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The natural history of inclusion body myositis

Submission date
23/08/2012

Recruitment status
No longer recruiting

Registration date
21/02/2013

Overall study status
Completed

Last Edited
11/04/2022

Condition category
Nervous System Diseases

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English summary of protocol

Background and study aims
Inclusion body myositis (IBM) is the most common muscle disease beginning in those aged over 50. It leads to progressive disability with, classically, a characteristic pattern of muscle involvement. IBM is still poorly understood and its cause unknown. At present, there is no conclusive diagnostic test and it has no treatment. Furthermore, information on the pattern and prognosis of IBM is based more on anecdote from clinical experience, rather than on firm fact. The largest published series of data on the natural history of the illness followed only 11 patients for six months. This study seeks to better characterise the condition by gathering data from as many cases of IBM as possible. This will build an important resource and so form the starting point for future studies of the illness.

Who can participate?
Any person who meets the established diagnostic criteria for inclusion body myositis

What does the study involve?
Participants are asked to volunteer to undergo a standardised assessment at least annually for a five-year period. Background information is collected on the history of the illness and any other medical conditions, plus how IBM currently affects everyday tasks and the findings of a physical examination. As well as this data, participants are also asked to donate a small blood sample for storage and extraction of DNA and serum. The serum and DNA samples are stored for used in future studies of the disease. All data is recorded on a secure central computer database. This is a multi-centre study and to allow as many people as possible to participate data entry is possible by other muscle disease specialists around the UK (over a secure internet link). This allows people to be seen near to home. Alternatively, the necessary data can be sent to the hospital for the researchers to enter into the database. The assessments are repeated over five years.

What are the possible benefits and risks of participating?
This study will improve knowledge of IBM and allow doctors to give a more accurate prediction of the likely progression of IBM. In time, analysis of the data plus studies of the DNA or serum gathered for the study may contribute further. Such future follow-on studies offer the possibility of identifying risks for developing IBM and could help generate interventions to reduce the disability IBM causes. The only burden to participants is the inconvenience posed by an additional annual trip to hospital for the assessment, and the discomfort of undergoing a single blood test.

Where is the study run from?
The study is run from The National Hospital for Neurology and Neurosurgery in London, with the inclusion of other participating centres around the UK

When is the study starting and how long is it expected to run for?
January 2012 to April 2022

Who is funding the study?
1. Muscular Dystrophy Campaign (UK)
2. National Institute for Health Research (NIHR) Rare Diseases Translational Research Collaboration (UK)

Who is the main contact?
1. Dr Pedro Machado (p.machado@ucl.ac.uk)
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Scientific

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Additional identifiers

Protocol serial number
11688

Study information

Scientific Title
The natural history of inclusion body myositis: an observational cohort study

Study objectives
Inclusion body myositis (IBM) is the most common muscle disease beginning in those aged over 50. However, it is still poorly understood and its cause is unknown. Furthermore, IBM has no treatment and leads to progressive disability.

To date, the largest published study of the illness followed only 11 patients for six months. Thus, information on the pattern and prognosis of IBM is based on anecdote from clinical experience, rather than firm fact.

This project seeks to better characterise the condition by gathering data from as many cases of IBM as possible. This will build a crucial resource and form the starting point for future studies of the illness.

Ethics approval required
Old ethics approval format

Ethics approval(s)
East Central London Research Ethics Committee, 07/06/2010, ref: 10HO72128

Study design
Non-randomised observational cohort study

Primary study design
Observational

Study type(s)
Other

Health condition(s) or problem(s) studied
Inclusion body myositis

Interventions
Participants in the project will be asked to volunteer to undergo a standardised assessment at least annually for a five-year period. This will consist of background information on the history of the illness and any other medical conditions, plus how IBM currently affects everyday tasks and the findings of a physical examination. All data will be recorded on a secure central computer database. To allow as many people as possible to participate in the study data entry will be available (over a secure internet link) to other medical specialists around the UK, so people can be seen nearer to home and information entered locally. Alternatively, the necessary clinical data can be sent to the trialist's hospital to enter into the database. By repeating the

assessments over five years, the trialists will be able to give a much more reliable and accurate prediction of the course of the disease. As well as this clinical data, participants will also be asked to donate a small blood sample for storage and extraction of DNA and serum. The serum and DNA samples will be stored for used in future studies of the disease.

Intervention Type
Other

Phase
Not Specified

Primary outcome(s)
Rate of strength decline over time

Key secondary outcome(s))
No secondary outcome measures

Completion date
01/04/2022

Eligibility

Key inclusion criteria
Any person who meets the established diagnostic criteria for inclusion body myositis. The age criteria set above in section A16 are to allow for one criterion being age at onset of over 30, while the upper limit is set so as not to exclude any participant on the grounds of being too old.

Griggs' diagnostic criteria for IBM:

Clinical features:
1. Illness duration of more than six months
2. Male and female, age at onset greater than 30 years
3. Proximal and distal weakness of arms and legs, with finger flexion weakness, wrist flexion more than extension weakness, and quadriceps weakness.

Laboratory features:
1. Creatine kinase (CK) less than 12 times normal
2. Neurophysiology consistent with myopathy

Muscle biopsy features:
1. Inflammation with mononuclear cell invasion of non-necrotic fibres
2. Vacuolated fibres
3. Intracellular amyloid and/or 1518 nm filaments on electron microscopy

If all the muscle biopsy features above are present, then the disease is labelled as "definite" (regardless of the presence or absence of other criteria). To meet the "probable" standard of disease, then the muscle biopsy must show at least inflammation and vacuolation of fibres, plus all the clinical and laboratory features above.

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
30 years

Sex
All

Key exclusion criteria
Does not meet inclusion criteria

Date of first enrolment
27/01/2012

Date of final enrolment
01/04/2017

Locations

Countries of recruitment
United Kingdom

England

Study participating centre
National Hospital for Neurology and Neurosurgery
London
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Sponsor information

Organisation
University College London Hospitals NHS Foundation Trust (UK)

ROR
https://ror.org/042fqyp44

Funder(s)

Funder type
Charity

Funder Name
Muscular Dystrophy Campaign (UK)

Funder Name
National Institute for Health Research (NIHR) Rare Diseases Translational Research Collaboration

Results and Publications

Individual participant data (IPD) sharing plan
The current data sharing plans for the current study are unknown and will be made available at a later date.

IPD sharing plan summary
Data sharing statement to be made available at a later date

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	01/05/2013		Yes	No