Dyne Therapeutics Receives FDA Orphan Drug and Rare Pediatric Designations for DYNE-251 for the Treatment of Duchenne Muscular Dystrophy

March 23, 2023

- Data from the Global, Multiple Ascending Dose Phase 1/2 DELIVER Clinical Trial Anticipated in the Second Half of 2023 -

WALTHAM, Mass., March 23, 2023 (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 DYNE-251, an investigational therapeutic for Duchenne muscular dystrophy (DMD) mutations amenable to exon 51 skipping, was granted U.S. Food and Drug Administration (FDA) orphan drug and rare pediatric disease designations. DYNE-251 is being evaluated in the Phase 1/2 DELIVER clinical trial.

“These regulatory designations highlight the urgent and critical need for new and better therapeutic options for people living with this fatal disease,” said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. “We are excited about DYNE-251 which we believe has the potential to transform the lives of people with DMD. We continue to advance our DELIVER clinical trial and look forward to sharing initial clinical data later this year.”

Orphan drug designation is granted by the FDA to drugs or biological products intended for treatment, prevention or diagnosis of a rare disease or condition that affects fewer than 200,000 people in the United States. Under the FDA's rare pediatric disease designation program, the FDA may grant a priority review voucher to a sponsor who receives a product approval for a rare pediatric disease.

About DYNE-251 and the DELIVER Trial

DYNE-251 is an investigational therapeutic being evaluated in the Phase 1/2 global DELIVER trial for people living with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. The DELIVER clinical trial consists 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 initial data from the MAD placebo-controlled portion of the DELIVER trial on safety, tolerability and dystrophin in the second half of 2023.

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. DYNE-251 demonstrated a favorable safety profile and achieved robust exon skipping in non-human primates, especially in the heart and diaphragm, muscles in people living with DMD that weaken over time leading to mortality. DYNE-251 was granted Fast Track designation by the U.S. Food and Drug Administration for the treatment of DMD in people who are amenable to exon 51 skipping.

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 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. Dyne has a broad pipeline for serious muscle diseases, including clinical programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD) and a preclinical program for 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 clinical trial and the trial design of the DYNE-251 clinical trial, 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; whether Dyne will benefit from the fast track, orphan drug and rare pediatric disease designations referred to above, 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-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.

Contacts:

Investors
Dyne Therapeutics
Amy Reilly
areilly@dyne-tx.com
857-341-1203

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