Myotonic Dystrophy, PrOteiN and Diet study. Part 1.

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Neurological disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON50601

Source
ToetsingOnline

Brief title
MD-POuND

Condition

- Neurological disorders congenital
- Muscle disorders
- Neuromuscular disorders

Synonym
'myotonic dystrophy type 1' en 'DM1'

Research involving
Human

Sponsors and support

Primary sponsor: Medisch Universitair Ziekenhuis Maastricht
Source(s) of monetary or material Support: Prinses Beatrix Spierfonds

Intervention

Keyword: body composition, metabolism, myotonic dystrophy type 1, nutrition

Outcome measures

Primary outcome

The main study endpoints are total energy expenditure (TEE), resting metabolic rate (RMR) and substrate selection at rest.

Secondary outcome

Secondary endpoints include physical activity level (PAL) measured by accelerometer, whole-body skeletal muscle mass measured by MRI, quadriceps muscle cross-sectional area measured by CT, maximal grip strength measured by dynamometer, body composition expressed as leg and whole-body lean tissue mass measured by DEXA, muscle tissue morphology, muscle fibre differentiation, muscle fibre type specific cross-sectional area and vascularization, muscle fiber biochemical analysis, maximal oxygen uptake capacity (VO2 max) and aerobic and anaerobic ventilatory thresholds determined by cycling exercise test and muscle strength measured by1-RM (only applicable for DM1 affected subjects taking part in the training program pilot).

Study description

Background summary

Myotonic dystrophy type 1 (DM1) is an autosomal dominant disorder that affects the skeletal, cardiac, and smooth musculature and many other tissues. While muscle weakness and myotonia (inability to relax muscles) are the main characteristics of disease, patients with DM1 frequently experience marked loss of muscle, as well as issues regarding nutrition and weight. Both a pilot study and literature suggest that observed nutritional and weight problems might be

caused by metabolic abnormalities.

Study objective

This study aims to assess metabolism in patients with DM1, in resting state, in the normal situation and during exercise, taking into account muscle mass, and compared to healthy age- and gender-matched subjects. We hypothesize that DM1 is accompanied by impairments in whole-body and/or muscle metabolism.

Study design

The study design is a cross-sectional case control study.

Study burden and risks

All participants will undergo a 15-day study period. Initially, participants will stay in a respiration chamber during 24 hours. Thereafter the study period continues with 14 days under normal free-living conditions, during which energy expenditure is determined by doubly labeled water and accelerometer. Throughout these 14 days, there will only be one hospital visit. On the 15th day, determination of muscle status will take place in the hospital through MRI, CT, dynamometry, DEXA scan, muscle biopsy and exercise test.
Moreover, DM1 affected subjects will be offered to take part in a pilot exercise program for a period of 6 weeks, training twice a week, after completion of the initial 15-day study period.
Despite the conduction of multiple experiments, the risks involved in participating in this study are minimal, as most carried out experiments are usually part of standard care, with exception of doubly labeled water and respiration chamber stay. Last mentioned tests are both considered safe for humans.
Eventually, study results will serve as the basis for the development of a targeted nutritional intervention in the future.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

DM1 affected subjects:
- Age 18 years or older.
- Legally competent adult.
- Defined DM1 of the adult subtype.
- Participants must be able to walk and to cycle (in order to perform exercise tests).
- Participants must give informed consent by signing and dating an informed consent form. Healthy control group:
- Age 18 years or older.
- Legally competent adult.

Exclusion criteria

- Implantation of pacemaker or ICD device, a implantable insulin device, a neurostimulator, internal hearing aid or artificial heart valve.
- Implantation of orthopaedic prostheses, screws or plates.
- Metal shreds or splinters inside the body.
- Vascular clips in the body.
- Big tattoo*s and/or permanent make-up.
- Claustrophobia.
- Use of medication interacting with muscle metabolism (such as steroids and statins).
- Diabetes mellitus.

- Weight loss of more than 3 kg in the last three months.
- Pregnant or lactating women.
- Use of protein supplements.
- Participation in an exercise program.
- Patients who are not able to perform basic activities of daily living such as walking or patients who are suffering from other disabling comorbidity that seriously hamper physical exercise (e.g. heart failure, coronary artery disease, chronic obstructive pulmonary disease (COPD), orthopedic conditions).
- Body mass index (BMI) <18 or >35.
- Use of oral anticoagulants.
- In case of DM1 affected subjects: muscular impairment rating scale (MIRS) score of 5 (which represents severe proximal muscle weakness).
- In case of the healthy control group: presence of a neuromuscular disorder or an abnormal neurological examination (specifically if muscle weakness is present), or other condition possibly interfering with muscle strength or muscle mass.

Study design

Design

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

Primary purpose: Other

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	01-02-2019
Enrollment:	30
Type:	Actual

Ethics review

Approved WMO

Date:	27-06-2018

Application type:	First submission
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)

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	NL65617.068.18