Evaluation of dysphagia in inclusion body myositis and oculopharyngeal muscle dystrophy by combining novel ultrasound and real-time MRI

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

Summary

ID

NL-OMON45446

Source
ToetsingOnline

Brief title
RT-MRI and ultrasound evaluation of dysphagia in IBM and OPMD

Condition

- Muscle disorders
- Neuromuscular disorders

Synonym
dysphagia, swallowing disorder

Research involving
Human

Sponsors and support

Primary sponsor: Neurologie
Source(s) of monetary or material Support: AFM (association francaise contre les myopathies)

Intervention

Keyword: diagnosis, dysphagia, inclusion body myositis, oculopharyngeale muscle dystrophy

Outcome measures

Primary outcome

A. to establish the pattern of weakness in a predefined set of muscles in

patients with IBM with and without clinical evidence of dysphagia.

B. to compare the findings in dysphagic IBM patients with another neuromuscular

disease that causes dysphagia: OPMD.

C. to evaluate the use of novel diagnostic tools in dysphagia, to develop a

diagnostic protocol in neuromuscular diseases to evaluate dysphagia with

minimal invasive techniques.

Secondary outcome

A. to promote international collaboration to expand the existing cohorts of

patients affected by these relatively rare diseases.

B. the creation of mechanism-based hypothesis on new treatment strategies in

dysphagia in IBM and OPMD, which might give rise to a follow-up trial.

C. to investigate various outcome measures in IBM with the ultimate goal to

find a sensitive, validated and relevant dysphagia outcome measure, with a high

sensitivity to change, that can be used in upcoming therapeutic trials.

Study description

Background summary

Sporadic inclusion body myositis (IBM) is the most frequently occurring acquired myopathy in patients aged over 50 years. The disease is characterised by slowly progressive asymmetric muscle weakness, especially the finger flexors and the quadriceps are affected early in the disease course. Dysphagia is present in a large subset of patients and can even precede the onset of generalized weakness with a few years. Oculopharyngeal muscular dystrophy (OPMD) is another neuromuscular disease that equally affects patients at and advanced age. The most important clinical characteristics are proximal weakness, ptosis and dysphagia. Dysphagia can lead to various problems, like dietary changes, pneumonia and malnutrition. In both diseases, the cause and pathophysiology of dysphagia i.e. the pattern of affected muscles is unknown. Up to recently, the analysis of dysphagia was restricted to clinical observation, combined with videofluoroscopy and oesophageal manometry. These techniques have some limitations, for example, soft tissues are not optimally imaged and patients perceive this kind of investigations as burdensome.

Study objective

In order to develop treatment strategies guided by the key mechanisms that play a role in dysphagia in OPMD and IBM, the exact pattern of weakness should be identified. The current protocol allows us to test the feasibility of two non-invasive techniques, quantitative muscle ultrasound (QMUS) and real-time magnetic resonance imaging (RT-MRI), to assess swallowing in detail. This can lead to the development of new diagnostic protocols and it will contribute to the development of validated outcome measures.

Study design

The included patients will undergo a series of testing (physical examination, questionnaire, swallowing tests, quantitative muscle ultrasound, real-time MRI).

Study burden and risks

The risks in this protocol are related to RT-MRI: swallowing of a small quantity of fluids imposes a risk of aspiration of fluids in patients with severe dysphagia. Furthermore, a first claustrophobic reaction could occur during the MRI-scan. The scan will take place in Göttingen, which means the participants will have to travel between the study sites. These risks and burdens are limited, as the expected results of the study (especially the development of non-invasive assessment of swallowing) will be of immediate

benefit to the patients.

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

patients diagnosed with IBM based on the ENMC 2011 criteria *clinicopathologically* or *clinically defined* IBM AND patients diagnosed with OPMD - genetic confirmation (PABPN1 mutation present) who can also be participating in the *Projet Stratégique eOPMD: Pathophysiology and therapeutic approaches in Oculopharyngeal Muscular Dystrophy*

Exclusion criteria

presence of diseases that might significantly influence the results of the current study, including: another neuromuscular disorder other than IBM/OPMD, another degenerative disease that might impede swallowing, significant intellectual impairment which impedes the performance of testing; contraindications to undergo MRI-scanning: the presence of metal (including metal splinters in the eye for example), the presence of electronic devices (e.g. a pacemaker), old clipping of cerebral vessels, body weight >140kg; a known pineapple-juice intolerance of allergy.

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	20-11-2017
Enrollment:	20
Type:	Actual

Ethics review

Approved WMO Date:	24-04-2017
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	25-07-2018
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	NL60136.091.17