Clinical Trial Protocol
Iranian Registry of Clinical Trials

22 Nov 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

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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

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Person responsible for scientific inquiries

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Person responsible for updating data

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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