Dyne Therapeutics Receives FDA Fast Track Designation for DYNE-251 for the Treatment of Duchenne Muscular Dystrophy

October 31, 2022

- Company Anticipates Reporting Data from Global, Multiple Ascending Dose DELIVER Clinical Trial in the Second Half of 2023 -

WALTHAM, Mass., Oct. 31, 2022 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for DYNE-251 for the treatment of Duchenne muscular dystrophy (DMD) mutations amenable to exon 51 skipping. DYNE-251 is being evaluated in the Phase 1/2 DELIVER global clinical trial.

“Every day is critical for patients and their families, and we are pleased that the FDA granted Fast Track designation as this provides an opportunity to expedite the development of DYNE-251,” said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. “Duchenne is a fatal disease and available therapies offer limited benefit. We are focused on driving toward meaningful clinical data in our DELIVER trial anticipated in the second half of 2023 and continuing to work closely with the DMD community and the FDA to advance a potentially transformative therapy.”

The FDA grants Fast Track designation to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need, with the ultimate goal of getting important new drugs to patients earlier. A drug that receives Fast Track designation may be eligible for more frequent meetings and communications with the FDA and rolling review of any application for marketing approval, which may lead to earlier drug approval and access by patients. A drug receiving Fast Track designation also may be eligible for Accelerated Approval and Priority Review if relevant criteria are met.

About DYNE-251

DYNE-251 is Dyne’s product candidate being developed for people living with Duchenne muscular dystrophy (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 a truncated, functional dystrophin protein, with the goal of stopping or reversing disease progression. In preclinical studies with Dyne’s FORCE™ platform, robust and durable exon skipping and dystrophin expression were observed in the*mdx* mouse model in skeletal and cardiac muscle as well as reduced muscle damage and increased muscle function. In non-human primates, DYNE-251 demonstrated a favorable safety profile and achieved impressive exon skipping, especially in the heart and diaphragm, muscles in people living with DMD that weaken over time leading to mortality.

In addition to DYNE-251, Dyne is building a global DMD franchise with preclinical programs for patients with mutations amenable to skipping other exons, including 53, 45 and 44.

About the DELIVER Trial

DELIVER is a Phase 1/2 global clinical trial evaluating DYNE-251, consisting of a 24-week multiple ascending dose (MAD) randomized placebo-controlled period, a 24-week open-label extension and a 96-week long-term extension. The trial, which is designed to be registrational, is expected to enroll approximately 46 ambulant and non-ambulant males with DMD who are ages 4 to 16 and have mutations amenable to exon 51 skipping therapy. The primary endpoints are safety, tolerability and change from baseline in dystrophin levels as measured by Western blot. Secondary endpoints include measures of muscle function, exon skipping and pharmacokinetics. Dyne anticipates reporting data from the MAD placebo-controlled portion of the DELIVER trial on safety, tolerability and dystrophin in the second half of 2023. For more information on the DELIVER trial, visit https://www.clinicaltrials.gov/ (NCT05524883).

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 a clinical-stage muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases. With its proprietary FORCE™ platform, Dyne is developing modern oligonucleotide therapeutics that are designed to overcome limitations in delivery to muscle tissue seen with other approaches. Dyne has a broad portfolio of programs for serious muscle diseases, including candidates for myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD). For more information, please visit https://www.dyne-tx.com/, and follow us on Twitter, 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 anticipated timeline for reporting data from the DYNE-251 DELIVER clinical trial and

expectations regarding the potential benefits of fast track designation 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 initiate and enroll patients in clinical trials; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process; whether Dyne’s cash resources will be sufficient to fund the Company’s foreseeable and unforeseeable operating expenses and capital expenditure requirements; uncertainties associated with the impact of the COVID-19 pandemic on Dyne’s business and operations; 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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