



## Dyne Therapeutics Announces Support for END-DM1 Natural History Study of Patients with Myotonic Dystrophy Type 1 (DM1)

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*Study sponsored by Myotonic Dystrophy Clinical Research Network will advance understanding of disease progression and enable development of clinical outcome assessments*

*Patients with DM1 aged 18-70 encouraged to enroll at study sites in U.S. and Europe*

CAMBRIDGE, Mass. – [Dyne Therapeutics](#), a biotechnology company pioneering targeted therapies for patients with serious muscle diseases, today announced its support for END-DM1, a natural history study to advance the understanding of disease progression in patients with myotonic dystrophy type 1 (DM1) and enable the development of clinical outcome assessments. END-DM1 (Establishing Biomarkers and Clinical Endpoints in Myotonic Dystrophy Type 1) is a non-interventional study sponsored by the Myotonic Dystrophy Clinical Research Network (DMCRN), a network of medical centers that aims to support future clinical trials of potential therapies for DM1 through the standardization of testing methods.

The END-DM1 study will enroll approximately 650 people with DM1 aged 18-70 across nine DMCRN study sites in the U.S. and seven sites in Europe. Financial support from Dyne Therapeutics enables the expansion of the study to Europe. In addition to Dyne, supporters of END-DM1 include the FDA, the Myotonic Dystrophy Foundation, the Wyck Foundation and the Muscular Dystrophy Association.

“At Dyne, we are striving to transform the lives of patients and families affected by DM1 by developing the first disease-modifying therapies,” said Romesh Subramanian, Ph.D., President and CEO of Dyne. “As we rapidly advance our development programs, we are proud to support this critical initiative that facilitates a global DM1 patient community and provides the foundation for clinical trial readiness.”

END-DM1 will be the third and largest natural history study sponsored by DMCRN since the network was established in 2012. Building on current knowledge of DM1 pathogenesis and therapeutic targets, the END-DM1 study will aim to:

- Enhance understanding of patient heterogeneity by characterizing baseline status and disease progression of a larger cohort through selected functional tests and patient-reported outcomes
- Develop reliable biomarkers of disease severity and therapeutic response using optimized biopsy collection and analysis among a subset of patients
- Identify possible genetic modifiers of disease severity through genome-wide association analysis

“This is an exciting time for the development of targeted RNA-based therapies in DM1, but our understanding of this disease is still growing,” said Nicholas Johnson, M.D., a co-principal investigator for END-DM1 at [VCU Health](#) in Richmond, Virginia. “Information collected during this study will allow improved assessment of the efficacy of potential new treatment options, and we encourage patients and caregivers to consider participating.”

For more information about END-DM1, including study requirements and a list of participating sites, please visit the [Myotonic Dystrophy Foundation website](#).

### **About Dyne Therapeutics**

Dyne Therapeutics is pioneering therapies that target muscle tissue with unprecedented precision to restore muscle health. The company’s FORCE™ platform delivers oligonucleotides and other molecules to skeletal, cardiac and smooth muscle to treat a range of serious muscle diseases. Dyne is advancing a treatment for myotonic dystrophy type 1 (DM1) in addition to programs for Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD). Dyne launched in 2019 and is based in Cambridge, Mass. For more information, please visit [www.dyne-tx.com](http://www.dyne-tx.com).

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