Study of ATX-01 in Participants With DM1

NCT06300307

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	ARTHEx Biotech S.L.
Enrollment	56 participants

Key Eligibility Criteria

Inclusion (3)

- Participants with a documented clinical diagnosis of DM1 (CTG expansion of \>150 repeats in DMPK gene measured in peripheral blood mononuclear cells)
- Ambulatory, defined as able to complete a 10-meter walk/run test at screening without the use of assistive devices such as canes, walkers, or orthoses, except for ankle-foot orthoses
- Presence for \>3 seconds of grip myotonia as confirmed by a central reader

Exclusion (3)

- Participants with congenital DM1
- Medical Research Council Muscle Scale score of less than 4 on ankle dorsiflexion or significant tibialis anterior atrophy that prevents a muscle biopsy
- Use of mexiletine or other agent for myotonia within 21 days or 5 half-lives, whichever is longer, prior to screening

Locations (12 total)

UCLA, Los Angeles, California, United States
University of Florida, Gainesville, Florida, United States
University of Iowa Health Care - Department of Neurology, Iowa City, Iowa, United States
... and 9 more locations