Dyne Therapeutics Announces “Breakthrough Article” Publication of Duchenne Muscular Dystrophy Program Data in Nucleic Acids Research

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- FORCE™ Platform Achieves Enhanced Exon Skipping and Prolonged Dystrophin Restoration in Duchenne in vivo mdx Model

WALTHAM, Mass., Aug. 10, 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 publication of Duchenne muscular dystrophy (DMD) preclinical data in a “Breakthrough Article” in Nucleic Acids Research, an honor reserved for approximately two percent of its accepted articles.

The data published demonstrate that the FORCE™ platform achieved robust and durable dystrophin expression in multiple muscle tissues and significant improvement in muscle function in *mdx* mice, a well-established preclinical model of DMD. The article, entitled “Enhanced exon skipping and prolonged dystrophin restoration achieved by TfR1-targeted delivery of antisense oligonucleotide using FORCE conjugation in *mdx* mice,” is available on the *Nucleic Acids Research* website.

“Today’s prestigious publication of our DMD program data in *Nucleic Acids Research* is a significant recognition of the FORCE platform and highlights the importance of targeted delivery. The magnitude and durability of dystrophin restoration observed in the *mdx* model, especially in the heart and diaphragm, muscles that are critical in the progression of Duchenne, provide a strong foundation to build our global DMD franchise and a tremendous sense of urgency as we prepare to bring DYNE-251 to patients in the clinic this summer,” said Oxana Beskrovnaya, Ph.D., Dyne’s chief scientific officer. “I’d like to thank the Dyne team for their fearless innovation and dedication in delivering this scientific advancement, leading to publication of this peer-reviewed article.”

The article outlines *in vivo* data in the DMD *mdx* mouse model that demonstrated that a single dose of a mouse-specific FORCE conjugate (FORCE-M23D) enhanced muscle delivery of an exon skipping phosphorodiamidate morpholino oligomer (PMO), leading to robust and durable dystrophin expression with up to 51% of wild-type levels in quadriceps, 72% in tibialis anterior, 62% in gastrocnemius, 90% in diaphragm, and 77% in heart. Additionally, the data show that FORCE achieved greater reduction in serum creatine kinase levels and improvement in muscle function, compared to an unconjugated PMO.

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. Dyne’s FORCE platform targets the transferrin receptor 1, which is highly expressed on the surface of muscle cells. In DMD, FORCE is designed to deliver a PMO to muscle tissue to promote the skipping of specific DMD exons in the nucleus, allowing muscle cells to create a truncated, functional dystrophin protein and potentially stop or reverse disease progression.

DYNE-251 is Dyne’s product candidate being developed for people living with DMD who are amenable to exon 51 skipping. DYNE-251 will be evaluated in a Phase 1/2 multiple ascending dose clinical trial, with patient dosing anticipated to commence in mid-2022. In addition, 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 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 the current 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 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-251 trial and the planned trial design of the DYNE-251 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.

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