Testosterone in DMD follow up study

Long term observational extension study of gonadal function after pubertal induction in Duchenne Muscular Dystrophy

Hub Summary

This study will be investigating the effects of testosterone being used over the course of 2 years to bring on puberty in boys with DMD. Puberty is often delayed in boys with DMD, and is a side effect of taking steroids, which are part of the Standards of Care. This study will be looking not only at the physical effects of taking testosterone, but also the emotional and psychological effects.

Please note this trial is only open to boys who took part in the original observational study (NCT02571205) and is by invitation only.

Study Number: Not on ClinicalTrials.gov

Description by Newcastle upon Tyne Hospitals NHS Foundation Trust

This is an observational study, designed to investigate the longer term effects of testosterone replacement therapy after treatment for delayed puberty in DMD. The aim is to continue to monitor testosterone levels, growth, muscle strength, mass and function, bone mineral density, body composition and quality of life in a cohort of young men with DMD, for two years after they have completed a previous clinical trial (NCT02571205) where they were given testosterone by injection to induce puberty. The extension to the original study will enable to investigate the longer term consequences of testosterone treatment after earlier induction of puberty.

Primary Outcome Measures

Evaluate the effect of previous pubertal induction on:

1. Hormonal assessment of gonadal function (measured by morning blood levels of testosterone, LH, FSH, AMH and Inhibin B)
2. Physical assessment of gonadal function (measured by testicular volume)

Secondary Outcome Measures

Evaluate the effect of previous pubertal induction on:

1. Quality of life and patient satisfaction as measured by Treatment Satisfaction Questionnaire for Medication (TSQM) and PEDSQoL questionnaires
2. Motor function as measured by standardised physiotherapy assessments (Northstar Ambulatory Assessment (NSAA), Performance of the Upper Limb (PUL) EK scale, timed tests (Time to Rise from the Floor, Time to run/walk 10 meters) and assessment of workable reach space using Kinect
3. Muscle strength as measured by hand-held myometry (knee flexion and extension, elbow flexion and extension, shoulder abduction) and grip strength
4. Bone mineral adjusted density of the lumbar spine and total body (minus head) using DXA and bone maturation using wrist x-ray.
5. Assessment of lean versus total body mass assessed by DXA
6. Bone turnover markers (bone-specific alkaline phosphatase, beta crosslaps, osteocalcin, P1NP, RANKL, OPG, sclerostin)
7. Muscle volume and fat fraction as assessed by muscle MRI of upper and lower limbs
8. Cortical and trabecular bone structure assessed by spinal x-ray
9. Trabecular bone microarchitecture assessed by bone MRI, in subset of patients
10. Forced vital capacity, forced expiratory volume and peak cough flow, measured by spirometry

Trial Status
Trial complete

UK Locations
Newcastle, Trial complete/terminated

Trial Sponsor
Newcastle upon Tyne Hospitals NHS Foundation Trust

Age
12-17

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
Yes

Phase
Observational

Length Of Participation
24 months

Recruitment Target
15

Ambulatory
Ambulant and non-ambulant

dmdhub.org

Can I take part?

Inclusion Criteria

Any participant enrolled to the original “Observational study of clinical outcomes for testosterone treatment of pubertal delay in Duchenne Muscular Dystrophy-NCT02571205”.

Exclusion Criteria

- Severe learning difficulties that would preclude participants from co-operating with examination.
- Participants/families who may have emotional or psychological problems if recruited to a study
- 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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