# The natural history of inclusion body myositis

Submission date	Recruitment status  No longer recruiting	Prospectively registered		
23/08/2012		☐ Protocol		
Registration date	Overall study status	Statistical analysis plan		
21/02/2013	Completed	[X] Results		
Last Edited	Condition category	Individual participant data		
11/04/2022	Nervous System Diseases			

#### 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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## Contact information

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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 United Kingdom WC1N 3BG

# 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
Participant information sheet	Participant information sheet	11/11/2025 11/1	11/2025 No	Yes