Dyne Therapeutics Receives FDA Orphan Drug Designation for DYNE-101 for the Treatment of Myotonic Dystrophy Type 1

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- On Track to Report Initial Data from the Global, Multiple Ascending Dose Phase 1/2 ACHIEVE Clinical Trial in the Second Half of 2023 -

WALTHAM, Mass., Sept. 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 that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for DYNE-101 for the treatment of myotonic dystrophy type 1 (DM1). DYNE-101 is being evaluated in the Phase 1/2 global ACHIEVE clinical trial with initial data on safety, tolerability and splicing from the multiple ascending dose, placebo-controlled portion of the trial anticipated in the second half of 2023.

“Receiving FDA orphan drug designation for DYNE-101 underscores the importance of bringing new treatment options to people living with DM1, a rare, devastating disease with no approved therapies. We believe DYNE-101 has the potential to be a transformative therapy which is why we designed the ACHIEVE trial to be registrational,” said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. “We recognize the sense of urgency within the DM1 community and look forward to sharing initial data from the ACHIEVE trial.”

Orphan drug designation is granted by the FDA to drugs or biologics intended for treatment, prevention or diagnosis of a rare disease or condition that affects fewer than 200,000 people in the United States. Under the Orphan Drug Act, orphan drug designation qualifies a company for incentives, including tax credits, exemptions from certain FDA fees for clinical trials, and the potential for seven years of market exclusivity following drug approval.

About DYNE-101

DYNE-101 is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE clinical trial 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) 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. DYNE-101 was also granted orphan drug designation by the European Medicines Agency for the treatment of DM1.

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 expected to enroll approximately 72 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. For more information on the ACHIEVE trial, visit https://www.clinicaltrials.gov/ (NCT05481879).

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 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-101 clinical trial, the trial design of the DYNE-101 clinical trial, and any potential benefit from receiving orphan drug designation, 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,” “on track,”, “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; 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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