

Pulmonary Hypertension: Assessment of Cell Therapy

Submission date 06/06/2006	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
Registration date 16/06/2006	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
Last Edited 30/09/2008	Condition category Circulatory System	<input type="checkbox"/> Individual participant data <input type="checkbox"/> 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. Dyspnea 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 $\leq 40\%$
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

Website

<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