Clinical Trial Readiness to Solve Barriers to Drug Development in facioscapulohumeral muscular dystrophy

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Musculoskeletal and connective tissue disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON46457

Source
ToetsingOnline

Brief title
ReSolve FSHD

Condition

- Musculoskeletal and connective tissue disorders congenital
- Musculoskeletal and connective tissue disorders congenital

Synonym
FSHD, Landouzy-Dejerine disease

Research involving
Human

Sponsors and support

Primary sponsor: Neurologie
Source(s) of monetary or material Support: Friends of FSH Research

Intervention

Keyword: clinical trial readiness, facioscapulohumeral muscular dystrophy, outcome measures

Outcome measures

Primary outcome

Reliability, validity, responsiveness of the newly developed functional outcome measure FSHD-COM will be assessed.

Secondary outcome

The FSHD-COM will be correlated and compared to traditional outcome measures used in FSHD, including manual muscle testing, quantitative muscle testing, patient-reported outcomes (PROMIS57, upper extremity functional index UEFI, FSHD-health index FSHD-HI), clinical severity score and FSHD clinical score.

Study description

Background summary

Facioscapulohumeral muscular dystrophy (FSHD) is an autosomal dominant inherited progressive muscular dystrophy. Several pharmaceutical companies have active programs for targeted treatments in FSHD, with time-frames for first-in-human trials starting in the next 5 years. There are several gaps that need to be addressed to accelerate efficient drug development and confirmatory drug trials. As drugs move from preclinical planning into human trials, it is essential that we validate clinical trial tools and methodology to hasten the drug development process. This study on clinical outcome measures will provide knowledge on the multi-site reliability of the clinical outcome measures, validity, and sensitivity to change, and define subgroups more likely to have consistent disease progression.

Study objective

The primary goal is to hasten drug development in FSHD by evaluating the usefulness of various outcome measures, validating new clinical outcome measures, and to elucidate cohort characteristics useful for determining

eligibility criteria to improve trial efficiency.

Study design

This study is an international multi-site prospective, longitudinal observational cohort study with a follow-up of 18 months.

Study burden and risks

Participants will be asked for 4 visits to the outpatient clinic at the department of neurology. Their medical history will be taken, they will undergo clinical examination and they will fill out questionnaires. Blood samples will be collected at each visit. DEXA scanning will be performed at two of the visits (baseline and 12 moths). Risks to subjects may be physical (e.g., bruising during muscle strength testing) or psychological (e.g., receiving confirmation of degree of muscle weakness). There is a small risk of bruising from blood draws. DEXA scanning uses a very small amount of radiation. All procedures will be performed according to standard accepted techniques to minimize risk exposure for subjects. We classify the risk of this study as negligible.

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

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

Inclusion criteria

• Clinical diagnosis of FSHD1 with genetic confirmation as previously described for FSHD110
• Age 18-75 years
• Symptomatic limb weakness
• Able to walk 30 feet without the support of another person.

Exclusion criteria

• Cardiac or respiratory dysfunction
• Orthopedic conditions that preclude safe testing of muscle function
• Regular use of available muscle anabolic/catabolic agents such as corticosteroids, oral testosterone or derivatives, or oral beta agonists
• Use of an experimental drug in an FSHD clinical trial within the past 90 days
• Pregnancy.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	03-07-2018
Enrollment:	25
Type:	Actual

Ethics review

Approved WMO Date:	12-04-2018
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	28-07-2020
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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	NL64221.091.18