Dyne Therapeutics Receives European Medicines Agency (EMA) Orphan Drug Designation for DYNE-251 in Duchenne Muscular Dystrophy

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- Recently presented data demonstrated sustained functional improvement with DYNE-251 treatment through 18 months -

- Data from the fully enrolled DELIVER registrational expansion cohort is planned for late 2025 -

WALTHAM, Mass., April 24, 2025 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage company focused on advancing life-transforming therapeutics for people living with genetically driven neuromuscular diseases, today announced that the European Commission (EC) has granted orphan drug designation for DYNE-251 for the treatment of Duchenne muscular dystrophy (DMD). DYNE-251 is being evaluated in the Phase 1/2 DELIVER global clinical trial in individuals with DMD who are amenable to exon 51 skipping. Long-term clinical data from the ongoing DELIVER trial of DYNE-251 that demonstrated unprecedented and sustained functional improvement at the selected registrational dose were presented in March at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference. Functional assessments in the DELIVER trial include Stride Velocity 95th Centile (SV95C), an objective digital outcome that is accepted as a primary endpoint for DMD clinical trials in Europe.

“Our recent long-term DELIVER trial results demonstrated clinically relevant and sustained functional improvement through 18 months, including as assessed by SV95C, which may support a strong rationale for regulatory approval in Europe,” said Doug Kerr, MD, PhD, chief medical officer of Dyne. “We are pleased that the EC has granted orphan drug designation to DYNE-251, reinforcing our belief that our next-generation exon 51 skipping investigational therapy for DMD may be able to bring clinically meaningful functional improvement to those living with this devastating disease. With full enrollment of the registrational expansion cohort in the DELIVER trial complete, we look forward to sharing data from this cohort in late 2025 and the potential to move forward with our first regulatory submissions in early 2026.”

The EC grants orphan drug designation to drugs and biologics intended for the treatment, diagnosis or prevention of rare, life-threatening or chronically debilitating diseases or conditions that affect fewer than five in 10,000 people in the European Union (EU). Orphan designation provides companies with certain benefits, including reduced regulatory fees, clinical protocol assistance, research grants and the potential for up to 10 years of market exclusivity in the EU if approved. DYNE-251 was also granted U.S. Food and Drug Administration (FDA) orphan drug and rare pediatric disease designations in March 2023.

Key Milestones for the DELIVER Trial

- Dyne continues to pursue expedited approval pathways globally for DYNE-251 in patients with DMD who are amenable to exon 51 skipping.
- Dyne has fully enrolled the registrational expansion cohort of 32 patients as part of the DELIVER trial. Data from this cohort are planned for late 2025.
- Dyne anticipates filing a Biologics License Application (BLA) submission for US accelerated approval in early 2026.

About the DELIVER Trial

DELIVER is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of DYNE-251 in patients with Duchenne muscular dystrophy (DMD) who 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 every four weeks. A registrational expansion cohort to support potential regulatory submissions for expediated approvals, including accelerated approval in the U.S., is fully enrolled. The primary endpoint for this cohort is the change from baseline in dystrophin protein levels as measured by Western blot. 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 people living with DMD who are amenable to exon 51 skipping. DYNE-251 consists of a phosphorodiamidate morpholino oligomer (PMO) conjugated to a fragment antibody (Fab) that binds to the transferrin receptor 1 (TfR1), which is highly expressed on muscle. It is designed to enable targeted muscle tissue delivery and promote exon skipping in the nucleus, allowing muscle cells to create internally shortened, near full-length dystrophin protein, with the goal of stopping or reversing disease progression. DYNE-251 has been granted fast track, orphan drug and rare pediatric disease designations by the U.S. Food and Drug Administration for the treatment of DMD mutations amenable to exon 51 skipping.

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

About Duchenne Muscular Dystrophy (DMD)

DMD is a rare disease caused by mutations in the gene that encodes for dystrophin, a protein critical for the normal function of muscle cells. These mutations, the majority of which are deletions, result in the lack of dystrophin protein and progressive loss of muscle function. DMD occurs primarily in males and affects an estimated 12,000 to 15,000 individuals in the U.S. and 25,000 in Europe. Loss of strength and function typically first appears in pre-school age boys and worsens as they age. As the disease progresses, the severity of damage to skeletal and cardiac muscle often results in patients experiencing total loss of ambulation by their early teenage years and includes worsening cardiac and respiratory symptoms and loss of upper body function by the later teens. There is no cure for DMD, and currently approved therapies provide limited benefit.

About Dyne Therapeutics

Dyne Therapeutics is discovering and advancing innovative life-transforming therapeutics for people living with genetically driven neuromuscular diseases. Leveraging the modularity of its FORCE™ platform, Dyne is developing targeted therapeutics that deliver to muscle and the central nervous system (CNS). Dyne has a broad pipeline for neuromuscular diseases, including clinical programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD), and preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease. For more information, please visit 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 therapeutic potential of DYNE-251, the anticipated timeline for reporting additional data from the DELIVER clinical trial, the availability of expedited approval pathways for DYNE-251, expectations regarding the timing and outcome of interactions with regulatory authorities, and expectations regarding the timing of submitting applications for U.S. Accelerated Approval and other regulatory approvals, 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," 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 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-K 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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