Dyne Therapeutics Receives Orphan Drug Designation in Japan for Zeleciment Basivarsen (DYNE-101) for Myotonic Dystrophy Type 1

January 20, 2026

- Zeleciment basivarsen (z-basivarsen) demonstrated sustained functional improvement across multiple clinical measures in the ongoing ACHIEVE clinical trial -

- Expect to complete enrollment in ACHIEVE Registrational Expansion Cohort in early Q2 2026 -

WALTHAM, Mass., Jan. 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 Ministry of Health, Labour and Welfare (MHLW) in Japan has granted Orphan Drug designation for zeleciment basivarsen (z-basivarsen), for the treatment of myotonic muscular dystrophy type 1 (DM1). Z-basivarsen is currently being evaluated in the Phase 1/2 ACHIEVE clinical trial in individuals with DM1.

“By targeting the underlying biology of DM1, z-basivarsen has shown early and sustained improvements in myotonia, muscle strength and function, with a favorable safety profile,” said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. “This designation in Japan, alongside those already granted in the U.S. and Europe, emphasizes the urgent need for new therapies and highlights the potential of z-basivarsen to deliver meaningful functional improvement for people living with DM1.”

In Japan, Orphan Drug designation is granted to drugs intended for the treatment of rare diseases affecting fewer than 50,000 patients in the country and for which there is a high medical need. Benefits include subsidies for development costs and potential market exclusivity for up to 10 years if approved. Z-basivarsen has also been granted Breakthrough Therapy, Fast Track and Orphan Drug designations from the U.S. Food and Drug Administration (FDA), and Orphan Drug designation from the European Medicines Agency (EMA) for the treatment of individuals with DM1.

About the ACHIEVE Trial
ACHIEVE is a global, randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of zeleciment basivarsen (z-basivarsen, formerly known as DYNE-101) in patients with myotonic dystrophy type 1 (DM1). The multiple ascending dose (MAD) portion of the study resulted in the selection of a registrational dose and regimen of 6.8 mg/kg z-basivarsen administered every eight weeks. A registrational expansion cohort has been initiated to support potential regulatory submissions, including Accelerated Approval in the U.S. The primary endpoint for this cohort is the change from baseline in middle finger myotonia as measured by video hand opening time (vHOT) at 6 months, compared to placebo. For more information on the ACHIEVE trial, visit www.clinicaltrials.gov and euclinicaltrials.eu.

About zeleciment basivarsen (z-basivarsen, formerly known as DYNE-101)
Z-basivarsen is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE clinical trial for people living with DM1. Z-basivarsen consists of an antisense oligonucleotide (ASO) conjugated to an antigen-binding fragment (Fab) that binds to the transferrin receptor 1 (TfR1) to enable delivery to muscle and the central nervous system. It is designed to deliver functional improvement in individuals living with DM1 by reducing toxic nuclear DMPK RNA to release splicing proteins and allow normal mRNA processing. Z-basivarsen has been granted Breakthrough Therapy, Orphan Drug and Fast Track designations by the U.S. Food and Drug Administration and Orphan Drug designation by the European Medicines Agency for the treatment of DM1.

About Myotonic Dystrophy Type 1 (DM1)
Myotonic dystrophy type 1 (DM1) is a rare, progressive, genetic neuromuscular disease with high morbidity and early mortality. DM1 affects ~40,000 people in the U.S. and ~55,000 people in the EU. The severity of symptoms and rate of progression varies. Symptoms can begin at any point in an affected person’s life, depending on the DM1 subtype. Adult-onset DM1 symptoms typically appear between 20 to 40 years of age. DM1 is caused by mutations in the DMPK gene, leading to a widespread disruption of RNA splicing, known as spliceopathy, which drives the multi-system manifestations of the disease. People experience a broad spectrum of symptoms, including: muscle weakness throughout the body, myotonia or difficulty relaxing muscles, excessive daytime sleepiness, fatigue, dysregulated sleep, cognitive impairments, cardiac arrhythmias, respiratory issues and gastrointestinal dysfunction. Although the genetic cause of DM1 is well understood, there are currently no approved disease-modifying treatments for DM1.

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), and preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease. 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 zeleciment basivarsen (z-basivarsen, also known as DYNE-101); the anticipated timelines for completing enrollment of the registrational expansion cohort of the ACHIEVE clinical trial; submitting applications for marketing approval and commercial launches; and expectations regarding the timing and outcome of interactions with regulatory authorities, including whether Dyne will realize the anticipated benefits of orphan drug designation for z-basivarsen in Japan, 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 timing of and Dyne's ability to enroll patients in clinical trials; whether results from preclinical studies and 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, debt service obligations 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.

Contacts:

Investors
Mia Tobias
ir@dyne-tx.com
781-317-0353

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