Safety, Tolerability, Pharmacodynamic, Efficacy, and Pharmacokinetic Study of DYNE-101 in Participants With Myotonic Dystrophy Type 1

NCT05481879

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Dyne Therapeutics
Enrollment	116 participants

Key Eligibility Criteria

Inclusion (5)

- Diagnosis of DM1 with trinucleotide repeat size \>100.
- Age of onset of DM1 muscle symptoms e12 years.
- Clinically apparent myotonia equivalent to hand opening time of at least 2 seconds in the opinion of the Investigator.
- Hand grip strength and ankle dorsiflexion strength.
- Able to complete 10-MWRT, stair ascend/descend (MAD cohorts only), and 5×STS at screening without the use of assistive devices such as canes, walkers, or orthoses.

Exclusion (10)

- History of major surgical procedure within 12 weeks prior to the start of investigative product administration or an expectation of a major surgical procedure (eg, implantation of cardiac defibrillator) during the study.
- History of anaphylaxis.
- Medical condition other than DM1 that would significantly impact ambulation or participation in functional assessments.
- Treatment with medications that can improve myotonia within a period of 5 half-lives of the medication prior to performing screening assessments.
- Electrocardiogram (ECG) with the corrected QT interval by Fridericia's Formula (QTcF) e450 milliseconds (ms) in men and QTcF e460 ms in women, PR e240 ms, left bundle-branch block, or a conduction defect, which is clinically significant in the opinion of the Investigator.

... and 5 more (see full listing online)

Locations (17 total)

Indiana University School of Medicine, Indianapolis, Indiana, United States
University of Iowa, Iowa City, Iowa, United States
University of Rochester Medical Center, Rochester, New York, United States
... and 14 more locations