

# 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

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

**Contact name**  
Dr John Bourke

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

## 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?
<a href="#">Basic results</a>				No	No
<a href="#">Protocol article</a>	protocol	19/12/2018	31/12/2020	Yes	No