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Duchenne muscular dystrophy: double-blind randomized trial to find optimum steroid regimen

Submission date
26/06/2012

Recruitment status
No longer recruiting

Registration date
26/06/2012

Overall study status
Completed

Last Edited
20/06/2022

Condition category
Nervous System Diseases

[X] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English summary of protocol

Background and study aims
This is a study to compare three different current treatments for Duchenne muscular dystrophy (DMD), to see which is the most effective. We aim to provide evidence to ensure that boys with DMD are given the best and most effective treatment, and to create guidelines for the treatment of patients. We will look at the benefit of the treatment, and how happy subjects and parents are with the treatment that the boy receives. We will also look at the side effects of each treatment, and have taken known side effects, and their treatment, into consideration when designing the study, as well as standards of care for the general management of DMD.

Who can participate?
Boys with DMD, aged 4-7 years.

What does the study involve?
Participants will be randomly allocated to receive either daily prednisone, daily deflazacort or intermittent prednisone (10 days on, 10 days off). All boys will receive study medication for a minimum of three years (36 months). All boys entering the trial will remain on study drug until the last boy completes the 36 months of the study. This may be up to 60 months (five years). Subjects will receive study medication in the form of tablets to swallow, in wallets each containing enough tablets for 20 days. Boys will take between two and six tablets per day, depending on their weight. Subjects will visit the hospital for screening and baseline visits, then visits at three and six months, then every six months up to a maximum of 60 months, for various tests and assessments, and they and their parent(s)/guardian(s) will complete study questionnaires at each visit. The most important outcomes will be time to stand from lying, forced vital capacity (a lung function test), and subject and parent/guardian satisfaction with treatment.

What are the possible benefits and risks of participating?
Participating boys may see a benefit from their treatment. They may, of course, not see a benefit from their treatment, but will be contributing to the treatment of boys with DMD in the future.

Where is the study run from?
Study co-ordination is shared between the University of Rochester, Newcastle University (UK) and the University of Freiburg (Germany). It will be carried out at sites in the USA, Canada, the UK, Germany and Italy, over 36-60 months.

When is the study starting and how long is it expected to run for?
We expect to open to recruitment in late 2012. The study will run until November 2017.

Who is funding the study?
The study sponsor is the University of Rochester, NY, USA; the funder is the National Institutes of Health (NIH) National Institute of Neurological Disorders and Stroke (NINDS).

Who is the main contact?

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Contact information

Type(s)
Scientific

Contact name
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Additional identifiers

EudraCT/CTIS number
2010-023744-33

IRAS number

ClinicalTrials.gov number
NCT01603407

Secondary identifying numbers
12159

Study information

Scientific Title
Duchenne Muscular Dystrophy: double blind randomized trial to find optimum steroid regimen

Acronym
FOR-DMD

Study objectives
The aim of the study is to identify the optimum steroid regimen for boys with Duchenne muscular dystrophy (DMD), with regard to functional outcomes and participant/parent satisfaction.

More details can be found at http://public.ukcrn.org.uk/search/StudyDetail.aspx?StudyID=12159

Ethics approval required
Old ethics approval format

Ethics approval(s)
NRES Committee Southampton - South Central, First MREC approval date 14/12/201, ref: B11/SC /0543

Study design
Randomised; Interventional; Design type: Treatment

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details below to request a patient information sheet

Health condition(s) or problem(s) studied
Topic: Medicines for Children Research Network; Subtopic: All Diagnoses; Disease: Duchenne Muscular Dystrophy

Interventions
One of three steroid regimens:
1. Daily prednisone (0.75 mg/kg/day)
2. Intermittent prednisone (0.75 mg/kg/day, 10 days on, 10 days off)
3. Daily deflazacort (0.9 mg/kg/day)

The study treatment will last for a minimum of 36 months for all participants, and a maximum of 60 months for those recruited into the trial first (since it is anticipated that it will take up to two

years to recruit the 300 participants). The longer duration of blinded intervention and followup for those subjects recruited early is in anticipation of the continued longterm unblinded followup of the full cohort of subjects at the end of this five year study.

Intervention Type
Drug

Phase
Phase III

Drug/device/biological/vaccine name(s)
Prednisone, deflazacort

Primary outcome measure
Multivariate outcome measured six-monthly

Secondary outcome measures
1. Adverse Event profile
2. Regimen tolerance
3. Scondary functional outcomes

Measured six-monthly

Overall study start date
07/11/2012

Completion date
07/11/2017

Eligibility

Key inclusion criteria
1. Evidence of signed and dated informed consent form indicating that the subject and his parents or guardian (according to local legislation) have been informed about all pertinent aspects of the study. The child might be asked to give his assent, possibly in writing, if considered intellectually capable, in line with the legal requirements in the participating countries and with the permission of the parent(s)/guardian(s).
2. Confirmed diagnosis of Duchenne muscular dystrophy defined as: Male with proximal weakness and a confirmed DMD mutation in the dystrophin gene (out of frame deletion OR point mutation OR duplication) OR absent/< 3% dystrophin on muscle biopsy (by immunohistochemistry or Western blot).
3. Male, Age >= 4 years and < 8 years
4. Ability to rise independently from floor, from supine to standing, as assessed at screening visit
5. Willingness and ability to comply with scheduled visits, drug administration plan and study procedures (including laboratory tests, NSAA, 6MWT, ECG, Echo, wrist XRay, DXA, PedsQL and TSQM questionnaires) as assessed by the site investigator at the end of the screening period.
7. Ability to maintain reproducible FVC measurements. Boys must have reproducible measurements of FVC. The boy will be observed to insure complete understanding of the instructions and that he has given maximal effort. If the values continue to increase, the boy may be learning and testing will continue, if necessary, beyond the three required trials until the boy

reaches a plateau. The evaluator will use his/her expert judgment as to whether or not the boy can produce, and will likely be able to continue to produce, a reliable FVC measurement.

Participant type(s)
Patient

Age group
Child

Lower age limit
4 Years

Upper age limit
8 Years

Sex
Male

Target number of participants
Planned Sample Size: 300; UK Sample Size: 75

Key exclusion criteria
1. History of major renal or hepatic impairment, immunosuppression or other contraindications to corticosteroid therapy
2. History of chronic systemic fungal or viral infections. Acute bacterial infection (including TB) would exclude from enrollment until the infection had been appropriately treated and resolved
3. Diabetes mellitus
4. Idiopathic hypercalcuria
5. Lack of chicken pox immunity and refusal to undergo immunization
6. Evidence of symptomatic cardiomyopathy at screening assessment. Asymptomatic cardiac abnormality on investigation would not be an exclusion
7. 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)
8. Inability to take capsules, as assessed by the site investigator by the end of the screening period
9. Allergy/sensitivity to study drugs or their formulations including lactose and/or sucrose intolerance
10. Severe behavioral problems, including severe autism
11. 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, in the judgment of the site investigator.
12. Weight of less than 13 kilograms
13. Exposure to any investigational drug currently or within 3 months prior to start of study treatment

Date of first enrolment
07/11/2012

Date of final enrolment

07/11/2017

Locations

Countries of recruitment
England

United Kingdom

Study participating centre
4th Floor William Leech Building
Newcastle Upon Tyne
United Kingdom
NE2 4HH

Sponsor information

Organisation
University of Rochester (USA)

Sponsor details
518 Hylan Building
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Sponsor type
University/education

ROR
https://ror.org/022kthw22

Funder(s)

Funder type
Government

Funder Name
National Institute of Health (USA)

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan
Not provided at time of registration

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		19/04/2022	20/06/2022	Yes	No
HRA research summary			28/06/2023	No	No