# Dyne Therapeutics Announces Upcoming Presentations on Initial Clinical Data From its ACHIEVE Trial in DM1 Patients and DELIVER Trial in DMD Patients at the 2024 Muscular Dystrophy Association Clinical & Scientific Conference

February 14, 2024

*- Presentations will Highlight Initial Data Announced in January 2024 Demonstrating Proof-of-Concept in its DM1 and DMD Programs, Validating the Promise of the FORCE™ Platform and Targeted Delivery to Muscle -*

*- Additional Data from ACHIEVE and DELIVER Trials Expected in the Second Half of 2024 -*

WALTHAM, Mass., Feb. 14, 2024 (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 two oral presentations at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference being held March 3-6, 2024, in Orlando, Florida and virtually.

Both presentations will highlight the initial clinical data from the ACHIEVE and DELIVER trials reported by Dyne in January 2024. The initial clinical data from the ACHIEVE trial of DYNE-101 in patients with myotonic dystrophy type 1 (DM1) includes 6-month data from the 1.8 mg/kg (approximate ASO dose) cohort (n=16) and 3-month data from the 3.4 mg/kg Q4W cohort (n=16). Initial 6-month clinical data from the 5 mg/kg (approximate PMO dose) cohort (n=6) of the DELIVER trial of DYNE-251 in patients with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping will also be presented. Additionally, both presentations will include safety data from multiple cohorts in ACHIEVE and DELIVER trials which was shared in January.

“We’re excited to present to the rare muscle disease community initial clinical data from our co-lead programs in DM1 and DMD, where there remains an urgent and significant need for new and better treatment options,” said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. “These data reinforce the potential of FORCE to deliver for patients, and we look forward to sharing additional clinical data from our ACHIEVE and DELIVER trials in the second half of this year.”

**Oral Presentations:**

Abstract Title: Initial Data from the ACHIEVE Trial of DYNE-101 in Adults with Myotonic Dystrophy Type 1 (DM1)
Date and Time: Wednesday, March 6, 2024, at 10:15 a.m. ET
Presenter: Valeria Sansone, M.D., Ph.D., Clinical and Scientific Director, Clinical Center NeMO, Milan; Professor of Neurology, University of Milan and a Principal Investigator for the ACHIEVE Trial

Abstract Title: Initial Data from the DELIVER Trial of DYNE-251 in Males with DMD Mutations Amenable to Exon 51 Skipping
Date and Time: Wednesday, March 6, 2024, at 11:30 a.m. ET
Presenter: Kevin Flanigan M.D., Director, Center for Gene Therapy, Abigail Wexner Research Institute of Nationwide Children’s Hospital in Columbus, Ohio and a Principal Investigator for the DELIVER Trial

The presentations will be available in the Scientific Publications & Presentations section of Dyne’s website following the session.

**About ACHIEVE**
ACHIEVE is a Phase 1/2 global clinical trial evaluating DYNE-101, 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 enrolling adult patients with myotonic dystrophy type 1 (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. For more information on the ACHIEVE trial, visit https://www.clinicaltrials.gov/ (NCT05481879).

**About DYNE-101**
DYNE-101 is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE clinical trial for people living with DM1. DYNE-101 consists of an antisense oligonucleotide (ASO) 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 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 progression. DYNE-101 has been granted orphan drug designation by the European Medicines Agency and the U.S. Food and Drug Administration for the treatment of DM1.

**About Myotonic Dystrophy Type 1 (DM1)**
DM1 is a rare, progressive, genetic disease that affects skeletal, cardiac and smooth muscle. It is a monogenic, autosomal dominant disease caused by an abnormal trinucleotide expansion in a region of the *DMPK* gene. This expansion of CTG repeats causes toxic RNA to cluster in the nucleus, forming nuclear foci and altering the splicing of multiple proteins essential for normal cellular function. This altered splicing, or spliceopathy, results in a wide range of symptoms. People living with DM1 typically experience myotonia and progressive weakness of major muscle groups, which can affect mobility, breathing, heart function, speech, digestion and vision as well as cognition. DM1 is estimated to affect more than 40,000 people in the United States and over 74,000 people in Europe, but there are currently no approved disease-modifying therapies.

**About the DELIVER Trial**
DELIVER 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 enrolling ambulant and non-ambulant males with Duchenne muscular dystrophy who are ages 4 to 16 and have mutations amenable to exon 51 skipping. 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. For more information on the DELIVER trial, visit https://www.clinicaltrials.gov/ (NCT05524883).

**About DYNE-251**
DYNE-251 is an investigational therapeutic being evaluated in the Phase 1/2 global DELIVER clinical trial for people living with 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. DYNE-251 has been granted fast track, orphan drug and rare pediatric disease designations 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 and has preclinical programs targeting 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 X, 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, and the anticipated timelines for reporting data from the DYNE-101 and DYNE-251 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 and initial data from early clinical trials will be predictive of the final results of the clinical trials or future trials; whether Dyne's cash resources will be sufficient to fund the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; 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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