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Carvedilol Treatment in Patients with Transfusion-Dependent Thalassemia

Submission date
18/07/2018

Recruitment status
No longer recruiting

Registration date
24/07/2018

Overall study status
Completed

Last Edited
08/11/2019

Condition category
Circulatory System

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[ ] Results
[ ] Individual participant data
[ ] Record updated in last year

Plain English summary of protocol

Background and study aims
Thalassemia is a blood disorder that causes the body to make an abnormal form of hemoglobin, a protein that carries oxygen in the blood. Severe thalassemia can lead to hemolysis (bursting of red blood cells) and ineffective production of red blood cells. This can lead to symptoms such as anemia, jaundice, swelling of the liver and spleen, changes to the skeleton and delayed growth. For patients with such symptoms, the main treatment is regular blood transfusion and removal of excess iron from the body (iron chelation therapy).
Transfusion-dependent thalassemia is a form of thalassemia that relies on this treatment. Iron chelation therapy is also essential for patients with this, as the most common cause of death in patients with transfusion-dependent thalassemia is iron overload cardiomyopathy, where iron deposits in the heart and leads to heart dysfunction (ventricular dysfunction). Therefore, iron chelation therapy is required to remove this iron from the heart.
Carvedilol is a drug used to treat ventricular dysfunction after a heart attack. The aim of this study was to determine whether carvedilol is an effective treatment for transfusion-dependent thalassemia patients with heart dysfunction.

Who can participate?
Patients with transfusion-dependent thalassemia who have heart dysfunction

What does the study involve?
All patients will be given carvedilol to take daily for 6 months, and will have their heart function assessed before taking the drug and 3 and 6 months after.

What are the possible benefits and risks of participating?
The possible benefit to participants is that taking carvedilol may improve their heart function. The possible risks of participating are side effects of the drug, such as bradycardia and hypotension.

Where is the study run from?
Department of Pediatrics, Faculty of Medicine, Chiang Mai, Thailand

When does the study start and how long is it expected to run for?
October 2011 to December 2017

Who is funding the study?
Thai Research Fund (Thailand)

Who is the main contact?
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Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
CTDT-2016

Study information

Scientific Title
Carvedilol improves left ventricular diastolic dysfunction in patients with Transfusion-Dependent Thalassemia

Acronym
CTDT

Study objectives
Carvedilol can attenuate LV diastolic dysfunction due to iron overload in patients with transfusion-dependent thalassemia who had ventricular dysfunction.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Research Ethics Committee 2, Faculty of Medicine, Chiang Mai University, 23/01/2012, PED-11-863-FB

Study design
Interventional prospective single-centre single-arm non-randomised cohort study

Primary study design
Interventional

Secondary study design
Non randomised study

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
No participant information sheet available

Health condition(s) or problem(s) studied
Transfusion-dependent thalassemia with ventricular diastolic dysfunction

Interventions
All patients were initially given carvedilol in doses of 0.1 mg/kg/day, the medication being divided into 2 separate doses. The dosage was doubled every two weeks until the target dose of 0.8 mg/kg/day was reached and continued for 6 months. The maximum dose was 50 mg/day. All patients had their cardiac function assessed by echocardiography at 3 and 6 months and the level of iron in the heart was also evaluated at 3 and 6 months using cardiac magnetic resonance imaging (MRI). Side effects of the drug were monitored though the study.

Intervention Type
Drug

Phase
Not Specified

Drug/device/biological/vaccine name(s)
Carvedilol

Primary outcome measure
Left ventricular diastolic function, measured by echocardiography at the baseline and 3 and 6 months after treatment. Echocardiographic data included pulse wave Doppler assessment of

mitral and pulmonary venous flows (ventricular diastolic filling analysis) and tissue Doppler imaging. Diastolic left ventricular dysfunction was graded according to pulse wave Doppler of mitral and pulmonary venous flows and tissue Doppler imaging.

Secondary outcome measures
Side effects of carvedilol use, assessed at the baseline and 3 and 6 months after treatment:
1. Blood pressure
2. Heart rate
3. Symptoms such as dizziness, chest pain and swelling

Overall study start date
01/10/2011

Completion date
01/12/2017

Eligibility

Key inclusion criteria
1. Severe forms of homozygous beta-thalassemia and beta-thalassemia/hemoglobin E disease
2. Transfusion-dependent thalassemia
3. Aged 8-25 years
4. Left ventricular diastolic dysfunction

Participant type(s)
Patient

Age group
Other

Sex
Both

Target number of participants
15

Key exclusion criteria
N/A

Date of first enrolment
01/01/2014

Date of final enrolment
31/12/2016

Locations

Countries of recruitment
Thailand

Study participating centre
Chiang Mai University
110 Tambon. Sripoom
Inthawaroroj Road
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Sponsor information

Organisation
Thailand Research Fund

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Sponsor type
Government

Website
https://www.trf.or.th

Funder(s)

Funder type
Not defined

Funder Name
Thailand Research Fund

Results and Publications

Publication and dissemination plan
Planned publication in a high-impact peer reviewed journal.

Intention to publish date
31/08/2018

Individual participant data (IPD) sharing plan
Only the final conclusion of analysis of the results of the study will be available upon request from asilvilairat@gmail.com.

IPD sharing plan summary
Available on request