Pfizer - Fordadistrogene Movaparvovec (PF-06939926)

A Phase 2, multicenter, single-arm study to evaluate the safety and dystrophin expression after Fordadistrogene Movaparvovec (PF-06939926) administration in male participants with early stage Duchenne Muscular Dystrophy

Summary

This is a gene therapy study that studies the safety and effectiveness of Fordadistrogene Movaparvovec (PF-06939926) in boys with DMD. It is a single-arm, non-randomized, open-label study, meaning that all participants will be receiving the gene therapy drug.

Study Number: NCT05429372

Description by Pfizer

The study will assess the safety and tolerability of fordadistrogene movaparvovec gene therapy. Approximately 10 participants will be enrolled in the study and receive a single IV infusion of PF-06939926; there is no placebo arm. The study includes boys who are at least 2 years old and less than 4 years old (including 3 year olds up until their 4th birthday). All boys will need to be negative for neutralizing antibodies against AAV9, as measured by the test done for the study as part of screening.

The primary analysis will occur when all participants have completed visits through Week 52 (or withdrawn from the study prior to Week 52). All participants will be followed in the study for 5 years after treatment with gene therapy.

Primary Outcome Measures

- Incidence and severity of Treatment-Emergent Adverse Events and Serious Adverse Events [Time Frame: Through Week 52]
- Number of participants with abnormal hematology test results [Time Frame: Through Week 52]

  Blood samples will be collected from subjects for the analysis of hematology
- Number of participants with abnormal biochemistry test results [Time Frame: Through Week 52]

  Blood samples will be collected from subjects for the analysis of biochemistry
- Number of participants with abnormal urine analysis [Time Frame: Through Week 52]

  Urine samples will be collected from subjects for the analysis of urine
- Number of participants with abnormal and clinically relevant changes in neurological examinations [Time Frame: Through Week 52]
- Number of participants with abnormal and clinically relevant changes in body weight [Time Frame: Through Week 52]
- Number of participants with abnormal and clinically relevant changes in vital signs [Time Frame: Through Week 52]
- Number of participants with abnormal and clinically relevant changes on cardiac troponin I [Time Frame: Through Week 52]
- Number of participants with abnormal and clinically relevant changes on electrocardiogram (ECG) [Time Frame: Through Week 52]
- Number of participants with abnormal and clinically relevant changes on echocardiogram [Time Frame: Through Week 52]

Secondary Outcome Measures

- Distribution of mini-dystrophin expression in muscle [Time Frame: At Week 9 and Week 52]

  Mini-dystrophin distribution from a muscle biopsy will be assessed by immunofluorescence
- Level of mini-dystrophin expression in muscle [Time Frame: At Week 9 and Week 52]

  Mini-dystrophin expression level from a muscle biopsy will be assessed by liquid chromatography mass spectrometry
- Incidence and severity of Treatment-Emergent Adverse Events and Serious Adverse Events [Time Frame: Through 5 years]
- Number of participants with abnormal hematology test results [Time Frame: Through 5 years]

  Blood samples will be collected from subjects for the analysis of hematology
- Number of participants with abnormal biochemistry test results [Time Frame: Through 5 years]

  Blood samples will be collected from subjects for the analysis of biochemistry
- Number of participants with abnormal urine analysis [Time Frame: Through 5 years]

  Urine samples will be collected from subjects for the analysis of urine

AUSNMD

Trial Status
Recruiting

Locations
Melbourne - Melbourne Children's Campus, Recruiting, Sydney - Westmead Children's Hospital, Recruiting

Trial Sponsor
Pfizer

Age
2 to under 4 years

Mutation Specific
Both, Exclusions apply for some mutations in exons 9-13 and 29-30; please see exclusion criteria for more details.

Muscle Biopsy
Yes

MRI
No

Phase
2

Length Of Participation
52 weeks to 5 years

Recruitment Target
10

Ambulatory
Yes

Therapeutic Category
Gene therapy

- Number of participants with abnormal and clinically relevant changes in neurological examinations [Time Frame: Through 5 years]
- Number of participants with abnormal and clinically relevant changes in body weight [Time Frame: Through 5 years]
- Number of participants with abnormal and clinically relevant changes in vital signs [Time Frame: Through 5 years]
- Number of participants with abnormal and clinically relevant changes on cardiac troponin I [Time Frame: Through 5 years]
- Number of participants with abnormal and clinically relevant changes on electrocardiogram (ECG) [Time Frame: Through 5 years]
- Number of participants with abnormal and clinically relevant changes on echocardiogram [Time Frame: Through 5 years]

Can I take part?

Inclusion Criteria

- Confirmed diagnosis of DMD by prior genetic testing.

Exclusion Criteria

- Any of the following genetic abnormalities in the dystrophin gene: a. Any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting any exon between exon 9 and exon 13, inclusive; OR b. A deletion that affects both exon 29 and exon 30.
- Positive test performed by Pfizer for neutralizing antibodies to AAV9.
- Motor and cognitive function not adequate to participate in the study, as assessed by protocol-specified criteria.
- Any prior treatment with gene therapy.
- Any treatment designed to increase dystrophin expression within 6 months prior to screening (including, but not limited to, exon-skipping and nonsense read through).
- Previous or current treatment with oral glucocorticoids or other immunosuppressive agents for the indication of DMD.
- Abnormality in specified laboratory tests, including blood counts, liver and kidney function.

For contact details and to find out more, please refer to ausnmd.org.