Phase 2 Study of SAT-3247 in Pediatric Ambulatory Patients

Status: RECRUITING

Eligibility Criteria

Age: 7 years to 9 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Key

Inclusion Criteria:

* 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 ≥ 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 ≥ 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 ≥ 3 months prior to the Screening Visit. * Stable doses of prescription medicines including ACE inhibitors, β-blockers, and diuretics (excluding glucocorticosteroids) and over-the-counter medicines and/or herbal supplements for supportive care ≥ 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 ≥ 3 months prior to Screening, as determined by investigator and documented in chart notes, will be eligible. * Participants that have previously received an exon skipper \> 6 months prior to Screening whose muscle function tests have stabilized or demonstrated decline ≥ 3 months prior to Screening, as determined by investigator and documented in chart notes, will be eligible. * Participants receiving a stable dose of givinostat (brand name Duvyzat) for at least 18 months or longer prior to the Screening Visit will be eligible. Participants unable to tolerate givinostat who discontinued treatment before 18 months are eligible to enroll if date of last dose is ≥ 30 days from the Screening date. Givinostat should not be discontinued, if tolerated, to meet study entry criteria. * Participants that have received prior treatment with an investigational gene therapy product (other than delandistrogene moxeparvovec) ≥ 24 months prior to the Screening Visit. * If participating in a physical therapy/strength training regimen, must be stable for ≥ 2 months prior to the Screening Visit and for the duration of the trial. Key

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Exclusion Criteria:

* Ambulatory patients expected to experience loss of ambulation within ≤ 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. * Ongoing participation in any other therapeutic clinical trial or follow-up study for a therapeutic intervention * Consumption of grapefruit juice or grapefruit containing products * Severe behavioural or cognitive problems that preclude participation in the study, in the opinion of the investigator. Additional entry criteria will be reviewed with the clinical site investigator.

Conditions & Interventions

Interventions:
DRUG: SAT-3247, DRUG: Placebo
Conditions:
Duchenne Muscular Dystrophy, Duchenne, DMD, Neuromuscular Diseases, Muscular Dystrophies
Keywords:
muscle regeneration, satellite cell, asymmetric division, dystrophin

More Information

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