Dyne Therapeutics Announces Initiation of Phase 3 FORZETTO Trial of Z-Rostudirsen in Duchenne Muscular Dystrophy (DMD) Ahead of Planned BLA Submission for U.S. Accelerated Approval

May 20, 2026

- 72-week trial will enroll approximately 90 participants; first site now open for enrollment -

- Primary endpoint is rise from floor (RFF) velocity with multiple secondary endpoints to assess muscle and pulmonary function -

- FORZETTO trial design and protocol aligned with FDA; trial intended to serve as confirmatory trial for traditional approval in the U.S. and support ex-U.S. marketing applications -

FORZETTO

FORZETTO trial for z-rostudirsen for individuals with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping

WALTHAM, Mass., May 20, 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 the initiation of the Phase 3 FORZETTO trial of zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251), in individuals with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The design of the FORZETTO trial will be presented at the 19th International Congress on Neuromuscular Diseases (ICNMD) being held July 7-11, 2026, in Florence, Italy.

“As we approach the planned submission of our application for Accelerated Approval in the U.S. later this quarter based on the unprecedented results seen in the DELIVER trial, we are pleased to have initiated a Phase 3 trial designed to further demonstrate the potential of z-rostudirsen to enable functional improvement for people with DMD amenable to exon 51 skipping,” said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. “Z-rostudirsen is designed to enable the production of near-full length dystrophin across muscle tissues and the central nervous system in order to improve how DMD patients feel and function. By assessing multiple measures of mobility, lung health and patient-reported outcomes, we aim to demonstrate the breadth of potential benefits of z-rostudirsen.”

FORZETTO is a global, randomized, placebo-controlled, double-blind, confirmatory Phase 3 trial designed to assess the efficacy, safety, and tolerability of z-rostudirsen administered intravenously to ambulatory male participants with DMD amenable to exon 51 skipping. The trial will enroll approximately 90 participants 4 to 18 years of age who will be randomized 1:1 to receive 20 mg/kg of z-rostudirsen or placebo every four weeks (Q4W). The first trial site is activated and open to enrollment.

The primary endpoint is the change from baseline in rise from floor (RFF) velocity, also referred to as time to rise (TTR) velocity, at Week 73. RFF velocity is a clinically meaningful measure of muscle strength and motor coordination commonly assessed in DMD clinical trials. In the registrational expansion cohort of the Phase 1/2 DELIVER trial, treatment with z-rostudirsen 20 mg/kg Q4W (n=21) led to an improvement in RFF velocity at 6 months of 0.04 rise/sec compared to pooled placebo (n=18; nominal $p<0.05$)[1], exceeding the published minimal clinically important difference (MCID)[2], along with a favorable safety profile.[3]

Secondary endpoints in the FORZETTO trial include changes from baseline in stride velocity 95th centile (SV95C), North Star Ambulatory Assessment (NSAA) total score, 10-meter walk/run (10MWR) velocity, four-stair climb (4SC) velocity and forced vital capacity percent predicted (FVC%p), as well as additional functional and patient-reported outcome measures. Following the 72-week double-blind placebo-controlled treatment period, participants will be eligible to enroll in a 96-week open-label long-term extension.

Dyne has aligned with the U.S. Food and Drug Administration (FDA) on the FORZETTO Phase 3 trial design and protocol. FORZETTO is intended to serve as a confirmatory trial to support the potential conversion of Accelerated Approval to traditional approval in the U.S. and to support ex-U.S. marketing applications.

Informed by the Duchenne community, the FORZETTO Phase 3 study design reflects the company's commitment to patient-centered drug development, with the goal of delivering functional improvement for people living with genetically driven neuromuscular diseases.

Poster Presentation Details
Abstract Title: FORZETTO, a Phase 3 Study of Z-Rostudirsen in Ambulatory Males with Exon 51 Amenable DMD
Poster Session Date and Time: Wednesday, July 8, 2026, 12:45 p.m. – 2:30 p.m. CEST (6:45 a.m. – 8:30 a.m. ET)

The poster presentation will be available in the Scientific Publications & Presentations section of Dyne’s website at the commencement of the poster session.

