

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

10 Jun 2026

### Effectiveness of Thalidomide in Transfusion Dependent Thalassemia patients

#### Protocol summary

##### Study aim

To determine the efficacy of thalidomide in the treatment of transfusion dependent thalassemia.

##### Design

Randomized Controlled Trial, single group study of 55 patients with non blinded outcome assessment. Non probability convenience sampling.

##### Settings and conduct

This study will be conducted at Department of Pediatrics, Sheikh Zayed Medical College/Hospital, Rahim Yar Khan. This study will be conducted after approval from Ethical Review Board (ERB) of Sheikh Zayed Hospital and University of Health Sciences, Lahore.

##### Participants/Inclusion and exclusion criteria

Inclusion criteria: thalassemia patients aged 2- 15 years  
Exclusion criteria: Patient with liver, renal, cardiac, pulmonary, neurological deficits or thrombotic episodes.

##### Intervention groups

Patients diagnosed with beta thalassemia diagnosed according to clinical characteristics and hemoglobin electrophoresis were included in this study according to the selection criteria. At the time of treatment the patients will be interviewed and tested for pre-treatment variables which include hemoglobin levels, transfusion rate. All the patients will then be given 2-5mg/kg thalidomide for 6 months as prescribed by the consultant and these variables will be tested again. Response rate will be tested on the basis of operational definition. The principal investigator will follow up with all the patients and see post-treatment results.

##### Main outcome variables

This study will help us determine if we can use thalidomide in the treatment of beta thalassemia patients for reduction of transfusion, increase Hemoglobin level and response rate.

#### General information

##### Reason for update

##### Acronym

##### IRCT registration information

IRCT registration number: **IRCT20220215054023N1**  
Registration date: **2022-04-27, 1401/02/07**  
Registration timing: **prospective**

Last update: **2022-04-27, 1401/02/07**

Update count: **0**

##### Registration date

2022-04-27, 1401/02/07

##### Registrant information

##### Name

Muhammad Saleem

##### Name of organization / entity

Sheikh Zayed Medical college/Hospital, Rahim Yar Khan, Punjab, Pakistan

##### Country

Pakistan

##### Phone

+92 68 9230109

##### Email address

profdrmsaleem@szmc.edu.pk

##### Recruitment status

**Recruitment complete**

##### Funding source

##### Expected recruitment start date

2022-06-01, 1401/03/11

##### Expected recruitment end date

2022-09-01, 1401/06/10

##### Actual recruitment start date

empty

##### Actual recruitment end date

empty

##### Trial completion date

empty

##### Scientific title

Effectiveness of Thalidomide in Transfusion Dependent

Thalassemia patients

## Public title

Effectiveness of Thalidomide in Transfusion Dependent Thalassemia patients

## Purpose

Health service research

## Inclusion/Exclusion criteria

### Inclusion criteria:

Beta thalassemia patients are diagnosed by clinical characteristics and by hemoglobin electrophoresis Age 2 years to 15 years Both Gender

### Exclusion criteria:

Patients already undergoing treatment for induction of HbF Patients with liver, renal, cardiac, pulmonary, neurological deficits or history of thrombotic episodes Patients not giving consent to treatment

## Age

From **2 years** old to **15 years** old

## Gender

Both

## Phase

N/A

## Groups that have been masked

*No information*

## Sample size

Target sample size: **55**

## Randomization (investigator's opinion)

Not randomized

## Randomization description

## Blinding (investigator's opinion)

Not blinded

## Blinding description

## Placebo

Not used

## Assignment

Parallel

## Other design features

## Secondary Ids

empty

## Ethics committees

### 1

#### Ethics committee

##### Name of ethics committee

Institutional Review Board Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

##### Street address

Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

##### City

Rahim Yar Khan

##### Postal code

64200

#### Approval date

2022-03-04, 1400/12/13

#### Ethics committee reference number

441-/IRB/SZMC/SZH

## Health conditions studied

### 1

#### Description of health condition studied

Beta thalassemia is genetic disorder which is caused by insertions and deletions in chromosome number 11. The disease is treated by blood transfusions which leads to iron over load and demand for chelation therapy. Some patients do not respond to this treatment and suffer from complications. Although treatment with hydroxyurea has leads to improved conditions by reducing transfusion demand, but some patients do not respond to this treatment. Hence, other treatment modalities which induce fetal hemoglobin are being explored. The aim of this study is to see the effectiveness of thalidomide in treating beta thalassemia patients

