# Dyne Therapeutics Presents New Preclinical Data Demonstrating the Potential of the FORCE™ Platform to Deliver Enzyme Replacement Therapy to Muscle and CNS in Pompe Disease

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WALTHAM, Mass., June 24, 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 new preclinical data in a Pompe disease model demonstrating the potential of the FORCE™ platform to deliver enzyme replacement therapy to cardiac and skeletal muscle and the central nervous system (CNS). The data were presented at the New Directions in Biology and Disease of Skeletal Muscle Conference, being held June 23-26 in Fort Lauderdale, FL.

“These preclinical data show for the first time the ability of FORCE to deliver enzymes to skeletal and cardiac muscle as well as the CNS, expanding the modularity of our platform beyond oligonucleotides. The data support the potential of FORCE to enable effective enzyme replacement therapy with the opportunity to substantially improve upon available treatments for Pompe disease,” Oxana Beskrovnaya, Ph.D., chief scientific officer of Dyne. “We look forward to continuing to explore this application of our platform as part of our mission to deliver life-transforming therapies for people with serious muscle diseases.”

Pompe disease is a rare, severe neuromuscular disorder caused by deficiency of the lysosomal enzyme, acid alpha glucosidase (GAA). Lack of GAA leads to glycogen accumulation and increase in lysosomal size in muscle and subsequent weakness, cardiomyopathy and respiratory failure. Enzyme replacement therapy with GAA is the standard of care and increases survival but has inadequate efficacy in skeletal muscle. Pompe is also characterized by CNS manifestations, including behavioral and cognitive deficits due to glycogen accumulation in CNS cells, which are not addressed by the standard of care therapy.

Dyne engineered FORCE-GAA by leveraging the FORCE platform and evaluated efficacy *in vivo* using hTfR1/6$^{Neo}$ mice, that were developed by crossing the well-established 6$^{Neo}$ mouse model of Pompe with mice expressing human transferrin receptor 1. Intravenous administration of FORCE-GAA cleared glycogen in muscle and the CNS and normalized lysosomal size in hTfR1/6$^{Neo}$ mice. FORCE-GAA reduced serum neurofilament light chain, a biomarker of axonal injury, providing evidence of benefit in the CNS. FORCE-GAA also displayed superior efficacy and dose potency compared to GAA alone. Additional data with FORCE-GAA showed the potential for monthly dosing which is less frequent than approved enzyme replacement therapies.

The presentation entitled, “FORCE™ Platform for the Development of Targeted Therapeutics for Rare Muscle Diseases” is available in the Scientific Publications & Presentations section of Dyne’s website at https://www.dyne-tx.com/our-forcetm-publications/.

**About Pompe Disease**

Pompe disease is a severe neuromuscular disorder caused by a deficiency of the lysosomal enzyme, acid alpha glucosidase (GAA). Lack of GAA causes glycogen accumulation in tissue leading to muscle weakness, cardiomyopathy, respiratory failure, and central nervous system (CNS) manifestations. Pompe disease belongs to a group of diseases known as the lysosomal storage disorders (LSDs). It can present as infantile-onset, the most severe form of the disease with early onset of symptoms in infancy that rapidly progress, or late-onset, which progressively damages muscles over time. An estimated 5,000 to 10,000 individuals worldwide are affected by Pompe.

**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. 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 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 anticipated timelines for reporting additional data for FORCE-GAA, expectations regarding the initiation of additional preclinical studies or clinical trials of FORCE-GAA, 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; 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.

**Contacts:**

**Investors**
Amy Reilly
areilly@dyne-tx.com
857-341-1203

**Media**
Stacy Nartker
snartker@dyne-tx.com
781-317-1938