



Dyne Therapeutics to Present New Preclinical Data at ASGCT Annual Meeting Demonstrating FORCE™ Platform Delivery to CNS

May 2, 2023

- FORCE Delivers to CNS in Non-Human Primates -

- FORCE Achieves Robust Reduction of Toxic Nuclear DMPK RNA and Foci in Brain of a DM1 Disease Model -

WALTHAM, Mass., May 02, 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 new preclinical data demonstrating the FORCE™ platform achieved delivery to the central nervous system (CNS) and robust pharmacological effects in the brain will be featured in an oral presentation at the [American Society of Gene & Cell Therapy \(ASGCT\) 26th Annual Meeting](#), being held May 16-20, 2023 in Los Angeles.

The FORCE platform was designed to overcome the limitations of delivering oligonucleotide therapeutics to muscle tissue by leveraging transferrin receptor 1 (TfR1). TfR1-mediated delivery has also been shown by the field to facilitate uptake of therapeutics by the CNS. Many people living with rare muscle diseases also experience CNS symptoms that contribute to the burden of disease, including cognitive deficits and dysregulated sleep, which affect individuals with myotonic dystrophy type 1 (DM1).

In data to be presented at ASGCT, intravenous (IV) administration of FORCE conjugate achieved delivery to the CNS via TfR1 in both non-human primates (NHPs) and the innovative hTfR1/DMSXL mouse model. The hTfR1/DMSXL model, developed by Dyne, expresses the human TfR1 and carries a human *DMPK* gene with more than 1,000 CTG repeats that represents a severe DM1 phenotype.

In NHPs, FORCE conjugate, a TfR1-binding Fab antibody conjugated to an antisense oligonucleotide (ASO), demonstrated broad distribution throughout the brain compared to an unconjugated ASO delivered either via intrathecal (IT) or IV administration. Similarly, FORCE conjugate delivered to the brain of hTfR1/DMSXL mice and demonstrated robust reduction of toxic nuclear *DMPK* RNA and foci. In the hTfR1/DMSXL model and NHPs, FORCE conjugate was well tolerated.

“Our initial focus at Dyne has been to harness the power of the FORCE platform to deliver oligonucleotide therapeutics to muscle. These new data show that FORCE can also deliver to the CNS which is critically important for the treatment of neuromuscular disorders, including DM1,” said Oxana Beskrovnyaya, Ph.D., chief scientific officer of Dyne. “We have now shown that FORCE enables delivery to skeletal, smooth and cardiac muscle, as well as the CNS in multiple preclinical studies, demonstrating the exciting potential of TfR1 as a delivery mechanism. We look forward to presenting these data at ASGCT and continuing to explore this application of FORCE as part of our commitment to comprehensively address neuromuscular diseases and bring potentially life-transforming therapies to patients.”

ASGCT meeting abstracts are available [on the meeting website](#), and the following are details on Dyne's presentation:

Oral Presentation: FORCE™ Platform Delivers Oligonucleotides to the Brain in a DM1 Mouse Model and in NHPs (abstract #82)

Session: Nucleic Acid Therapeutics

Date/Time: Wednesday, May 17, 2023, at 4:00 p.m. PT

Presenter: Stefano Zanotti, Ph.D., Executive Director, Head of Neuromuscular Research, Dyne

The presentation will be available in the [Scientific Publications & Presentations](#) section of Dyne's website following the session.

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, and the potential of the FORCE platform, 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-K 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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