Dyne Therapeutics Announces FDA Breakthrough Therapy Designation for DYNE-251 in Duchenne Muscular Dystrophy (DMD)

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- Data from the DELIVER registrational expansion cohort is expected in late 2025, with a potential BLA submission for U.S. accelerated approval anticipated in early 2026 -

WALTHAM, Mass., Aug. 04, 2025 (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 granted Breakthrough Therapy Designation to DYNE-251 for the treatment of patients with Duchenne muscular dystrophy (DMD), amenable to exon 51 skipping. The designation is based on data from the ongoing DELIVER clinical trial.

"This Breakthrough Therapy Designation for DYNE-251 is a testament to its potential as a next-generation therapy designed to bring meaningful functional improvement to individuals with DMD for whom exon 51 skipping can lead to the production of near full-length dystrophin," said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. "As we've previously disclosed, DYNE-251 has demonstrated sustained functional improvement through eighteen months, as assessed by key measures such as time to rise and stride velocity 95th centile. The level of near-full-length dystrophin expression observed marks a significant step forward to potentially deliver meaningful benefits to patients."

Both of Dyne's lead programs now have FDA Breakthrough Therapy Designation, with DYNE-101 having been granted Breakthrough Therapy Designation in myotonic dystrophy type 1 (DM1) earlier this year. The FDA grants Breakthrough Therapy Designation to expedite the development and review of drugs that are intended to treat a serious condition with preliminary clinical evidence indicating that the drug may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints. This designation offers benefits to DYNE-251 in the U.S. including:

- Enhanced FDA support, including senior-level involvement to guide efficient development as well as decision-making
- Early and frequent communication with FDA reviewers on trial design and regulatory strategy
- Rolling and Priority Review eligibility, potentially reducing the Biologics License Application (BLA) review timeline from 12 to 8 months

DYNE-251 in DMD

- DYNE-251 has been granted Fast Track, Orphan Drug and Rare Pediatric disease designations by the FDA and Orphan Drug designation by the European Medicines Agency (EMA) for the treatment of DMD.
- Dyne has completed enrollment of 32 patients in the Registrational Expansion Cohort of the DELIVER trial. Data from this cohort are planned for late 2025.
- Dyne anticipates a potential BLA submission for U.S. Accelerated Approval in early 2026.
- Dyne also continues to pursue approval pathways outside of the U.S. for DYNE-251 in patients with DMD with a confirmed mutation 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 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.

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, X-linked, recessive, progressive neuromuscular disorder and results from mutations in the *DMD* gene which lead to total absence or nearly undetectable levels of dystrophin protein, essential for muscle structure, function and preservation. DMD occurs primarily in males and affects approximately 12,000 individuals in the U.S. and approximately 16,000 in the EU. 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 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 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; the availability of expedited approval pathways for DYNE-251; and expectations regarding the timing and outcome of interactions with regulatory authorities, 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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