

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

Status: RECRUITING

Eligibility Criteria

Age: 18 years to 65 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Diagnosis of DM1 with trinucleotide repeat size ≥ 100 . * Age of onset of DM1 muscle symptoms ≥ 12 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 5xSTS at screening without the use of assistive devices such as canes, walkers, or orthoses.

Exclusion Criteria:

* 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) ≥ 450 milliseconds (ms) in men and QTcF ≥ 460 ms in women, PR ≥ 240 ms, left bundle-branch block, or a conduction defect, which is clinically significant in the opinion of the Investigator. * Percent predicted forced vital capacity (FVC) $< 50\%$. * History of tibialis anterior biopsy within 3 months of Day 1 or planning to undergo tibialis anterior biopsies during study period for reasons unrelated to the study. * Participant has a history of suicide attempt, suicidal behavior, or has any suicidal ideation within 6 months prior to Screening that meets criteria at a level of 4 or 5 of the Columbia Suicide Severity Rating Scale (C-SSRS) or who, in the opinion of the Investigator, is at significant risk to commit suicide. * Use of glucagon-like peptide 1 (GLP-1) agonist medications including semaglutide, dulaglutide, liraglutide, exenatide, or tirzepatide within a period of 5 half-lives of the medication prior to performing screening assessments. * Significant weight loss during study participation may impact weight-based dosing, performance on muscle function assessments, and pharmacodynamic (PD) biomarkers. Note: Other inclusion and exclusion criteria may apply.

Conditions & Interventions

Interventions:

DRUG: DYNE-101, DRUG: Placebo

Conditions:

Myotonic Dystrophy Type 1 (DM1)

More Information

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Principal Investigator:

IRB

Number:

System ID: NCT05481879

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