Phase 2 Study of SAT-3247 in Pediatric Ambulatory Patients

NCT07287189

Status	RECRUITING
Phase	Phase 2
Sponsor	Satellos Bioscience, Inc.
Enrollment	51 participants

Key Eligibility Criteria

Inclusion (9)

- Has a definitive diagnosis of DMD based on documented clinical findings and prior genetic testing with a confirmed mutation in the DMD gene.
- Male DMD patients who are ambulatory and aged e 7 to \< 10 years at the time of screening.
- Stable dose of systemic glucocorticoids (i.e., prednisolone, deflazacort, or vamorolone) according to the standard of care for e 3 months prior to the Screening Visit and for the duration of the trial. Patients who are not receiving glucocorticosteroids are also eligible if stopped e 3 months prior to the Screening Visit.
- Stable doses of prescription medicines including ACE inhibitors, ²blockers, and diuretics (excluding glucocorticosteroids) and over-the-counter medicines and/or herbal supplements for supportive care e 1 month prior to the Screening Visit and for the duration of the trial.
- Participants that have previously received delandistrogene moxeparvovec (brand name Elevidys) either in a prior clinical trial or in the commercial setting \> 18 months prior to screening whose muscle function tests have stabilized or demonstrated decline e 3 months prior to Screening, as determined by investigator and documented in chart notes, will be eligible.

... and 4 more (see full listing online)

Exclusion (9)

- Ambulatory patients expected to experience loss of ambulation within d 12 months.
- Participants for whom MRI or open muscle biopsy are contraindicated.
- Evidence of significant hepatic dysfunction, defined as GLDH \> 2X upper limit of normal (ULN) at the Screening Visit.
- Impaired cardiac function defined as a left ventricular ejection fraction of \< 50% on screening cardiac assessments (echocardiogram or MRI) or evidence of symptomatic cardiomyopathy.
- A forced vital capacity \< 60% predicted at the Screening Visit.

... and 4 more (see full listing online)

Locations (19 total)

University of California Los Angeles, Los Angeles, California, United States
Colorado Children's, Aurora, Colorado, United States
Lurie Children's, Chicago, Illinois, United States
... and 16 more locations