FOR-DMD

Finding the optimum regimen for Duchenne Muscular Dystrophy

Hub Summary

FOR-DMD study is designed to compare three different ways of giving corticosterioids to boys with DMD. The aim of this study is to see which method increases muscle strength the most and which produces the fewest side effects. The results of this study should provide patients and caregivers clearer information and guidelines about the best ways to take corticosteroids. The study will look at the following administration of corticosteroids:

- Prednisone 0.75mg/kg/day
- Prednisone 0.75mg/kg/day with 10 days on/10 days off treatment
- Deflazacort 0.9mg/kg/day

Study Number: NCT01603407

Description by University of Rochester

The Finding the Optimum Regimen for Duchenne Muscular Dystrophy (FOR DMD) study will compare three ways of giving corticosteroids to boys with Duchenne muscular dystrophy (DMD) to determine which of the three ways increases muscle strength the most, and which causes the fewest side effects. Using the results of this study, the investigators aim to provide patients and families with clearer information about the best way to take these drugs.

Boys with Duchenne muscular dystrophy experience progressive muscle weakness as they grow up. Corticosteroids have been shown to increase muscle strength in boys with DMD. Benefits include an increase in the length of time that boys could continue to walk, reduction in the development of curvature of the spine, a longer time of adequate breathing, and possible protection against the development of heart problems.

Doctors have tried different ways of prescribing corticosteroids in order to decrease undesirable side effects of the drug. Different doctors in different countries prescribe the drugs in different ways, and some do not prescribe corticosteroids at all. No controlled, long-term study has ever compared the different corticosteroids and regimes to see which one improves strength the most and which one causes the fewest side effects, over a long period of time.

The FOR DMD study compares the three most commonly prescribed corticosteroid regimes in DMD:

1. Prednisone 0.75mg/kg/day
2. Prednisone 0.75mg/kg/day switching between 10 days on and 10 days off treatment
3. Deflazacort 0.9mg/kg/day.

The study will take place at more than 30 academic medical centres in the United States, Canada, United Kingdom, Germany and Italy.

Boys participating in FOR-DMD may be able to participate in other Duchenne trials at the same time – concurrent participation is currently allowed in Italfarmaco's GIvinostat trial and Summit's SMTC 11005 study.

Primary Outcome Measures

- Three-dimensional (multivariate) outcome consisting of the following three components:

1. time to stand from lying (log-transformed)
2. forced vital capacity (FVC)
3. subject/parent global satisfaction with treatment, as measured by the Treatment Satisfaction Questionnaire for Medication (TSQM)

Secondary Outcome Measures

Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated, Alder Hey, Trial complete /terminated, Birmingham, Trial complete/terminated, Glasgow, Trial complete /terminated, Leeds, Trial complete/terminated, Manchester, Trial complete /terminated, Newcastle, Trial complete/terminated

Trial Sponsor
University of Rochester

Phase
3

Length Of Participation
36-60 months plus screening period

Recruitment Target
196

Ambulatory
Ambulant

Therapeutic Category
Steroids

Age
4-7

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

- Adverse event profile: occurrence and severity of known side effects of corticosteroids- including behaviour problems, bone fractures, weight gain, slow growth (hight restriction), cushingoid features, gastro-intestinal symptoms, cataracts, hypertension, immune /adrenal suppression and diabetes.
- Regimen tolerance assessed as: the ability to tolerate the prescibed dosage level.
- North Star Ambulatory Assessment (NSAA) - total score: 17 Item scale to reliably evaluate motor ability in ambulant children with DMD.
- 6 minute walk test (6MWT): measures the total distance walked in 6 minutes and the number of falls.
- Range of motion (goniometry): Range of motion at the ankle joint in dorsiflexion measured in degrees from plantigrade.
- Quality of life: measured by child self-report and by proxy (parent(s)/guardian(s)) report for all children. Utilising Generic Peds QoL (23 questions) and NMD Disease-specific PedsQL module (25 questions).
- Cardiac function: Monitored by trans-thoracic echocardiogram and 12-lead ECG.

Can I take part?

Inclusion Criteria

- ✔ Evidence of signed and dated informed consent form.
- ✔ Genetically confirmed diagnosis of Duchenne muscular dystrophy
- ✔ Age greater than or equal to 4 years and less than 8 years old
- ✔ Ability to rise independently from floor, from supine to standing
- ✔ Willingness and ability to comply with scheduled visits, drug administration plan and study procedures
- ✔ Ability to maintain reproducible FVC measurements.

Other inclusion criteria may apply.

Exclusion Criteria

- ✖ History of major renal or hepatic impairment, immunosuppression or other contraindications to corticosteroid therapy.
- ✖ History of chronic systemic fungal or viral infections.
- ✖ Diabetes mellitus.
- ✖ Idiopathic hypercalcuria.
- ✖ Lack of chicken pox immunity and refusal to undergo immunization.
- ✖ Evidence of symptomatic cardiomyopathy.
- ✖ Current or previous treatment (greater than four consecutive weeks of oral therapy) with corticosteroids or other immunosuppressive treatments for DMD or other recurrent indications (e. g., asthma).
- ✖ Inability to take tablets.
- ✖ Allergy/sensitivity to study drugs or their formulations including lactose and/or sucrose intolerance.
- ✖ Severe behavioural problems, including severe autism.
- ✖ Previous or ongoing medical condition, medical history, physical findings or laboratory abnormalities that could affect safety, make it unlikely that treatment and follow up will be correctly completed or impair the assessment of study results.
- ✖ Weight of less than 13 kilograms.
- ✖ Exposure to any investigational drug currently or within 3 months prior to start of study treatment, unless exposure to investigational drug is a result of participation in a clinical trial that has a concurrent participation in FOR-DMD agreement in place.

Other exclusion criteria may apply.

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

Duchenne UK

PDF created on 29/08/2025.