Safety, Tolerability, PK, and PD Study of PGN-EDODM1 in Participants With Myotonic Dystrophy Type 1

Status: RECRUITING

Eligibility Criteria

Age: 18 years to 60 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Confirmed diagnosis of DM1, as defined as having a repeat sequence in the DMPK gene with at least 100 CTG repeats * Medical Research Council (MRC) score of ≥ Grade 4- in bilateral tibialis anterior (TA) muscles (the ability to move through full range of motion and hold against at least moderate pressure from the examiner) * Presence of myotonia

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Exclusion Criteria:

* Congenital DM1 * Known history or presence of any clinically significant conditions that may interfere with study safety assessments * Abnormal laboratory tests at screening * Medications specific for the treatment of myotonia within 2 weeks prior to screening * Percent predicted forced vital capacity (FVC) \<40% Note: Other inclusion and exclusion criteria may apply.

Conditions & Interventions

Interventions:
DRUG: PGN-EDODM1 for infusion, OTHER: Placebo
Conditions:
Myotonic Dystrophy 1
Keywords:
Myotonic Dystrophy, Muscular Dystrophies, Genetic Diseases, Inborn, Neuromuscular Diseases, Nervous System Diseases, Musculoskeletal Diseases, Myotonic Disorders, Muscular Disorders, Atrophic, Heredodegenerative Disorders, Nervous System, Neurodegenerative Diseases, Muscular Diseases, Steinert Disease

More Information

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Phase: PHASE1
IRB
Number:
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