



Dyne Therapeutics Announces First Patient Dosed in Phase 1/2 DELIVER Clinical Trial of DYNE-251 for the Treatment of Duchenne Muscular Dystrophy

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- Data from Global, Multiple Ascending Dose DELIVER Trial Anticipated in the Second Half of 2023 -

- Dyne to Host "Spotlight on the Clinic" Virtual Event on September 12 -

WALTHAM, Mass., Sept. 06, 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 first patient has been dosed in its Phase 1/2 clinical trial, DELIVER, evaluating DYNE-251 for the treatment of Duchenne muscular dystrophy (DMD) mutations amenable to exon 51 skipping.

"We are excited to bring our FORCE™ platform to the clinic for the first time with the initiation of patient dosing in the DELIVER trial. This is a significant milestone for Dyne and our efforts to build a global franchise of DMD exon skipping therapies. We have designed the DELIVER trial with the potential to be registrational and are focused on driving toward meaningful clinical data, including dystrophin, to understand the potential of DYNE-251 in DMD. We thank the Duchenne community for their continued partnership as we work to expand the DELIVER trial globally, and advance toward our ultimate goal of developing new, transformative therapeutic options for individuals living with this devastating disease," said Wildon Farwell, M.D., MPH, chief medical officer of Dyne.

The DELIVER trial 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.

"There is a significant need for new therapies for people living with Duchenne. The comprehensive preclinical data supporting DYNE-251 demonstrate the magnitude of dystrophin expression in cardiac and skeletal muscle. We look forward to being a part of the DELIVER trial to understand the potential of DYNE-251," said Kevin Flanigan, M.D., Robert F. and Edgar T. Wolfe Foundation Endowed Chair in Neuromuscular Research and director of the Center for Gene Therapy in The Research Institute at Nationwide Children's Hospital in Columbus, Ohio.

In the MAD placebo-controlled portion of the DELIVER trial, patients will be randomized to receive DYNE-251 or placebo every four weeks intravenously based on a global protocol designed to incorporate feedback from multiple regulatory authorities, including on starting dose. Patient cohorts will be dosed from 0.7 mg/kg to 40 mg/kg (approximate PMO dose) in the U.S. Outside the U.S., starting doses and number of cohorts will vary by region. Following the placebo-controlled period, patients will transition to DYNE-251 treatment in the open-label portion of the trial and in the long-term extension.

Visit <https://www.clinicaltrials.gov/> (NCT05524883) to learn more about the DELIVER trial.

Virtual Event on September 12, 2022

Dyne will host a "Spotlight on the Clinic" virtual event on Monday, September 12, 2022 from 7:30-9:00 a.m. ET. The Company will review its pipeline with a focus on the clinical programs and opportunity for DYNE-101 in myotonic dystrophy type 1 (DM1) and DYNE-251 in DMD and be joined by leading neuromuscular disease experts. The event will feature presentations, discussion and Q&A with the following speakers:

- Valeria Sansone, M.D., Ph.D., Clinical and Scientific Director at Clinical Center NeMO, Milan; Professor of Neurology, University of Milan
- Richard Finkel, M.D., Director, Center for Experimental Neurotherapeutics, St. Jude Children's Research Hospital, Memphis, TN
- Joshua Brumm, President and Chief Executive Officer
- Wildon Farwell, M.D., MPH, Chief Medical Officer

A live webcast of the event will be available in the Events & Presentations page of the Investors & Media section of Dyne's website and a replay will be accessible for 90 days following the presentation. An accompanying slide presentation will also be available. To register for the live webcast and replay, please visit <https://investors.dyne-tx.com/news-and-events/events-and-presentations>.

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 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 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 trial design of the DYNE-251 trial, and the planned timeline for reporting data for 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.

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