

Arimoclomol for inclusion body myositis (IBM)

Submission date 02/02/2011	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
Registration date 02/02/2011	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 04/10/2017	Condition category Musculoskeletal Diseases	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data

Plain English summary of protocol

Not provided at time of registration

Contact information

Type(s)

Scientific

Contact name

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Additional identifiers

EudraCT/CTIS number

2008-008208-42

IRAS number

ClinicalTrials.gov number

NCT00769860

Secondary identifying numbers

7748

Study information

Scientific Title

A randomised, double blinded, placebo-controlled pilot study assessing the safety and tolerability of arimoclomol in adult patients with inclusion body myositis

Study objectives

Sporadic inclusion body myositis (IBM) is the commonest muscle disease acquired by those aged over 50 years. Nevertheless, despite being the subject of several clinical trials, it remains without any proven treatment. This study seeks to make the first assessment of a novel therapeutic compound, arimoclomol, in IBM. This is the first potential treatment of IBM not to target purely inflammation.

The primary objective of this proposed study is to assess the safety and tolerability of arimoclomol (100 mg three times a day [TDS]). The secondary objective is to determine whether arimoclomol has its anticipated pharmaceutical action to augment the concentration of key heat shock proteins (HSPs) in muscle tissue of IBM patients. The further objective is to evaluate a framework of clinical assessment, including measures of muscle strength, which can be used for subsequent practical and statistical planning of a larger future study of efficacy.

The study will include 12 patients with IBM, 8 of whom will receive arimoclomol and 4 a matching placebo over 4 months. The primary outcome measure will be adverse event reporting. Secondary outcome measures will include muscle strength testing (by manual muscle testing and myometry), muscle mass measure (DEXA), IBM functional rating scale, and muscle biopsies pre- and post-treatment to measure levels of HSP70, and to assess pathological changes in muscle fibres.

More details can be found here: <http://public.ukcrn.org.uk/Search/StudyDetail.aspx?StudyID=7748>

Ethics approval required

Old ethics approval format

Ethics approval(s)

The Joint UCL/UCLH Committees on the Ethics of Human Research (committee A) - currently Central London REC 4 - approved (ref: 09/H0714/22)

Study design

Randomised multicentre interventional treatment trial

Primary study design

Interventional

Secondary study design

Randomised controlled trial

Study setting(s)

Other

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use the contact details below to request a patient information sheet

Health condition(s) or problem(s) studied

Topic: Musculoskeletal; Subtopic: Musculoskeletal (all Subtopics); Disease: Musculoskeletal

Interventions

Twelve subjects will be randomised to one of two groups: placebo (4 patients) or Arimoclomol 100 mg three times daily (TDS) (8 patients). Participants will receive study medication for 4 months. During the treatment trial patients will be seen at screening, day 0 (baseline) and months 0.4, 1, 1.5, 2, 2.5, 3, 3.5 and 4. After the 4 month drug treatment trial, patients will be followed monthly for 8 months in order to obtain clinical endpoint measures for a total of 12 months. Muscle biopsies will be obtained at baseline (day 0) and month 4.

Follow-up length: 8 months

Study entry: registration and one or more randomisations

Intervention Type

Drug

Phase

Phase II

Drug/device/biological/vaccine name(s)

Arimoclomol

Primary outcome measure

Adverse events, measured throughout the study

Secondary outcome measures

1. Muscle strength testing (by manual muscle testing and myometry)
2. Muscle mass measures (by DEXA scans)
3. IBM functional rating scale
4. Pre- and post-treatment concentrations of HSP70
5. Pathological changes in muscle fibres

Overall study start date

01/10/2010

Completion date

01/10/2012

Eligibility

Key inclusion criteria

1. Meet the diagnostic criteria for definite or probable inclusion body myositis (IBM) (Griggs 1995)

2. Muscle function adequate for quantitative muscle testing. At least 8 of the following 16 muscle groups have a Manual Muscle Test (MMT) Grade greater than 3, or greater on the modified Medical Research Council Scale
3. Aged greater than 50 years, either sex
4. Women must be post-menopausal (no menses in greater than 12 months) or status post-hysterectomy

Participant type(s)

Patient

Age group

Adult

Sex

Both

Target number of participants

Planned sample size: 24; UK sample size: 12

Key exclusion criteria

1. Presence of any one of the following medical conditions:
 - 1.1. Diabetes mellitus or patients taking anti-diabetic medications
 - 1.2. Chronic infection
 - 1.3. Chronic renal insufficiency
 - 1.4. Cancer other than skin cancer less than 5 years previously
 - 1.5. Multiple sclerosis or prior episode of central nervous system demyelination
 - 1.6. Other chronic serious medical illnesses
2. Presence of any of the following on routine blood screening:
 - 2.1. White blood cell count (WBC) less than 300/cm³
 - 2.2. Platelets less than 100,000/cm³
 - 2.3. Haematocrit less than 30%
 - 2.4. Urea greater than 10 mmol/l
 - 2.5. Creatinine greater than 150 µmol/l
 - 2.6. Symptomatic liver disease with serum albumin less than 30 g/l
 - 2.7. Prothrombin time or activated partial thromboplastin greater than upper range of control values
3. Currently taking riluzole
4. Women who are pregnant or lactating
5. History of non-compliance with other therapies
6. Coexistence of other neuromuscular disease
7. Drug or alcohol abuse within last 3 months
8. Inability to give informed consent
9. Known bleeding disorder (e.g. haemophilia, Von Willebrand's Disease)
10. Use of potentially nephrotoxic drugs
11. Prior difficulties with local anaesthetic

Date of first enrolment

01/10/2010

Date of final enrolment

01/10/2012

Locations

Countries of recruitment

England

United Kingdom

United States of America

Study participating centre

National Hospital for Neurology and Neurosurgery

London

United Kingdom

WC1N 3BG

Sponsor information

Organisation

University College London (UCL) (UK)

Sponsor details

UCL Biomedicine Research and Development Unit

Maple House

149 Tottenham Court Road

London

England

United Kingdom

W1T 7NF

Sponsor type

University/education

Website

<http://www.ucl.ac.uk/>

ROR

<https://ror.org/02jx3x895>

Funder(s)

Funder type

Charity

Funder Name

Arthritis Research UK

Alternative Name(s)**Funding Body Type**

Private sector organisation

Funding Body Subtype

Other non-profit organizations

Location

United Kingdom

Results and Publications

Publication and dissemination plan

Not provided at time of registration

Intention to publish date**Individual participant data (IPD) sharing plan****IPD sharing plan summary**

Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	23/03/2016		Yes	No
HRA research summary			28/06/2023	No	No