PreU7-53

Observational study of patients with DMD theoretically treatable with exon skipping 53

Hub Summary

PreU7-53 is an observational cohort study. This natural history study is designed to monitor upper limb muscle impairment in patients potentially treatable with AAV-mediated exon skipping.

Study Number: NCT01385917

Description by Genethon

PreU7-53 is a natural history study. The objective is to monitor the clinical and radiological course of upper limb muscle impairment in patients with Duchenne Muscular Dystrophy (DMD), potentially treatable with AAV-mediated exon 53 skipping.

Primary Outcome Measures

PreU7-53 is a natural history study [Time Frame: Every year]

The objective is to monitor the clinical and radiological course of upper limb muscle impairment in patients with DMD, potentially treatable with AAV-mediated exon 53 skipping.

Can I take part?

Inclusion Criteria

- ✔ Diagnosis of Duchenne muscular dystrophy confirmed by at least genetic testing, theoretically treatable by exon 53 skipping.
- ✔ Age between 12 and <20 years old.
- ✔ Non ambulant patients (ie; inability to walk more than 10 meters without any of assistance).
- ✔ Patients covered by a national health insurance scheme.
- ✔ Signed informed consent.

Exclusion Criteria

- ✖ Patient incapable of sitting upright in a wheelchair for at least one hour.
- ✖ Patients with severe intellectual impairment preventing them from fully understanding the exercises to be performed.
- ✖ Recent (less than 6 months ago) upper limb surgery or trauma. This criteria is however not definitive. Patients who have undergone upper limb surgery or trauma may nonetheless be enrolled once the 6 month period is over.
- ✖ Known immune deficiency.
- ✖ Contraindications to NMR exams

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

Duchenne UK

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Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated

Trial Sponsor
Genethon

Phase
Observational

Length Of Participation
TBC

Recruitment Target
45

Ambulatory
Non-ambulant

Therapeutic Category
Natural History

Age
12-20

Mutation Specific
Mutation specific therapies, Amenable to treatments with Exon 53 skipping

Muscle Biopsy
No Muscle Biopsy Required

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