# Dyne Therapeutics Announces FDA Clearance of IND Application for DYNE-251 for the Treatment of Duchenne Muscular Dystrophy

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*- Initiation of Dosing in Multiple Ascending Dose Clinical Trial in Patients with Mutations Amenable to Skipping Exon 51 On Track for Mid-2022 -*

WALTHAM, Mass., July 05, 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 lifted the clinical hold and cleared its Investigational New Drug (IND) application to initiate a clinical trial of DYNE-251 in patients with Duchenne muscular dystrophy (DMD) amenable to skipping exon 51. The Company expects to begin dosing patients in a Phase 1/2 clinical trial evaluating DYNE-251 in mid-2022.

“Today marks a significant step in our journey to build a DMD franchise to serve people across the globe with Duchenne mutations amenable to exon skipping. The clearance of our first IND is an important achievement for Dyne, and we appreciate the partnership with the FDA throughout this process. Our team has worked extensively with key opinion leaders, patient advocacy groups and individuals living with DMD to thoughtfully design and execute our global multiple-ascending dose Phase 1/2 clinical trial of DYNE-251,” said Joshua Brumm, president and chief executive officer of Dyne. “We believe we are well-positioned to deliver on our commitment of initiating dosing in both of our DMD and DM1 programs in mid-2022. The entire Dyne team is proud of the progress we have made to advance our mission and address the urgent need to bring new therapeutic options to people living with serious muscle diseases.”

Dyne plans to evaluate DYNE-251 in a global, randomized, placebo controlled, multiple ascending dose (MAD) clinical trial with a long-term extension study. The trial aims to enroll 30 to 50 ambulant and non-ambulant males with Duchenne, who are age 4 to 16 and have mutations amenable to exon 51 skipping therapy. The study will evaluate safety, tolerability, dystrophin expression as measured by Western Blot, pharmacokinetics and pharmacodynamics, and measures of muscle function. Dyne plans to outline additional details regarding the trial design and timing of data upon initiation of dosing.

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-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. DYNE-251 demonstrated a favorable safety profile and achieved impressive exon skipping in non-human primates, especially in the heart and diaphragm, muscles that weaken over time leading to mortality in people living with DMD.

**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 timelines for dosing patients in the DYNE-251 trial and the DYNE-101 trial and the planned trial design of the DYNE-251 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'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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