A Phase 1/2 Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of BMN 351 in Participants With Duchenne Muscular Dystrophy

NCT06280209

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	BioMarin Pharmaceutical
Enrollment	18 participants

Key Eligibility Criteria

Inclusion (5)

- Age 4 to 10
- Diagnosis of Duchenne muscular dystrophy with a specific genetic change amenable to exon 51 skipping
- Able to walk
- Not requiring assistance from a ventilator to breathe
- Currently on consistent doses of steroid treatment for the last 12 weeks

Exclusion (3)

- The participant will have some initial clinical labs and studies to assess baseline level of heart and lung function.
- Treatment with an exon skipping therapy within 12 weeks prior to the first visit.
- Any history of treatment with gene therapy

Locations (8 total)

Children's Hospital LHSC, London, Ontario, Canada
Fondazione Serena ETS - Centro Clinico NeMO Milano, Milan, Italy
UOC Fase I - Fondazione Policlinico Universitario A. Gemelli IRCCS - Universita Cattolica del Sacro Cuore, Rome, Italy
... and 5 more locations