Dyne Therapeutics Announces Presentations on its DM1 and DMD Programs at the 2023 Muscular Dystrophy Association Clinical & Scientific Conference

March 20, 2023

- Company Presents Overviews of ACHIEVE and DELIVER Global Clinical Trials with Data Anticipated from Both Studies in the Second Half of 2023 -

- FORCE™ Platform Achieves Robust Exon Skipping, Restores Dystrophin at the Sarcolemma and Halts Progression of Fibrosis in a Severe In Vivo Model of Duchenne -

WALTHAM, Mass., March 20, 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 three poster presentations at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference being held March 19-22, 2023, in Dallas, TX, and virtually.

Overviews of the company's global Phase 1/2 clinical trial designs will be presented in two poster sessions: ACHIEVE evaluating DYNE-101 for the treatment of myotonic dystrophy type 1 (DM1) and DELIVER evaluating DYNE-251 in males with Duchenne muscular dystrophy (DMD) who have mutations amenable to exon 51 skipping. Dyne anticipates reporting initial data from these clinical trials in the second half of 2023. In a separate poster presentation, new preclinical data showed that repeat monthly dosing with a mouse-specific FORCE conjugate (FORCE-M23D) achieved robust exon skipping in cardiac and skeletal muscles, dystrophin restoration, and improved skeletal muscle morphology in D2-*mdx* mice, a well-established preclinical model of DMD with a severe disease phenotype. Additionally, in the D2-*mdx* mice, starting FORCE-M23D treatment at six weeks of age led to lower deposition of fibrotic tissue compared with initiation at 14 weeks of age, indicating that earlier treatment with FORCE-M23D may lead to greater benefit. These data build upon Dyne's comprehensive preclinical data and further support the company's ongoing development in DMD.

"We're pleased to highlight for the muscle disease community our clinical trials in progress for our co-lead programs in DM1 and DMD where an urgent need exists for new and better treatment options," said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. "We look forward to sharing clinical data from our DELIVER and ACHIEVE trials in the second half of this year, furthering our goal to advance potentially transformative therapies for individuals living with these diseases."

Poster Presentations:

- ACHIEVE Trial, a Randomized, Placebo-Controlled, Multiple Ascending Dose Study of DYNE-101 in Individuals with Myotonic Dystrophy Type 1 (DM1) (Poster #80)
- DELIVER, a Randomized, Double-blind, Placebo-Controlled, Multiple Ascending Dose Study of DYNE-251 in Boys with DMD Amenable to Exon 51 Skipping (Poster #101)
- FORCE™ Platform Achieves Robust Exon Skipping, Restores Dystrophin at the Sarcolemma and Halts Progression of Fibrosis in the D2-*mdx* Model of DMD (Poster #237)

Poster sessions are from 6:00 p.m. – 8:00 p.m. CT Sunday, March 19 through Tuesday, March 21 and will be viewable in the conference exhibit hall throughout the conference. The posters will also be available in the Scientific Publications & Presentations section of Dyne's website .

About the FORCE™ Platform

The proprietary FORCE™ platform drives Dyne's efforts to develop targeted, modern oligonucleotide therapeutics with the potential to be life-transforming for patients with serious muscle diseases. Dyne designed the FORCE platform using its deep knowledge of muscle biology and oligonucleotide therapeutics to overcome limitations in delivery to muscle tissue with the goal of stopping or reversing disease progression. The FORCE platform leverages the importance of transferrin receptor 1 (TfR1) in muscle biology as the foundation for its novel approach. TfR1, which is highly expressed on the surface of muscle cells, is required for iron transport into muscle cells. Dyne links therapeutic payloads to its TfR1-binding fragment antibody (Fab) to develop targeted therapeutics for muscle diseases.

About DYNE-101 and the ACHIEVE Trial

DYNE-101 is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE trial, for people living with myotonic dystrophy type 1 (DM1). The ACHIEVE 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 64 adult patients with DM1 who are 18 to 49 years of age. The primary endpoints are safety and tolerability, with secondary endpoints of pharmacokinetics and pharmacodynamics, including change from baseline in splicing, as well as measures of muscle strength and function. Dyne anticipates reporting initial data from the MAD placebo-controlled portion of the ACHIEVE trial on safety, tolerability and splicing in the second half of 2023.

DYNE-101 consists of an antigen-binding fragment antibody (Fab) conjugated to an antisense oligonucleotide (ASO) and is designed to enable targeted muscle tissue delivery with the goal of reducing toxic *DMPK* RNA in the nucleus, releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins, and potentially stopping or reversing the disease. Dyne has generated comprehensive preclinical data supporting its DM1 program, including reduction of nuclear foci and correction of splicing in patient cells, robust knockdown of toxic human nuclear *DMPK* RNA and correction of splicing in a novel *in vivo* model developed by Dyne, and reversal of myotonia in a disease model. In non-human

primates, DYNE-101 demonstrated a favorable safety profile and achieved enhanced muscle distribution as evidenced by significant reduction in wild-type *DMPK* RNA.

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 mutations 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 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 timelines for reporting data from the DYNE-251 and DYNE-101 clinical trials, and the trial design of the DYNE-251 and DYNE-101 clinical 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 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-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.

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