

# 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  $\geq 12$  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](http://dmdhub.org).




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**Trial Status**  
Fully recruited

 **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

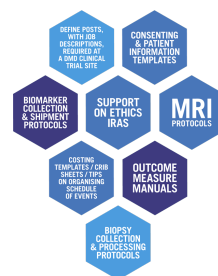
 **Phase**  
Observational

 **Length Of Participation**  
TBC

 **Recruitment Target**  
45

 **Ambulatory**  
Non-ambulant

 **Therapeutic Category**  
Natural History



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