Dyne Therapeutics Presents Preclinical Data from its Duchenne Muscular Dystrophy Programs Targeting Exons 51 and 53 at World Muscle Society 2022 Congress

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- Poster Highlights In Vivo Data for DYNE-251, Now Being Evaluated in Phase 1/2 DELIVER Clinical Trial in Patients Amenable to Exon 51 Skipping -

- First Presentation of In Vitro Data for FORCE™ Platform Conjugate Targeting Exon 53 -

WALTHAM, Mass., Oct. 11, 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 presentation of preclinical data from its Duchenne muscular dystrophy (DMD) programs for the treatment of individuals with DMD mutations amenable to skipping exons 51 or 53. The data are featured in a poster at the 27th International Hybrid Annual Congress of the World Muscle Society taking place in Halifax, Nova Scotia, Canada from October 11-15, 2022.

"We are excited to present data which highlight the potential of our FORCE™ platform in building a global DMD franchise of exon skipping therapies. Beyond DYNE-251, our product candidate targeting exon 51, we are leveraging the modularity of the FORCE platform to rapidly identify candidates for other exons, including 53, 45 and 44. The poster being presented at World Muscle features foundational preclinical data underpinning the clinical development program for DYNE-251 and the first *in vitro* data for a FORCE conjugate targeting exon 53," said Oxana Beskrovnaya, Ph.D., chief scientific officer of Dyne. "We look forward to reporting clinical data from our Phase 1/2 DELIVER trial of DYNE-251 anticipated in the second half of 2023, as this is an important milestone in our efforts to develop new exon skipping therapies for people living with this devastating disease."

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 phosphorodiamidate morpholino oligomer (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.

Poster Highlights

- As previously reported, in the *mdx* mouse model, a validated disease model of DMD which has a mutation in exon 23, a single dose of FORCE-M23D (a mouse-specific FORCE conjugate) achieved robust and durable exon skipping and dystrophin expression in cardiac and skeletal muscle as well as functional improvement. Specifically, dystrophin expression of up to 51% of wild-type levels in the quadriceps, 90% in the diaphragm, and 77% in the heart was observed. In non-human primates (NHPs), administration of five weekly doses of DYNE-251 led to pronounced exon 51 skipping in the heart (43%), diaphragm (52%) and quadriceps (18%). In addition, DYNE-251 demonstrated a favorable safety profile in a 13-week GLP toxicology study in NHPs.
- In DMD patient-derived myotubes, Dyne's novel exon 53 skipping FORCE conjugate achieved a higher level of exon skipping than an unconjugated PMO. Moreover, exon 53 skipping observed with the FORCE conjugate was dose responsive and induced restoration of dystrophin protein expression.

The poster (P.131) entitled, "Building a FORCE™ platform-based DMD franchise for the treatment of individuals with mutations amenable to exon skipping" will be available in the Scientific Publications & Presentations section of Dyne's website .

About DYNE-251 and the DELIVER Trial
DYNE-251 is Dyne's product candidate being developed for people living with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. DYNE-251 is currently being evaluated in the DELIVER trial, a Phase 1/2 global clinical trial 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 46 ambulant and non-ambulant males with DMD who are ages 4 to 16 and have mutations amenable to exon 51 skipping therapy. 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. Dyne anticipates reporting data from the MAD placebo-controlled portion of the DELIVER trial on safety, tolerability and dystrophin in the second half of 2023.

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. In preclinical studies with Dyne's FORCE™ platform, robust and durable exon skipping and dystrophin expression were observed in the*mdx* mouse model in skeletal and cardiac muscle as well as reduced muscle damage and increased muscle function. DYNE-251 demonstrated a favorable safety profile and achieved impressive exon skipping in non-human primates, especially in the heart and diaphragm, muscles in people living with DMD that weaken over time leading to mortality.

In addition to DYNE-251, 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 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 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, Dyne's ability to develop, and the therapeutic potential for, product candidates for DMD targeting exons other than exon 51, including exons 53, 45 and 44 and the anticipated timeline for reporting data from the DYNE-251 clinical 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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