Non-invasive assessment of skeletal muscle involvement in Duchenne muscular dystrophy

Published: 08-07-2009
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To develop non-invasive markers for tissue changes in skeletal muscle using MR methods

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Muscle disorders
Study type	Observational non invasive

Summary

ID

NL-OMON39843

Source
ToetsingOnline

Brief title
Skeletal muscle MRI in DMD

Condition

- Muscle disorders

Synonym
Duchenne Muscular Dystrophy

Research involving
Human

Sponsors and support

Primary sponsor: Leids Universitair Medisch Centrum
Source(s) of monetary or material Support: Prinses Beatrixfonds

Intervention

Keyword: Duchenne muscular dystrophy, MRI, Muscle status

Outcome measures

Primary outcome

Quantitative assessment of pathological changes in skeletal muscle of DMD

patients by assessment of:

- the level of fatty infiltration

- T2 relaxation parameters as a marker for muscle edema

- individual muscle volume

Secondary outcome

Variation of MR parameters between different muscles, or between different ages

in DMD patients.

Correlation of MR parameters with strength measurements.

Differences of T2 relaxation parameters between steroid users and non-steroid

users

Explore the use of other MR muscle parameters like diffusion tensor imaging.

Study description

Background summary

Duchenne muscular dystrophy (DMD) is a debilitating disease caused by mutations in the dystrophin gene. The disease is characterized by severe and progressive muscle weakness and patients have a reduced life expectancy of about 27 years. Recently, the Leiden Duchenne research group showed the first successful restoration of dystrophin expression in skeletal muscle in humans. For the further development of therapies it is imperative to develop a non-invasive tool that is capable of quantitatively assessing tissue changes in skeletal muscle. The development of new drugs for DMD patients requires accurate and

repeated assessment of changes in skeletal muscle. The current gold standard, via muscle biopsies, only yields local information, is invasive and cannot be repeated often, especially not in children. The current CT or MRI techniques only analyze small selected parts of muscle with a non-quantitative technique. A non-invasive and quantitative method is imperative to enable accurate long term assessment of therapy effectiveness.
It is almost a year ago that the experimental treatment in the OLE study has been terminated and there are advanced plans to restart the experimental treatment in these boys. With a view on the possible effect of the treatment on the inflammation process and fat infiltration of the muscles in these boys. This addendum offers the unique chance to determine the clinical applicability of the recently developed non-invasive MR technique and to further evaluate this in a target population. This will ultimately test MRI as outcome measure.

Study objective

To develop non-invasive markers for tissue changes in skeletal muscle using MR methods

Study design

Observational, case-control study

Study burden and risks

There are no known risks associated with participating in an MRI study. Subjects with intracranial or intraocular metal, a pacemaker, and claustrophobia will be excluded because of potential contraindications of MRI in such subjects. The Nederlandse Vereniging voor Kindergeneeskunde (NVK) code of conduct; Gedragscode verzet bij minderjarigen die deelnemen aan medisch-wetenschappelijk onderzoek will be applied to this study.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

DMD patients of 8 years and older with typical muscle weakness and a known genetic mutation in the dystrophin gene.
Healthy boys of 8 years or older form the control group.

Exclusion criteria

General exclusion criteria are:
- Claustrophobia
- Pacemakers and defibrillators
- Nerve stimulators
- Intracranial clips
- Intraorbital or intraocular metallic fragments
- Cochlear implants
- Ferromagnetic implants (e.g. thoracic implant for scoliosis)
- Inability to lie supine during less than 60 minutes;Exclusion criteria for healthy controls
- any muscle disease
- recent muscle trauma

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)

Primary purpose: Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	01-11-2009
Enrollment:	60
Type:	Actual

Ethics review

Approved WMO Date:	08-07-2009
Application type:	First submission
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	17-04-2012
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	13-11-2013
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	10-11-2014
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO	

Date:	31-08-2015
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL28198.058.09

Study results

Date completed:	16-03-2016
Actual enrolment:	38

Summary results
Trial is onging in other countries