



Dyne Therapeutics Announces Submission of IND Application to Initiate Clinical Trial of DYNE-251 for Duchenne Muscular Dystrophy

December 2, 2021

- DYNE-251, Dyne's First DMD Program, is Being Developed for Patients with Mutations Amenable to Skipping Exon 51 -

- DYNE-101 IND Submission in DM1 Expected in the First Quarter of 2022 -

- Initiation of Patient Dosing of DYNE-251 and DYNE-101 in Multiple Ascending Dose Clinical Trials Anticipated by Mid-2022 -

WALTHAM, Mass., Dec. 02, 2021 (GLOBE NEWSWIRE) -- [Dyne Therapeutics, Inc.](#) (Nasdaq: DYN), a muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases, today announced the submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) to initiate a clinical trial of DYNE-251 in patients with Duchenne muscular dystrophy (DMD) amenable to skipping exon 51.

"In a little over three years, we've been able to move Dyne from concept to clinic because we are steadfast in our commitment to bring potentially transformational therapies to people living with serious muscle diseases," said Joshua Brumm, president and chief executive officer of Dyne. "The IND submission for DYNE-251 represents a significant milestone. I am so proud of what our team has accomplished, and I want to thank all Dynamos and our partners in the rare muscle disease community. We look forward to an exciting 2022 as we expect to submit an IND for DM1 in the first quarter, begin dosing patients in clinical trials for both our DMD and DM1 programs by mid-year, and submit an IND for our FSHD program in the second half."

The IND application for DYNE-251 includes [in vivo data from recent studies](#) in the *mdx* mouse model and in non-human primates, and outlines plans for a global, randomized, placebo controlled, multiple ascending dose (MAD) clinical trial with a long term extension study. The Phase 1/2 trial aims to enroll approximately 30 to 40 ambulant and non-ambulant male patients ages 4 to 16 with symptomatic DMD and mutations amenable to exon 51 skipping therapy. Planned endpoints include safety and tolerability, PK/PD, dystrophin expression as measured by Western Blot, and measures of muscle function.

In addition to DYNE-251, Dyne is building a DMD franchise with programs for patients with mutations amenable to skipping exons 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 muscles 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 therapeutic 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 1 receptor (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 muscles as well as reduced muscle damage and increased muscle function. DYNE-251 was well tolerated and demonstrated 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 Duchenne.

About Dyne Therapeutics

Dyne Therapeutics is building a leading muscle disease company dedicated to 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 therapeutic candidates for serious muscle diseases, including 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 expected timeline for submitting investigational new drug applications and dosing patients in trials and the anticipated design of the trials, 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 conduct of research activities and the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies; the timing of and Dyne's ability to submit and obtain regulatory clearance for investigational new drug applications and initiate clinical trials; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; whether investigators and regulatory agencies will agree with the design of Dyne's planned 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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