Dyne Therapeutics Receives Orphan Drug Designation in Japan for DYNE-251 in Duchenne Muscular Dystrophy

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- Data from ongoing DELIVER trial demonstrated sustained functional improvement through 18 months; results from Registrational Expansion Cohort expected late 2025 -

WALTHAM, Mass., Sept. 29, 2025 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage company, today announced that the Ministry of Health, Labour and Welfare (MHLW) in Japan has granted Orphan Drug designation for DYNE-251 in individuals with Duchenne muscular dystrophy (DMD) who have mutations in the *DMD* gene that are amenable to exon 51 skipping. DYNE-251 is being evaluated in the ongoing Phase 1/2 DELIVER global clinical trial.

“In the DELIVER trial, we’ve seen unprecedented and sustained functional improvement, driven by significant dystrophin expression, underscoring the potential of DYNE-251 to transform the care of people living with Duchenne muscular dystrophy,” said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. “With orphan drug designation now granted in Japan, complementing existing designations in the U.S. and Europe, DYNE-251 continues to advance as a promising next-generation exon 51 skipping therapy. Individuals with mutations amenable to exon 51 skipping are among the most prevalent and functionally impacted in the DMD population. Potentially addressing this unmet need further fuels our commitment to develop a therapy that delivers functional improvement for the Duchenne community, and we look forward to collaborating with the Japanese regulators to determine the regulatory pathway for DYNE-251 in Japan.”

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. DYNE-251 has also been granted Breakthrough Therapy designation, Fast Track and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA), and Orphan Drug designation from the FDA and European Medicines Agency (EMA) for the treatment of individuals with DMD, amenable to exon 51 skipping.

About the DELIVER Trial

DELIVER is a global, randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy (as measured by both biomarker and functional improvement) of DYNE-251 in individuals with Duchenne muscular dystrophy (DMD) who have mutations in the *DMD* gene that are amenable to exon 51 skipping. The multiple ascending dose (MAD) portion of the study resulted in the selection of a registrational dose and regimen of 20 mg/kg of DYNE-251 administered every four weeks. A registrational expansion cohort to support a potential regulatory submission for U.S. Accelerated Approval is fully enrolled. The primary endpoint for this cohort is the change from baseline in dystrophin protein levels as measured by Western blot at 6 months. For more information on the DELIVER trial, visit clinicaltrials.gov (NCT05524883) and euclinicaltrials.eu (2023-510351-31-00).

About DYNE-251

DYNE-251 is an investigational therapeutic being evaluated in the Phase 1/2 global DELIVER clinical trial for individuals with DMD who have mutations in the *DMD* gene that are amenable to exon 51 skipping. DYNE-251 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. DYNE-251 has received Breakthrough Therapy designation, 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 and European Medicines Agency (EMA) for the treatment of individuals with DMD, amenable to exon 51 skipping.

In addition to DYNE-251, Dyne is building a DMD franchise and has preclinical programs targeting other exons, including 53, 45 and 44.

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 myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD), 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 DYNE-251; the anticipated timelines for reporting additional data from the DELIVER 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 DYNE-251 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.

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