Testosterone therapy for Pubertal delay in DMD

Hub Summary

This observational study is looking at the outcomes of testosterone treatment in boys with DMD. The study is following the progression of adolescent males with DMD and delayed puberty. These patients are treated with testosterone to induce puberty. The participants are treated with the standard regiment of testosterone and this study is collecting data to review the effectiveness and tolerability of the current treatment regimen.

The researchers will use the results to explore what effect testosterone treatment has on pubertal development, growth, muscle strength and function, bone mineral density and body composition. The study also aims to define and understand side effects testosterone treatment may have.

Study Number: NCT02571205

Description by Newcastle-upon-Tyne Hospitals NHS Trust

"Observational study of clinical outcomes for testosterone treatment of pubertal delay in Duchenne Muscular Dystrophy" is a single centre observational study that aims to follow the progress of 20 adolescents with Duchenne Muscular Dystrophy (DMD) and delayed puberty who are treated by the Newcastle muscle team, as they are treated with testosterone to induce puberty. The participants will all be treated with the standard stepwise regimen of testosterone injections every 4 weeks and data will be collected to help determine the effectiveness and tolerability of the current treatment regimen. The investigators will use the data to explore the effect of testosterone on pubertal development, growth, muscle strength and function, bone mineral density and body composition and characterise any side effects. Semi-structured interviews will also be carried out to learn the boys' views on the tolerability of the regimen. The study will last up to a maximum of 27 months in total for each participant, but may be less if they are happy with pubertal development before this time. It is important to do this study because from the investigator's limited experience in this group, testosterone treatment seems to be well liked and tolerated but the best treatment regimen to use remains unknown and there is no current consensus. It is not currently part of the standard of care in DMD but it would be important to include it if this study can show that it is an effective treatment for pubertal delay.

Primary Outcome Measures

- Total score in the Treatment Satisfaction Questionnaire for Medication (TSQM)

Secondary Outcome Measures

- Subject's reported effectiveness of testosterone therapy as assessed by semi-structured interviews pre and post treatment.
- Total score in Northstar Ambulatory Assessment or Performance of the Upper Limb if non-ambulant.
- Z-score from Bone mineral adjusted density of the lumbar spine and total body (minus head) using Dual Xray Absorptiometry (DXA).
- Percentage of body mass assessed by DXA.
- Osteocalcin level, measured by blood test.
- P1NP level, measured by blood test.
- Percentage fat fraction as assessed by muscle Magnetic Resonance Imaging (MRI) of upper and lower limbs.
- Pubertal staging assessed using Tanner staging and testicular volume.
- Bone age as assessed by wrist and hand X-Ray.
- Hormonal assessment of pubertal staging using testosterone level.
- Forced vital capacity, measured by spirometry.
- Cardiac function, assessed by Electrocardiogram (ECG) and echo.

Can I take part?

Trial Status
Trial complete

UK Locations
Newcastle, Trial complete/terminated

Trial Sponsor
Newcastle-upon-Tyne Hospitals NHS Trust

Age
12-17

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
Yes

Phase
Observational

Length Of Participation
27 months

Recruitment Target
15

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Observational study

Inclusion Criteria

- A molecular diagnosis of Duchenne Muscular Dystrophy
- Males aged between 12 and 17 years of age at time of first dosing
- Prepubertal (Tanner stage 1, testicular volume <4 mls, initial testosterone level of <2.0 nmol/l)
- Subjects are receiving the standard of care for DMD as recommended by the NorthStar UK and TREAT-NMD guidelines
- Patients are capable of sitting upright in a wheelchair for at least an hour
- Patients have stable respiratory function. Artificial ventilation with either Bipap/continuous positive airways pressure (CPAP) or tracheostomy is not a contraindication to the study
- Informed consent/assent signed by the patient (or parent/guardian if under 16 years of age)

Exclusion Criteria

- Severe learning difficulties that would preclude them from cooperating with examination.
- Anticipated surgery during the study period
- Symptomatic cardiac failure
- Participants/families who may have emotional or psychological problems if recruited to a study
- Hypersensitivity to the active substance or to any of the excipients, including arachis oil or derivatives (including hypersensitivity and allergy to peanuts or soya.)
- Any contra-indication to receiving an intramuscular injection
- Any additional chronic disease that affects androgen production
- Anti-coagulant therapy
- If participation in the study is not recommended in the opinion of the investigators

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

Duchenne UK

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