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Pulmonary Hypertension: Assessment of Cell Therapy

Submission date	Recruitment status	[ ] Prospectively registered
06/06/2006	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
16/06/2006	Completed	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
30/09/2008	Circulatory System	[ ] Record updated in last year

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

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
CT-PAH 001

Study information

Scientific Title

Acronym
The PHACeT trial

Study objectives
The primary objective of this phase I clinical trial is to establish the safety of autologous progenitor cell-based gene delivery of human nitric oxide synthase (heNOS) in patients with severe symptomatic pulmonary arterial hypertension (PAH) refractory to conventional treatment.

Please note that, as of 24/09/2008, the anticipated end date of this trial has been updated from 08/05/2008 to 31/10/2009.

Ethics approval required
Old ethics approval format

Ethics approval(s)
This study was approved by the Research Ethics Board (REB) of St. Michael's Hospital in May 2006 (ref: REB 04-253)

Study design
Phase I, open-label, non-randomised, dose-escalation trial. Doses are assigned sequentially.

Primary study design
Interventional

Secondary study design
Non randomised controlled trial

Study setting(s)
Not specified

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Idiopathic pulmonary arterial hypertension

Interventions
A total of 18 patients will be studied using an open-label, dose-escalating protocol; three patients will be entered into each of the five dosing panels. An additional three patients will be entered into the final dose panel to establish safety at the maximum tolerated dose.

Apheresis is performed to obtain mononuclear cells from the patients blood. These cells will then be engineered with human nitric oxide synthase (heNOS) and returned back to the patient (autologous) via the right ventricular port of a pulmonary arterial line in divided doses over a three-day elective hospitalisation. Follow-up hemodynamic measures are recorded at three months post-cell delivery.

Intervention Type
Drug

Phase
Phase I

Drug/device/biological/vaccine name(s)
Nitric oxide

Primary outcome measure
1. Tolerability and safety of the injection of genetically engineered progenitor cells in patients with severe PAH
2. Clinically significant changes in hemodynamic parameters
3. Time to clinical worsening
4. Contrast echo assessment of pulmonary arterial-venous shunting
5. Pulmonary function with diffusing capacity of the lung for carbon monoxide (DLCO)
6. Changes in ventilation perfusion scan
7. Dypnea by Borg index
8. Immune surveillance
9. Human nitric oxide synthase (heNOS) plasmid detection in systemic arterial blood pre- and post-cell delivery

Secondary outcome measures
Potential efficacy of this approach will be assessed by changes in hemodynamic pressures, patient perceived quality of life and exercise capacity.

Overall study start date
08/05/2006

Completion date
31/10/2009

Eligibility

Key inclusion criteria
1. Age >=18 years and <=80 years
2. Clinical diagnosis of idiopathic PAH
3. Familial PAH or anorexigen-induced PAH
4. Specified 6-minute walk distance

Participant type(s)
Patient

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
18

Key exclusion criteria
1. Intra or extra cardiac communication between the right- and left-sided circulations
2. Hemodynamic instability
3. Left ventricular ejection fraction <=40%
4. Thromboembolic event or recent hospitalisation for worsening right-sided heart failure in last three months
5. Central venous pressure (CVP) >20 mmHg at time of research heart catheterisation
6. Pregnancy
7. Concurrent hepatitis or HIV

Date of first enrolment
08/05/2006

Date of final enrolment
31/10/2009

Locations

Countries of recruitment
Canada

Study participating centre
30 Bond Street
Toronto
Canada
M5B 1W8

Sponsor information

Organisation
Northern Therapeutics Inc (Canada)

Sponsor details

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Sponsor type
Industry

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http://www.northernther.com

ROR
https://ror.org/02pv1pj08

Funder(s)

Funder type
Industry

Funder Name
Northern Therapeutics Inc (Canada)

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