AFFINITY DUCHENNE: RGX-202 Gene Therapy in Participants With Duchenne Muscular Dystrophy (DMD)

Status: RECRUITING

Eligibility Criteria

Age: 1 year and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Part 1

•Key

Inclusion Criteria:

* The participant's legal guardian(s) is (are) willing and able to provide written, signed informed consent prior to any study-related procedures; and, where applicable, the minor participant has provided written or verbal assent according to local requirements. * Is a male at least 4 years of age and less than 12 years of age at consent or 1 to \<4 years of age at the time of dosing and ≥ 10 kg at the time of screening. * Must meet any of the following criteria: * DMD gene mutation in exons 18 and above, and a clinical picture consistent with typical DMD with the exception of a participant (Cohort 1b) with DMD gene mutation in exons 12-17. * Participant is able to walk 100 meters independently without assistive devices. Cohort 2c participant must be able to walk 10 meters independently without assistive devices. Cohort 1b participant must be able to walk with or without assistive devices. * Participant is able to complete the TTSTAND per protocol-specific criteria. * Participant has been on a stable dose of systemic glucocorticoids according to the standard of care for at least 12 weeks. Cohort 2c participants must be consistently on or off a stable dose of systemic glucocorticoids according to the standard of care for at least 12 weeks. * Clinical laboratory test results, including hepatic and renal function, are within the normal range during screening, or if abnormal, are not clinically significant, in the opinion of the investigator. * Documentation is provided at screening visit for participant's adherence to the local country's vaccination schedule. The parent(s) or legal guardian(s) must be willing to have their child receive a meningococcal vaccine, if not already vaccinated. * Participant and parent(s)/legal guardian(s) are willing and able to comply with scheduled visits, study intervention administration plan, and study procedures. Part 2 and 3

Inclusion Criteria:

* The participant's legal guardian(s) is (are) willing and able to provide written, signed informed consent prior to any study-related procedures; and, where applicable, the minor participant has provided written or verbal assent according to local requirements. * DMD gene mutation with any mutation except for those with deletions or point mutations in exons 8, 9 and/or 10. * Participant is able to complete the TTSTAND per protocol-specific criteria. * Clinical laboratory test results, including hepatic and renal function, are within the normal range during screening, or if abnormal, are not clinically significant, in the opinion of the investigator. * Documentation is provided at screening visit for participant's adherence to the local country's vaccination schedule. The parent(s) or legal guardian(s) must be willing to have their child receive a meningococcal vaccine, if not already vaccinated. * Participant and parent(s)/legal guardian(s) are willing and able to comply with scheduled visits, study intervention administration plan, and study procedures. * Is a male at least 1 year of age and ≥ 10 kg at the time of screening. * Participants 1 to \<4 years of age must meet the following criteria: * is able to walk 10 meters independently without assistive devices. * must be consistently on or off a stable dose of systemic glucocorticoids according to the standard of care for at least 12 weeks. * Participants 4 years and older must meet the following criteria: * are able to walk 100 meters independently without assistive devices. * have been on a stable dose of systemic glucocorticoids according to the standard of care for at least 12 weeks. * have a NSAA total score ≥16. Part 1

Exclusion Criteria:

* Participant has any condition that would contraindicate treatment with immunosuppression. * Participant has received ataluren (a protein restoration therapy) or an exon-skipping therapy for the treatment of DMD within 6 months of study entry or is unable to refrain from taking ataluren or exon-skipping therapy for a duration of 5 years from the time of RGX-202 administration. * Participant has received any investigational or commercial gene therapy product over his lifetime. * Participant is currently taking any other investigational intervention (other than corticosteroids) or has taken any other investigational intervention (other than corticosteroids) within 3 months prior to the scheduled Day 1 intervention. If your corticosteroid is vamorolone, the participant will be asked to temporarily convert his daily dosing to prednisolone/prednisone during a short period of time around RGX-202 administration. He will be allowed to revert back to his baseline vamorolone regimen at the original per kilogram dose at which he entered the study and should remain on this for 24 months unless the investigator determines that this is not clinically indicated or possible. * Participant has impaired cardiac function defined as a left ventricular ejection fraction of \< 55% on screening cardiac assessments (echocardiogram or MRI). * Participant is not a good candidate for the study, in the opinion of the investigator. Part 2 and 3

Exclusion Criteria:

* Participant has any condition that would contraindicate treatment with immunosuppression. * Participant has received givinostat within 3 months of study entry or has received ataluren (a protein restoration therapy) or an exon-skipping therapy for the treatment of DMD within 6 months of study entry or is unable to refrain from taking ataluren or exon-skipping therapy for a duration of 5 years from the time of RGX-202 administration. * Participant has received any investigational or commercial gene therapy product over his lifetime. * Participant is currently taking any other investigational intervention (other than corticosteroids) or has taken any other investigational intervention (other than corticosteroids) within 3 months prior to the scheduled Day 1 intervention. If your corticosteroid is vamorolone, the participant will be asked to temporarily convert his daily dosing to prednisolone/prednisone during a short period of time around RGX-202 administration. He will be allowed to revert back to his baseline vamorolone regimen at the original per kilogram dose at which he entered the study and should remain on this for 24 months unless the investigator determines that this is not clinically indicated or possible. * Participant has detectable AAV8 total binding antibodies in serum. * Participant has impaired cardiac function defined as a left ventricular ejection fraction of \< 55% on screening cardiac assessments echocardiogram or MRI). * Participant is not a good candidate for the study, in the opinion of the investigator.

Conditions & Interventions

Interventions:

GENETIC: RGX-202

Conditions:

Duchenne Muscular Dystrophy

Keywords:

Gene therapy, DMD, Duchenne Muscular Dystrophy, Duchenne

More Information

Contact(s): Patient Advocacy - Duchenne@regenxbio.com
Principal Investigator:
Phase: PHASE2
IRB
Number:
System ID: NCT05693142
