A Study of SGT-003 Gene Therapy in Ambulant Males With Duchenne Muscular Dystrophy (IMPACT DUCHENNE)

NCT07160634

Status	RECRUITING
Phase	Phase 3
Sponsor	Solid Biosciences Inc.
Enrollment	80 participants

Key Eligibility Criteria

Inclusion (7)

- Participant is ambulatory.
- Established clinical diagnosis of DMD and documented DMD gene mutation predictive of DMD phenotype.
- Negative for antibodies against adeno-associated virus.
- On a stable daily oral regimen of at least 0.5 mg/kg/day prednisone or 0.75 milligrams per kilogram per day (mg/kg/day) deflazacort for at least 6 months prior to entering the study, allowing for weight-based dose modifications in accordance with clinical practice.
- Meet 10-meter walk/run time criteria.

... and 2 more (see full listing online)

Exclusion (3)

- Current or prior treatment with an approved or investigational gene transfer drug or gene editing therapy.
- Exposure to vamorolone, givinostat, approved or investigational dystrophin- or disease-modifying drugs (such as eteplirsen, golodirsen, casimersen, viltolarsen, and ataluren), or another investigational drug for any indication within 6 months or 5 half-lives, whichever is longer, prior to enrollment.
- Established clinical diagnosis of DMD that is associated with any deletion variant or variant predicted not to express exons 1 to 11, exons 42 to 45, or exons 57 to 69, inclusive of the DMD gene as documented by a genetic report.

Locations (2 total)

The Children's Hospital of Westmead, Sydney, New South Wales, Australia
BC Children's Hospital, Vancouver, British Columbia, Canada