

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
Registration date 13/08/2007	Overall study status Completed	<input checked="" type="checkbox"/> Protocol
Last Edited 31/12/2020	Condition category Nervous System 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
Dr John Bourke

Contact details
Freeman Hospital
Newcastle upon Tyne
United Kingdom
NE7 7DN

Additional identifiers

Clinical Trials Information System (CTIS)

2007-005932-10

Protocol serial number

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

Study type(s)

Treatment

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(s)

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.

Key secondary outcome(s)

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.

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

Healthy volunteers allowed

No

Age group

Child

Lower age limit

7 years

Upper age limit

12 years

Sex

Male

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**

United Kingdom

England

Study participating centre**Freeman Hospital**

Newcastle upon Tyne

United Kingdom

NE7 7DN

Sponsor information**Organisation**

Newcastle upon Tyne Hospitals NHS Foundation Trust (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

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