#### ICD-10 code

D56.1

#### ICD-10 code description

Beta thalassemia

## Primary outcomes

### 1

#### Description

Response to treatment

#### Timepoint

before intervention and after 6 months after intervention

#### Method of measurement

become transfusion free after treatment

## Secondary outcomes

### 1

#### Description

number of transfusion and hemoglobin level

#### Timepoint

before intervention and after 6 months of intervention

#### Method of measurement

number of transfusion and hemoglobin level

## Intervention groups

### 1

#### Description

Intervention group: Patients diagnosed with beta thalassemia diagnosed according to clinical characteristics and hemoglobin electrophoresis were included in this study according to the selection criteria. At the time of treatment the patients will interviewed and tested for pre-treatment variables which include hemoglobin levels, transfusion rate. All the patients will then be given 2-5mg/kg thalidomide for 6 months as prescribed by the consultant and these variables will be tested again. Response rate will be tested on the basis of operational definition. The principal investigator will follow up with all the patients and see post-treatment

results. Drug used in the study is Capsule Thalido(Thalidomide) with strength 50mg and 100mg will ne given as 2-5mg/kg in once daily dose for 6 months manufactured by ATCO laboratories

**Category**

Treatment - Drugs

**2****Description**

Control group: This group will not receive any type of treatment as assigned to interventional group. Effectiveness will be measured in interventional group only

**Category**

N/A

**Recruitment centers****1****Recruitment center****Name of recruitment center**

Department of Pediatrics (unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**Full name of responsible person**

Prof. Dr. Muhammad Saleem Laghari

**Street address**

Department of Pediatrics (unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**City**

Rahim Yar Khan

**Postal code**

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**Phone**

+92 333 8808633

**Email**

profdrmsaleem@szmc.edu.pk

**Sponsors / Funding sources****1****Sponsor****Name of organization / entity**

Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**Full name of responsible person**

Prof. Dr. Muhammad Saleem Laghari

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**Grant name****Grant code / Reference number****Is the source of funding the same sponsor organization/entity?**

Yes

**Title of funding source**

Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**Proportion provided by this source**

100

**Public or private sector**

Public

**Domestic or foreign origin**

Domestic

**Category of foreign source of funding**

*empty*

**Country of origin****Type of organization providing the funding**

Academic

**Person responsible for general inquiries****Contact****Name of organization / entity**

Department of Pediatrics (unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**Full name of responsible person**

Prof. Dr. Muhammad Saleem Laghari

**Position**

Professor

**Latest degree**

Specialist

**Other areas of specialty/work**

Pediatrics

**Street address**

Department of Pediatrics(unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

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**Person responsible for scientific inquiries****Contact****Name of organization / entity**

Department of Pediatrics (unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

**Full name of responsible person**

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## Person responsible for updating data

**Contact**

**Name of organization / entity**

Department of Pediatrics (unit 2), Sheikh Zayed Medical College/Hospital, Rahim Yar Khan

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**Email**

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## Sharing plan

**Deidentified Individual Participant Data Set (IPD)**

Yes - There is a plan to make this available

**Study Protocol**

Yes - There is a plan to make this available

**Statistical Analysis Plan**

Yes - There is a plan to make this available

**Informed Consent Form**

Yes - There is a plan to make this available

**Clinical Study Report**

Yes - There is a plan to make this available

**Analytic Code**

Yes - There is a plan to make this available

**Data Dictionary**

Yes - There is a plan to make this available

**Title and more details about the data/document**

Effectiveness of Thalidomide in Transfusion Dependent Thalassemia Patients This study will be conducted at Department of Pediatrics, Sheikh Zayed Medical College/Hospital, Rahim Yar Khan. This study will be conducted after approval from Ethical Review Board (ERB) of Sheikh Zayed Hospital and University of Health Sciences, Lahore. Performa, attached with the synopsis, will be filled by the principle investigator. Patients diagnosed with beta thalassemia diagnosed according to clinical characteristics and hemoglobin electrophoresis were included in this study according to the selection criteria. At the time of treatment the patients will interviewed and tested for pre-treatment variables which include hemoglobin levels and transfusion rate. All the patients will then be given 2-5mg/kg thalidomide for 6 months as prescribed by the consultant hematologist and these variables will be tested again. Response rate will be tested on the basis of operational definition. The principal investigator will follow up with all the patients and see post-treatment results.

**When the data will become available and for how long**

Starting 6 months after publication

**To whom data/document is available**

For people working in academic institutions

**Under which criteria data/document could be used**

This data will de used for research purposes and also improve the treatment of beta thalassemia

**From where data/document is obtainable**

Contact the relevant person via phone number, email addresses or postal

**What processes are involved for a request to access data/document**

Just contact the relevant person via phone number, email addresses or postal to request to access data

**Comments**