Dyne Therapeutics Announces Initiation of Phase 1/2 ACHIEVE Clinical Trial of DYNE-101 for the Treatment of Myotonic Dystrophy Type 1

September 6, 2022

- First Patient Expected to be Dosed in September -

- Data from Global, Multiple Ascending Dose ACHIEVE 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 the initiation of its ACHIEVE Phase 1/2 clinical trial evaluating DYNE-101 for the treatment of myotonic dystrophy type 1 (DM1).

"Advancing DYNE-101 into the clinic is an exciting moment for Dyne and for the DM1 community as there are no approved therapeutic options available for people living with this devastating disease. We are grateful to the teams at New Zealand Clinical Research and University of Auckland for their partnership in being our first ACHIEVE clinical trial site and to the other sites we are working to activate across the globe," said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. "DM1 is widely recognized as a spliceopathy by the global community. We have generated comprehensive preclinical data supporting DYNE-101 across multiple validated models, including demonstrating robust toxic *DMPK* knockdown leading to the correction of splicing, giving us a great deal of confidence as we advance to the clinic. In the second half of 2023, we anticipate sharing meaningful clinical data, including splicing in the MAD portion of our ACHIEVE trial that will advance our understanding of the potential for DYNE-101 to transform the lives of people living with DM1."

The ACHIEVE trial 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 expected to enroll approximately 64 adult patients with DM1 who are 18 to 49 years of age. The primary endpoints are safety and tolerability; secondary endpoints include pharmacokinetics and pharmacodynamics, including change from baseline in splicing, as well as measures of muscle strength and function. Dyne anticipates reporting data from the MAD placebo-controlled portion of the ACHIEVE trial on safety, tolerability and splicing in the second half of 2023.

The ACHIEVE trial is designed to efficiently optimize dose level and frequency. In the MAD portion of the trial, patients will be randomized to receive DYNE-101 or placebo intravenously every four weeks or every eight weeks for 24 weeks, depending on cohort. Patient cohorts will be dosed from 1.8 mg/kg to 10.2 mg/kg (approximate ASO dose) across four cohorts. Following the placebo-controlled period, patients will transition to DYNE-101 treatment in the open-label portion of the trial and in the long-term extension.

Visit https://www.clinicaltrials.gov/ (NCT05481879) to learn more about the ACHIEVE 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 DM1 and DYNE-251 in Duchenne muscular dystrophy (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-101

DYNE-101 is Dyne's product candidate being developed for people living with myotonic dystrophy type 1 (DM1). DYNE-101 consists of an antigen-binding fragment antibody (Fab) conjugated to an antisense oligonucleotide (ASO) 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 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 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 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-101 trial, the trial design of the DYNE-101 trial, and the planned timeline for reporting data from the DYNE-101 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