Dyne Therapeutics Receives European Medicines Agency (EMA) Orphan Drug Designation for DYNE-101

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- Initial Data from Global, Multiple Ascending Dose Phase 1/2 ACHIEVE Clinical Trial in Myotonic Dystrophy Type 1 Anticipated in the Second Half of 2023 -

WALTHAM, Mass., May 25, 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 European Medicines Agency (EMA) has granted orphan drug designation for DYNE-101. DYNE-101 is being evaluated in the Phase 1/2 ACHIEVE global clinical trial in adults with myotonic dystrophy type 1 (DM1).

“We are pleased to receive orphan drug designation from the EMA for DYNE-101, further supporting our efforts to develop a potentially transformative therapy for DM1,” said Wildon Farwell, M.D., MPH, chief medical officer of Dyne. “The DM1 community has waited far too long for a therapy that addresses the underlying cause of this devastating rare muscle disease. We are committed to advancing DYNE-101 as quickly as possible and anticipate our first clinical data readout from our ACHIEVE trial later this year.”

The EMA grants orphan drug designation to drugs and biologics intended for the treatment, diagnosis or prevention of rare, life-threatening or chronically debilitating diseases or conditions that affect fewer than five in 10,000 people in the European Union. Orphan designation allows companies certain benefits, including reduced regulatory fees, clinical protocol assistance, research grants and up to 10 years of market exclusivity in the European Union if approved.

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 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.

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 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. 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 the potential benefits of 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,” “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 will benefit from the orphan drug designation referred to above; 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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