

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?

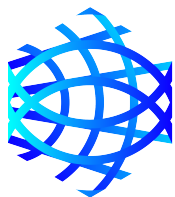
Inclusion Criteria

- 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


Trial Status


Trial complete

 **UK Locations**
London - GOSH, Trial complete/terminated

 **Trial Sponsor**
Genethon

 **Age**
12-20

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

 **Muscle Biopsy**
No Muscle Biopsy Required

 **Phase**
Observational

 **Length Of Participation**
TBC

 **Recruitment Target**
45

 **Ambulatory**
Non-ambulant

 **Therapeutic Category**
Natural History

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