Dyne Therapeutics Presents Data at World Muscle Society Congress Highlighting Promise of FORCE™ Platform to Address Underlying Causes of Neuromuscular Diseases

October 9, 2024

- Presentations Will Feature Recent Clinical Data From DYNE-251 and DYNE-101 as Well as Preclinical Data in FSHD and Pompe Disease -

WALTHAM, Mass., Oct. 09, 2024 (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 previously reported clinical and preclinical data across its pipeline will be featured in poster presentations at the 29th Annual Congress of the World Muscle Society, held virtually and in Prague, Czech Republic, October 8-12, 2024. The presentations highlight the promise of the FORCE™ platform to deliver targeted therapeutics to address neuromuscular diseases.

"The full breadth and versatility of our FORCE platform is on display at this year's World Muscle Society Congress, with presentations covering both of our co-lead clinical programs in DM1 and DMD, along with preclinical work in FSHD and Pompe disease," said John Cox, president and chief executive officer of Dyne. "We've observed targeted delivery with the FORCE platform showing broad distribution to key tissues like skeletal, cardiac, smooth muscle and the CNS – which has the potential to transform the lives of individuals living with these diseases."

Poster Highlights

- The Phase 1/2 DELIVER trial evaluating DYNE-251 in males with Duchenne muscular dystrophy (DMD) mutations amenable to exon 51 skipping includes 6-month biomarker and functional data from patients enrolled in the 20 mg/kg (approximate PMO dose) cohort and 12-month functional data from the 10 mg/kg cohort. DYNE-251 demonstrated dose dependent exon skipping and dystrophin expression and improvement in multiple functional endpoints in both cohorts. Patients treated with 20 mg/kg of DYNE-251 Q4W had a mean absolute dystrophin expression of 3.7% of normal (unadjusted for muscle content) and when adjusting for muscle content, it reached 8.7%. Importantly, treatment with DYNE-251 resulted in meaningful improvements in Stride Velocity 95th Centile (SV95C), a digital objective outcome measure of ambulatory performance in a patient's normal daily environment. The change from baseline in SV95C met the published minimal clinically important difference as defined by the European Medicines Agency (0.1 m/sec) at 6 months for both the 10 and 20 mg/kg cohorts (approximately 0.2 m/sec change from baseline for both cohorts).
- The Phase 1/2 ACHIEVE trial evaluating DYNE-101 in adult participants with myotonic dystrophy type 1 (DM1) includes 12-month data from the 1.8 mg/kg Q4W (approximate ASO dose) cohort, 6-month data from the 3.4 mg/kg Q4W cohort, and 3-month data from the 5.4 mg/kg Q8W cohort. DYNE-101 demonstrated robust muscle delivery and dose-dependent, consistent splicing correction while also showing improvement in myotonia, muscle strength, and timed function tests and in the Myotonic Dystrophy Type 1 Activity and Participation Scale (DM1-ACTIV^{C}) and the Myotonic Dystrophy Health Index (MDHI) patient reported outcomes. Patients in the 5.4 mg/kg Q8W cohort had a 27% mean splicing correction from baseline across a broad, 22-gene panel at 3 months, with all participants demonstrating splicing correction.
- Both DYNE-251 and DYNE-101 have demonstrated favorable safety profiles. [1] [2]
- Preclinical data for DYNE-302 in facioscapulohumeral muscular dystrophy (FSHD), demonstrated robust and durable *DUX4* suppression and functional benefit in an innovative hTfR1/iFLExD mouse model developed by Dyne. In hTfR1/iFLExD mice, a single intravenous dose of DYNE-302 resulted in dose-dependent and robust reduction of the DUX4 transcriptome (D4T) that lasted up to three months, with benefit on muscle structure and function.
- Preclinical data in a Pompe disease model demonstrated the potential of the FORCE platform to deliver enzyme replacement therapy to cardiac and skeletal muscle and the central nervous system (CNS), potentially expanding the modularity of the platform beyond oligonucleotides.

Presentation Information

Title: Initial Data from the DELIVER Trial of DYNE-251 in Males with *DMD* Mutations Amenable to Exon 51 Skipping
Date/Time: October 9, 2024, 2:30 p.m. CEST/8:30 a.m. ET
Presenter: Liesbeth De Waele, M.D., Ph.D., Pediatric Neurologist at University Hospital Leuven, Belgium

Title: Initial Data from the ACHIEVE Trial of DYNE-101 in Adults with Myotonic Dystrophy Type 1 (DM1)
Date/Time: October 9, 2024, 2:30 p.m. CEST/8:30 a.m. ET
Presenter: Joost Kools, M.D., Neuromuscular Department at Radboud University Medical Center, Netherlands

Title: The FORCE™ Platform Achieves Robust and Durable DUX4 Suppression and Improves Muscle Function in Facioscapulohumeral Muscular Dystrophy Mouse Model
Date/Time: October 9, 2024, 5:15 p.m. CEST/11:15 a.m. ET
Presenter: Tyler Picariello, Ph.D., Director, Neuromuscular Research, Dyne Therapeutics

Title: The FORCE™ Platform Enables Tfr1-mediated Delivery of Enzyme Replacement Therapy to Muscle and Central Nervous System, Resolving

Pompe Pathology in Mice
Date/Time: October 11, 2024, 3:45 p.m. CEST/ 9:45 a.m. ET
Presenter: Tyler Picariello, Ph.D., Director, Neuromuscular Research, Dyne Therapeutics

The presentations will be available in the Scientific Publications & Presentations section of Dyne's website following the session.

Additionally, a symposium titled "Potential of the FORCE™ Platform to Address Unmet Needs in Rare Neuromuscular Diseases" will be held on October 10 at 8:15 a.m. CEST/ 2:15 a.m. ET.

About the FORCE™ Platform

The proprietary FORCE™ platform drives Dyne's efforts to develop targeted, modern oligonucleotide innovative 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. Dyne has a broad pipeline for serious muscle diseases, including clinical programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD) and preclinical programs for facioscapulohumeral muscular dystrophy (FSHD). For more information, please visit https://www.dyne-tx.com/, and follow us on X, 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 potential of DYNE-101, DYNE-251 and DYNE-302, expectations regarding the timing and outcome of interactions with global regulatory authorities, and plans to provide future updates on pipeline programs, 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 enroll patients in clinical trials; whether results from preclinical studies and initial data from early clinical trials will be predictive of the final results of the clinical trials or future trials; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from Dyne's clinical trials and acceptance of Dyne's clinical programs and the regulatory approval process; whether Dyne's cash resources will be sufficient to fund its 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.

1. Safety data as of August 21, 2024
2. Safety data as of August 20, 2024

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