

Safety and Efficacy of NMD670 in Ambulatory Adult Patients With Type 3 Spinal Muscular Atrophy

NCT05794139

Status	RECRUITING
Phase	Phase 2
Sponsor	NMD Pharma A/S
Enrollment	54 participants

Key Eligibility Criteria

Inclusion (8)

- Participants with a clinical diagnosis of Type 3 SMA.
- Participants who are ambulatory, defined as being able to walk at least 50 metres without walking aids at screening during the 6-minute walk test.
- Participant with genetic confirmation of diagnosis (e.g., homozygous deletion or compound heterozygous deletion and mutation of survival of motor neuron 1 gene \[SMN1\])
- Participant with 3 to 5 copies of survival of motor neuron 2 gene \[SMN2\].
- Participant has a body mass index (BMI) within the range 19-35 kg/m² (inclusive).
- ... and 3 more (see full listing online)

Exclusion (5)

- Participants with prior surgery or fixed deformity (scoliosis, contractures) which would restrict ability to perform study-related tasks.
- Participants with other significant disease that may interfere with the interpretation of study data (e.g., other neuromuscular or muscular diseases).
- Participants with other significant clinical and/or laboratory safety findings that may interfere with the conduction or interpretation of the study
- Participants received treatment with an investigational medical product (IMP) within 30 days (or 5 half-lives of the medication, whichever is longer) prior to Day 1.
- Participants with history of poor compliance with relevant SMA therapy.

Locations (31 total)

UCLA David Geffen School Of Medicine - Neurology, Los Angeles, California, United States
Stanford University Medical Center, Palo Alto, California, United States
University of Colorado Neurology Clinic, Aurora, Colorado, United States
... and 28 more locations

<https://clinicaltrials.gov/study/NCT05794139>

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