ISRCTN80057573 https://doi.org/10.1186/ISRCTN80057573

Arimoclomol for inclusion body myositis (IBM)

Submission date
02/02/2011

Recruitment status
No longer recruiting

Registration date
02/02/2011

Overall study status
Completed

Last Edited
04/10/2017

Condition category
Musculoskeletal Diseases

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

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Ms Gisela Barreto

Contact details
Centre for Neuromuscular Diseases
Queen Square
London
United Kingdom
WC1N 3BG
-
Gisela.Barreto@uclh.nhs.uk

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 compond, 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 arimiclomol (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)
Arimiclomol

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