Source: *Dyne Therapeutics, Inc.*

July 20, 2026 07:30 ET

Dyne Therapeutics Announces U.S. FDA Acceptance of Biologics License Application (BLA) for Z-Rostudirsen in Exon 51 Duchenne Muscular Dystrophy (DMD)

- Priority Review granted; PDUFA target action date set for January 21, 2027 -

- Submission for Accelerated Approval based on dystrophin as a surrogate endpoint -

- In the registrational expansion cohort of the DELIVER trial, treatment with z-rostudirsen once every 4 weeks resulted in a robust and statistically significant increase in dystrophin production with functional improvement observed across multiple clinical endpoints and a favorable safety profile[1] -

WALTHAM, Mass., July 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 that the U.S. Food and Drug Administration (FDA) has accepted for review the Biologics License Application (BLA) for zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251) for the treatment of individuals with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The FDA has granted the BLA Priority Review and assigned a Prescription Drug User Fee Act (PDUFA) target action date of January 21, 2027. Dyne continues to expect a potential U.S. launch of z-rostudirsen in Q1 2027, assuming approval is received on the anticipated timeline.

“This milestone represents significant progress toward our goal of delivering functional improvement for those living with DMD amenable to exon 51 skipping,” said John Cox, president and chief executive officer of Dyne. “With z-rostudirsen, we set out to advance the treatment paradigm in DMD by combining a robust increase in near-full length dystrophin with broad delivery to relevant tissues. With a potential approval in six months, we are continuing our launch

preparations with the aim of making z-rostudirsen available as quickly as possible. We are deeply grateful to the entire Duchenne community, whose partnership and support have been essential in developing this potential therapy."

In addition to z-rostudirsen, Dyne is advancing four development candidates (DYNE-253, DYNE-245, DYNE-244 and DYNE-255) for the potential treatment of DMD amenable to skipping of exons 53, 45, 44, and 55, respectively.

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 served as the basis for a Biologics License Application (BLA) for potential U.S. Accelerated Approval, which has been granted Priority Review. The FDA grants Priority Review to applications for medicines that, if approved, provide significant improvements in the safety or effectiveness of the treatment of a serious condition. 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 and include progressive muscle weakness, loss of lower and upper limb function and eventually cardiac and respiratory failure. 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 and therapeutic potential of zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251), the potential of z-rostudirsen to advance the treatment paradigm in Duchenne muscular dystrophy, expectations regarding the timing and outcome of interactions with regulatory authorities and the timing of regulatory authority action, and expectations regarding the timing of commercialization of z-rostudirsen, 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; 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. Z-rostudirsen safety data as of August 19, 2025.

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