Genethon - Natural History of Duchenne Muscular Dystrophy

A Prospective, Interventional, Baseline Study In Young Male Subjects Aged From 5 to 9 Years

Hub Summary

This natural history study is looking to collect data on the natural disease course in a cohort in young male subjects aged from 5 to 9 Years over a period of 6 to 36 months using disease appropriate evaluations.

Study Number: NCT03882827

Description by Genethon

Natural history studies are becoming more and more a critical prerequisite for the development of interventional therapies, as they provide the relevant knowledge of the course of a disease without an intervention. They are also used to develop and apply standardised outcome measures for assessing changes over time, which form the basis for monitoring therapeutic efficacy in patients undergoing an experimental treatment.

GNT-014; Natural History of Duchenne Muscular Dystrophy study can offer an up-to-date description of the progression of the condition according to concurrent standards of care, and provide baseline data on selected outcomes, prior entry of the subject into a phase I/II first in man study.

Primary Outcome Measures

NSAA scale [Time Frame: Screening 36 months]
NSAA scale (age appropriate modified North Star Ambulatory Assessment)

10 Meter Walk/ Run test (10MW/RT) [Time Frame: Screening 36 months]
Time function Test

6 Minutes Walk Test (6 MWT) [Time Frame: Screening 36 months]
Motor Function Measurement

Myoset : Myo-grip, -pinch [Time Frame: Inclusion 36 months]
Motor Function Measurement

ACTIMYO [Time Frame: Inclusion 36 months]
Motor Function Measurement

Muscle Imaging Nuclear Magnetic Resonance Imaging (NMRI) [Time Frame: Inclusion 36 months]
Muscle Imaging

Pulmonary Function Test (PFT) [Time Frame: Inclusion 36 months]
Respiratory Function Assessment

ECG - Echocardiography [Time Frame: Inclusion 36 months]
Cardiac Function Assessment

ACTIVLIM [Time Frame: Inclusion 36 months]
Patient Reported Outcome

EQ-5D [Time Frame: Inclusion 36 months]
Questionnaire of Life

Can I take part?

Inclusion Criteria

1. Male
2. 5 to 9 years old inclusive
3. Body-Weight < or = 75th percentile of BMI body-mass index scale (according to validated scale in force in the country site)
4. Diagnosis of DMD based upon Gene testing positive with detailed genotyping
5. Able to achieve:

Trial Status
Recruiting

UK Locations
London - GOSH, Recruiting, Newcastle, Recruiting

Trial Sponsor
Genethon

Length Of Participation
6 to 36 months

Recruitment Target
100 participants

Ambulatory
Ambulant

Therapeutic Category
Natural history study

Age
5 to 9 Years

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
Yes

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- NSAA (North Star Ambulatory Assessment) scale > or =18 (with a maximum of 2 points difference between inclusion and screening visits) and/or:
- Gowers test < or =7 sec
- 6 Minute Walk Test (6MWT) > or = 350 meters at screening visit (M1) and at inclusion visit (M0) with the distance being 20% of each other

6. Ongoing corticosteroid therapy or initiation of corticosteroid therapy according to standard of care in the previous 3 months
7. Signed informed consent by at least one parent (s) or both parents or legal guardian representative(s), when applicable and according to the country regulation
8. Affiliated Beneficiary of the National Health Care scheme

Exclusion Criteria

1. Cardiomyopathy based on physical cardiological examination and echocardiography with Left Ventricular Ejection Fraction (LVEF) below 55%
2. Respiratory Assistance: need for either a diurnal and/or a nocturnal ventilation
3. Any co-morbidity (ies) and or previous or planned surgical event(s) which may interfere with DMD natural evolution and or evaluation of outcomes designed to assess DMD Natural History
4. Muscle testing: inability to cooperate with
5. Nuclear Magnetic Resonance Imaging (NMRI): metal implants in regions of interest for the study
6. Unwilling and/or unable to comply with all the study protocol requirements and or procedures
7. Previous inclusion to another clinical trial with an Investigational Medicinal Product (IMP), within the 3 months (or IMP washout period) prior to the screening visit of the study
8. Concomitant participation to any other clinical trial

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

Duchenne UK