About the FORZETTO Trial
FORZETTO is a global, randomized, placebo-controlled, double-blind, confirmatory Phase 3 clinical trial evaluating the efficacy, safety and tolerability of zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251) in ambulatory male participants with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The trial will enroll approximately 90 participants 4 to 18 years of age who will receive 20 mg/kg of z-rostudirsen or placebo once every four weeks for 72 weeks. Participants who complete the placebo-controlled period may enter a long-term extension and receive 20 mg/kg of z-rostudirsen once every four weeks for up to an additional 96 weeks. The primary endpoint of FORZETTO is the change from baseline in rise from floor (RFF) velocity at Week 73. RFF velocity is a clinically meaningful measure of muscle strength and motor coordination commonly assessed in DMD clinical trials. Secondary endpoints include changes from baseline in stride velocity 95th centile (SV95C), North Star Ambulatory Assessment (NSAA) total score, 10-meter walk/run (10MWR) velocity, four-stair climb (4SC) velocity and forced vital capacity percent predicted (FVC%p), as well as additional functional and patient-reported outcome measures.

About Zeleciment Rostudirsen (z-rostudirsen, also known as DYNE-251)
Z-rostudirsen is an investigational therapeutic for individuals with DMD who have mutations in the *DMD* gene that are amenable to exon 51 skipping. The registrational expansion cohort of the global Phase 1/2 DELIVER clinical trial of z-rostudirsen met its primary endpoint. Data from the DELIVER trial will serve as the basis for a planned Biologics License Application (BLA) for potential U.S. Accelerated Approval. Z-rostudirsen continues to be evaluated in the long-term extension portion of the DELIVER trial and in the global confirmatory Phase 3 FORZETTO clinical trial. Z-rostudirsen consists of a phosphorodiamidate morpholino oligomer (PMO) conjugated to an antigen-binding fragment (Fab) that binds to the transferrin receptor 1 (TfR1). It is designed to enable the production of near full-length dystrophin in muscle and the central nervous system (CNS) to provide functional improvement. Z-rostudirsen has received Breakthrough Therapy, Fast Track and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA), as well as Orphan Drug designation from the FDA, European Medicines Agency (EMA) and the Ministry of Health, Labour and Welfare (MHLW) in Japan for the treatment of individuals with DMD amenable to exon 51 skipping.

In addition to z-rostudirsen, Dyne is building a DMD franchise and has preclinical programs targeting other exons, including DYNE-253, DYNE-245, DYNE-244 and DYNE-255.

About Duchenne Muscular Dystrophy (DMD)
Duchenne muscular dystrophy (DMD) is a rare X-linked progressive neuromuscular disorder caused by mutations in the *DMD* gene. These mutations result in a complete or near-complete absence of dystrophin, a protein critical for maintaining muscle structure and function. DMD is the most common form of childhood-onset muscular dystrophy, affecting approximately 12,000 individuals in the U.S. and 16,000 in the EU. Symptoms typically emerge between ages 3 and 5, beginning with muscle weakness in the upper arms, thighs and pelvic region, and progressively impacting the lower limbs, forearms, neck and trunk. In addition to physical decline, individuals may experience cognitive impairment and neuropsychiatric challenges such as intellectual disabilities, learning difficulties and behavioral disorders. Despite existing therapies, there remains a significant unmet need for new treatment options that deliver functional improvement.

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 zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251), expectations regarding the timing of submitting applications for U.S. Accelerated Approval, expectations regarding the timing and outcome of interactions with global regulatory authorities and the availability of expedited approval pathways for z-rostudirsen, and the ability of Dyne's FORCE platform to produce near full-length dystrophin across muscle tissues as well as the central nervous system, 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; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from Dyne's clinical trials and the regulatory 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 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.

1. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint.
2. Duong et al. *J Neuromusc Dis.* 2021; 8(6):939-948; MCID = 0.023 rise/sec.
3. Z-rostudirsen safety data as of August 19, 2025.

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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/a9bc6e9f-f2a5-4129-978f-751f41a83728