



Dyne Therapeutics Presents Preclinical Data from its Facioscapulohumeral Muscular Dystrophy Program During the FSHD Society International Research Congress

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- *FORCE™ platform enables targeted muscle delivery with lead FSHD program candidate demonstrating potent suppression of DUX4 biomarkers in patient cell line -*

WALTHAM, Mass., June 25, 2021 (GLOBE NEWSWIRE) -- [Dyne Therapeutics, Inc.](https://www.dyne-tx.com) (Nasdaq: DYN), a muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases, is presenting today preclinical data from its facioscapulohumeral muscular dystrophy (FSHD) program during the [28th Annual FSHD Society International Research Congress](#). Data from *in vitro* studies in an FSHD patient cell line being presented highlight that Dyne's proprietary FORCE™ platform enabled targeted muscle delivery with its lead FSHD candidate demonstrating potent suppression of DUX4 transcriptome markers.

FSHD is caused by aberrant activation of the double homeobox 4 (DUX4) transcription factor in muscle cells, leading to skeletal muscle loss, progressive muscle weakness and wasting. Dyne's approach is designed to address the genetic basis of the disease by reducing *DUX4* mRNA expression. The company's lead FSHD candidate consists of a transferrin 1 receptor-binding fragment antibody (Fab) conjugated to a phosphorodiamidate morpholino oligomer (PMO) targeting *DUX4* mRNA.

"It is critically important that we continue to advance the science and new therapeutic candidates for FSHD, a highly debilitating, progressive genetic disease with no approved treatments," said Oxana Beskrovnaya, Ph.D., chief scientific officer of Dyne. "We are excited to share these data, which build upon our prior *in vitro* studies and support our approach to targeting the genetic basis of FSHD. These results demonstrate that our lead FSHD candidate achieved potent suppression of key DUX4 biomarkers, reinforcing the potential of the FORCE platform to enable targeted delivery to muscle tissue. We look forward to advancing our FSHD program toward the clinic as one of the three IND submissions we have planned between the fourth quarter of 2021 and the fourth quarter of 2022."

The *in vitro* proof of concept studies were conducted using an immortalized FSHD patient cell line. Three well-known DUX4 transcriptome markers MBD3L2, TRIM43, and ZSCAN4 were tracked and a significant increase in expression was observed in these markers once cells were differentiated to myotubes. Dyne's lead FSHD candidate was subsequently evaluated *in vitro* and showed potent suppression of these DUX4 transcriptome markers with IC50 in the low nanomolar range and also demonstrated superior reduction of these same markers compared to naked (unconjugated) PMO.

Today's presentation entitled, "FORCE™ platform enables muscle-targeted delivery of antisense oligonucleotide and silencing of DUX4 activity in an FSHD cell line" will be available in the Scientific Publications & Presentations section of Dyne's website following the meeting at <https://www.dyne-tx.com/our-forcetm-publications/>.

About Facioscapulohumeral Muscular Dystrophy (FSHD)

FSHD is a rare, progressive, genetic disease caused by a mutation in the *DUX4* gene, leading to skeletal muscle loss, muscle weakness and wasting. In healthy individuals, *DUX4*-driven gene expression is active for only a short time in early embryonic development. In individuals with FSHD, the *DUX4* gene remains "on" long after it is supposed to be silenced. This genetic mutation leads to surplus production of the DUX4 protein, which causes the gradual destruction of muscle cells throughout the body. People living with FSHD experience weakness in all major muscle groups including the face, joint and spinal abnormalities, and often limited mobility. An estimated 16,000-38,000 individuals in the United States and approximately 35,000 in Europe are affected by FSHD, but there are currently no approved disease-modifying treatments.

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 1 receptor, 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 building a leading muscle disease company dedicated to 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's broad portfolio of therapeutic candidates for serious muscle diseases includes programs 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, 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 conduct of research activities and the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies; the timing of and Dyne's ability to submit and obtain regulatory clearance for investigational new drug applications; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; whether estimates of patient populations will accurately reflect the addressable patient populations of our candidates; 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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