

A double-blind randomised multi-centre, placebo-controlled trial of combined angiotensin converting enzyme-inhibitor and beta-blocker therapy in preventing the development of cardiomyopathy in genetically characterised males with Duchenne Muscular Dystrophy without echo-detectable left ventricular dysfunction

Submission date 12/06/2007	Recruitment status No longer recruiting	<input checked="" type="checkbox"/> Prospectively registered
		<input checked="" type="checkbox"/> Protocol
Registration date 13/08/2007	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
Last Edited 31/12/2020	Condition category Nervous System Diseases	<input type="checkbox"/> Individual participant data

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number

2007-005932-10

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers

1.1

Study information

Scientific Title

A double-blind randomised multi-centre, placebo-controlled trial of combined angiotensin converting enzyme-inhibitor and beta-blocker therapy in preventing the development of cardiomyopathy in genetically characterised males with Duchenne Muscular Dystrophy without echo-detectable left ventricular dysfunction

Acronym

DMD Heart

Study objectives

To determine whether the introduction of Angiotensin Converting Enzyme-inhibitor (ACE-inhibitor) (perindopril) combined with beta-blocker therapy (bisoprolol), before the onset of echo-detectable left ventricular dysfunction, can delay the age of onset and/or slow the rate of progression of cardiomyopathy in males with Duchenne Muscular Dystrophy (DMD).

Ethics approval required

Old ethics approval format

Ethics approval(s)

Ethics pending as of 12/06/2007. No patients will be recruited before ethics approval has been received.

Study design

Double-blind randomised multi-centre placebo-controlled trial

Primary study design

Interventional

Secondary study design

Randomised controlled trial

Study setting(s)

Hospital

Study type(s)

Treatment

Participant information sheet

Health condition(s) or problem(s) studied

Duchenne muscular dystrophy

Interventions

Presentation of Investigational Medicinal Product (IMP):

Each participant will receive:

1. A one-month supply of perindopril 2 mg/bisoprolol 1.25 mg or placebo for the run-in period
2. Six-monthly supplies of perindopril 4 mg/bisoprolol 2.5 mg or placebo for the remainder of the trial

Introduction of IMP or placebo therapies:

The IMP or placebo therapy will be introduced in the following stepwise manner:

Step 1: combined capsule containing perindopril 2 mg/bisoprolol 1.25 mg or matching placebo to be administered by parent(s)/legal guardian(s) at bedtime

Step 2 (one month later): change to maintenance capsule containing perindopril 4 mg/bisoprolol 2.5 mg or matching placebo to be administered by parent(s)/legal guardian(s) at bedtime

Treatment period is for two years. Follow up is for up to 60 months.

Intervention Type

Drug

Phase

Not Applicable

Drug/device/biological/vaccine name(s)

Perindopril, bisoprolol

Primary outcome measure

Change in left ventricular ejection fraction by Simpson's biplane disk method, compared to baseline, after a minimum of two years of combination therapy or placebo. To assess robustness of ejection fraction result, similar comparisons will be made for parameters of left ventricular end-systolic volume and wall motion index.

Secondary outcome measures

1. Death from any cause
2. Development of symptoms and signs of congestive cardiac failure
3. Sufficient objective deterioration in cardiac function, without symptoms to make continued placebo therapy unethical

Secondary outcomes are measured at baseline and 6, 12, 18, 24, 30, 36, 42, 48, 54 and 60 months.

Overall study start date

01/09/2007

Completion date

30/03/2019

Eligibility

Key inclusion criteria

1. Boys aged 7 to 12 years
2. Genetically confirmed DMD with normal left ventricular function on trans-thoracic echocardiography (i.e., left ventricular ejection fraction by Simpson's biplane method greater than 55% [normal mean + SD = 63 + 5%], no global or regional wall motion abnormalities)

Participant type(s)

Patient

Age group

Child

Lower age limit

7 Years

Upper age limit

12 Years

Sex

Male

Target number of participants

140

Key exclusion criteria

1. Contraindication to ACE-inhibitor or beta-blocker therapy
2. Patients, whose initial echo is of insufficient quality to allow reliable measurements of ejection fraction or wall motion
3. Patients with abnormal echocardiograms at baseline
4. Patients with abnormal renal function (creatinine greater than upper limit of local laboratory range; typically greater than 120 mmol/l) or consistently abnormally high serum potassium level (K greater than upper limit of local laboratory range; typically 5 mmol/l)

Date of first enrolment

01/09/2007

Date of final enrolment

30/03/2018

Locations**Countries of recruitment**

England

United Kingdom

Study participating centre

Freeman Hospital

Newcastle upon Tyne

United Kingdom
NE7 7DN

Sponsor information

Organisation

Newcastle upon Tyne Hospitals NHS Foundation Trust (UK)

Sponsor details

Research and Development Office
4th Floor Leazes Wing
Royal Victoria Infirmary
Newcastle upon Tyne
England
United Kingdom
NE1 4LP

Sponsor type

Hospital/treatment centre

Website

<http://www.newcastle-hospitals.org.uk/>

ROR

<https://ror.org/05p40t847>

Funder(s)

Funder type

Charity

Funder Name

British Heart Foundation (UK)

Alternative Name(s)

the_bhf, The British Heart Foundation, BHF

Funding Body Type

Private sector organisation

Funding Body Subtype

Trusts, charities, foundations (both public and private)

Location

United Kingdom

Results and Publications

Publication and dissemination plan

Not provided at time of registration

Intention to publish date

31/10/2019

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?
Basic results				No	No
Protocol article	protocol	19/12/2018	31/12/2020	Yes	No