscular disease -

WALTHAM, Mass., July 28, 2026 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage company focused on delivering functional improvement for people living with genetically driven neuromuscular diseases, today announced that it has received clearance from the U.S. Food and Drug Administration (FDA) for its IND application to initiate a Phase 1 clinical trial for DYNE-302 in facioscapulohumeral muscular dystrophy (FSHD). DYNE-302 leverages the same FORCE™ platform as Dyne's first two clinical programs, zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251) in exon 51 Duchenne muscular dystrophy (DMD) and zeleciment basivarsen (z-basivarsen, also known as DYNE-101) in myotonic dystrophy type 1 (DM1).

"By leveraging the advantages of our FORCE platform, we aim to develop an impactful therapy to address the significant medical needs of individuals living with FSHD," said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. "The broad tissue distribution and unique binding characteristics of our TfR1-targeting Fab and siRNA observed in preclinical studies bolster our belief that DYNE-302 has the potential for a differentiated profile."

FSHD is a rare, progressive, inherited muscle disease with no approved therapies. De-repression of *DUX4* in skeletal muscle drives disease pathogenesis, leading to muscle damage and loss of function. This results in a range of symptoms that restrict daily activities and have a high physical, emotional and financial burden. DYNE-302 is designed to leverage a TfR1-targeting Fab for muscle delivery of an siRNA payload highly specific for *DUX4* mRNA with the aim of suppressing *DUX4* expression and the downstream DUX4 transcriptome.

In preclinical models of FSHD, administration of DYNE-302 resulted in robust knockdown of the DUX4 transcriptome in skeletal muscle, significant reversal of muscle fiber damage and functional improvement in a severe disease model. These findings suggest that preexisting skeletal muscle damage in FSHD has the potential to be reversed by targeting *DUX4* mRNA with DYNE-302.

Phase 1 Trial Details
Dyne plans to evaluate DYNE-302 in a Phase 1 randomized, placebo-controlled, double-blind, multiple ascending dose (MAD) clinical trial in ambulatory adult individuals with FSHD. The primary endpoint will be safety and tolerability. The trial will also assess pharmacokinetics and pharmacodynamics, including change from baseline in muscle DUX4 transcriptome and plasma KHDC1L levels, which Dyne has independently identified as a DUX4-regulated biomarker in FSHD.

In the first cohort, 9 participants will receive three intravenous doses administered every four weeks (Q4W), randomized 2:1 to DYNE-302 1.5 mg/kg (approximate siRNA dose) or placebo. Following the completion of this cohort, Dyne intends to evaluate higher dosing and less frequent administration.

Participants who complete the placebo-controlled period may enter an open-label long-term extension and receive DYNE-302 for up to an additional 96 weeks.

Dyne intends to pursue a traditional approval pathway in the U.S. for DYNE-302.

About DYNE-302
DYNE-302 is an investigational therapeutic for people living with FSHD. DYNE-302 consists of an antigen-binding fragment (Fab) that binds to the transferrin receptor 1 (TfR1), conjugated to an siRNA designed to reduce *DUX4* expression. Dyne has generated extensive preclinical data that demonstrate robust and durable *DUX4* suppression and functional improvement in a preclinical *in vivo* model of FSHD developed by Dyne.

About Facioscapulohumeral Muscular Dystrophy (FSHD)
FSHD is a rare, progressive, genetic disease caused by a mutation in the *DUX4* gene, leading to skeletal muscle loss, muscle weakness and wasting. Individuals with FSHD carry a genetic mutation that allows the *DUX4* gene to be sporadically activated in muscle cells, causing their gradual destruction throughout the body. People living with FSHD experience weakness in all major muscle groups throughout the body and limited mobility. An estimated 15,000 to 40,000 individuals in the United States and approximately 20,000 to 50,000 in Europe are affected by FSHD, but there are currently no approved therapies.

About Dyne Therapeutics
Dyne Therapeutics is focused on delivering functional improvement for people living with genetically driven neuromuscular diseases. We are developing therapeutics that target muscle and the central nervous system (CNS) to address the root cause of disease. The company is advancing clinical programs for Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1) as well as preclinical programs for facioscapulohumeral muscular dystrophy (FSHD), Pompe disease and multiple DMD mutations. At Dyne, we are on a mission to deliver functional improvement for individuals, families and communities. Learn more at 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 clinical potential of DYNE-302 and the potential for DYNE-302 to provide broad tissue distribution and exhibit unique binding characteristics, expectations regarding the initiation of a Phase 1 clinical trial of DYNE-302, including its design, timing and potential outcomes, and expectations regarding potential future regulatory interactions and strategies, 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,” “will” 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 preliminary data from clinical trials will be predictive of the final results of the clinical trials or other trials; whether data from clinical trials will support submission for regulatory approvals; 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 as to the regulatory approval process for Dyne’s product candidates; 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 the company’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.

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