

# Carvedilol Treatment in Patients with Transfusion-Dependent Thalassemia

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<b>Registration date</b> 24/07/2018	<b>Overall study status</b> Completed	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
<b>Last Edited</b> 08/11/2019	<b>Condition category</b> Circulatory System	<input type="checkbox"/> Individual participant data <input type="checkbox"/> 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?  
Dr Suchaya Silvilairat  
asilvilairat@gmail.com

## Contact information

**Type(s)**  
Scientific

**Contact name**  
Dr Suchaya Silvilairat

**ORCID ID**  
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**Contact details**  
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Chiang Mai  
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50200

## Additional identifiers

**Protocol serial number**  
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)**

## **Study design**

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

## **Primary study design**

Interventional

## **Study type(s)**

Treatment

## **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(s)**

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.

## **Key secondary outcome(s)**

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

## **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

**Healthy volunteers allowed**

No

**Age group**

Other

**Sex**

All

**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

Inthawaroraj Road

A.Muang

Chiang Mai

Thailand

50200

**Sponsor information****Organisation**

Thailand Research Fund

## **Funder(s)**

### **Funder type**

Not defined

### **Funder Name**

Thailand Research Fund

## **Results and Publications**

### **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