

Clinical Trial Protocol

Iranian Registry of Clinical Trials

10 Jul 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 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

64200

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

Street address

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

City

Rahim Yar Khan

Postal code

64200

Phone

+92 333 8808633

Email

profdrmsaleem@szmc.edu.pk

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

City

Rahim Yar Khan

Province

Punjab

Postal code

64200

Phone

+92 333 8808633

Email

profdrmsaleem@szmc.edu.pk

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

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

City

Rahim Yar Khan

Province

Punjab

Postal code

64200

Phone

+92 333 8808633

Email

profdrmsaleem@szmc.edu.pk

Person responsible for updating data

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. 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

City

Rahim Yar Khan

Province

Punjab

Postal code

64200

Phone

+92 333 8808633

Email

profdrmsaleem@szmc.edu.pk

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