Clinical Trial Protocol
Iranian Registry of Clinical Trials

22 Nov 2026

The Effect of Everolimus on Renal, Brain, Skin Lesions and Seizure of Tuberous Sclerosis Complex

Protocol summary

Study aim

The evaluation and comparison of the brain, renal, skin lesions, and seizure severity in the TSC patients before and after 6 months of everolimus treatment.

Design

20 eligible children, age under 15 years with TSC will include in this study after obtaining consent. A code was allocated to each one of them.

Settings and conduct

TSC patients referring to the Neurology Clinic of Tabriz Children Hospital after including in the study, brain, renal, skin lesions, and seizure severity will be evaluated in the patients by standard methods. Based on the references, 3 mg/m2/day of everolimus will be administered to the patients for 6 months. The patients will be evaluated for any probable side effect of the drug during the administration period. After six months of the treatment, the severity of the lesions will be evaluated again by the standard methods and will be statistically compared with the basal severity of the lesions.

Participants/Inclusion and exclusion criteria

20 Children with TSC, age under 15 years that referred to Neurology Clinic of Tabriz Children Hospital will purposefully be chosen and included in the study after the diagnisis and confirmation of the TSC disease by a medical specialist and getting informed consent. The patients with underlying diseases and or with drug prohibition will be excluded from the study.

Intervention groups

Based on the references, 3 mg/m2/day of everolimus will be administered to the patients for 6 months. The patients will be evaluated for any probable side effect of the drug during the administration period.

Main outcome variables

The results of this study can be considered as an important step in controlling and treating TSC. If the positive therapeutic effect of everolimus on TSC-associated lesions is proven; this drug could also be used in the treatment process of the TSC.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20131012014988N2**
Registration date: **2017-12-05, 1396/09/14**
Registration timing: **registered_while_recruiting**

Last update: **2017-12-05, 1396/09/14**
Update count: **0**

Registration date

2017-12-05, 1396/09/14

Registrant information

Name

Mohammad Barzegar

Name of organization / entity

Pediatric Health Research Center,Tabriz University of Medical Sciences

Country

Iran (Islamic Republic of)

Phone

+98 41 3526 2265

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phrc_mb@tbzmed.ac.ir

Recruitment status

Recruitment complete

Funding source

Pediatric Health Research Center, Tabriz University of Medical Sciences.

Expected recruitment start date

2017-07-04, 1396/04/13

Expected recruitment end date

2018-01-03, 1396/10/13

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date
empty

Scientific title
The Effect of Everolimus on Renal, Brain, Skin Lesions and Seizure of Tuberous Sclerosis Complex

Public title
The Effect of Everolimus in the Treatment of Tuberous Sclerosis Complex

Purpose
Treatment

Inclusion/Exclusion criteria
Inclusion criteria:
20 children, age under 15 years, with TSC proven by an expert physician through clinical and paraclinical criteria will include in this study Parental consent of the patients
Exclusion criteria:
Having any underlying disease Drug prohibition

Age
To **15 years** old

Gender
Both

Phase
N/A

Groups that have been masked
No information

Sample size
Target sample size: **20**

Randomization (investigator's opinion)
N/A

Randomization description

Blinding (investigator's opinion)
Not blinded

Blinding description

Placebo
Not used

Assignment
Single

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee
Name of ethics committee
Regional Ethics Committee, Office of the Vice Chancellor for Research, Tabriz University of Medical
Street address
International Relations Office, No 2 Central Building, Tabriz University of Medical Sciences, University Street, Tabriz, PO Box: 5165665931, Iran
City
Tabriz
Province
East Azarbaijan
Postal code
5165665931
Approval date
2017-07-03, 1396/04/12
Ethics committee reference number
IR.TBZMED.REC.1396.287

Health conditions studied

1

Description of health condition studied
Tuberous sclerosis
ICD-10 code
Q85.1
ICD-10 code description
Other congenital malformations

Primary outcomes

1

Description
Renal lesions severity
Timepoint
before treatment-six months after the treatment
Method of measurement
MRI, Medical ultrasound (sonography), questionnaire

2

Description
Brain lesions severity
Timepoint
before treatment-six months after the treatment
Method of measurement
MRI, Medical ultrasound (sonography), questionnaire

3

Description
Skin lesions severity
Timepoint
before treatment-six months after the treatment
Method of measurement
Medical examination, questionnaire

4

Description
Seizure
Timepoint
before treatment-six months after the treatment
Method of measurement
Questionnaire

Secondary outcomes

1

Description
constipation
Timepoint

During the six months of the administration period

Method of measurement

Clinical Examination, Questionnaire

2

Description

nausea

Timepoint

During the six months of the administration period

Method of measurement

Clinical Examination, Questionnaire

3

Description

diarrhea

Timepoint

During the six months of the administration period

Method of measurement

Clinical Examination, Questionnaire

4

Description

vomiting

Timepoint

During the six months of the administration period

Method of measurement

Clinical Examination, Questionnaire

5

Description

Body weight

Timepoint

During the six months of the administration period

Method of measurement

Scale, Questionnaire

Intervention groups

1

Description

In the case (intervention) group, based on the scientific references, 3 mg/m2/day "Everolimus" (C53H83NO14), in 5 mg tablet form (Novartis Co, Swiss), will administer for 6 months.

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Tabriz Children Hospital

Full name of responsible person

Dr Mohammad Barzegar

Street address

Tabriz Children Hospital, Sheshgelan Street, Tabriz, Iran.

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Pediatric Health Rsearch Center, Tabriz University of Medical Sciences

Full name of responsible person

Dr. Mohammad Barzegar

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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

Pediatric Health Rsearch Center, Tabriz University of Medical Sciences

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
Pediatric Health Research Center, Tabriz University of Medical Sciences

Full name of responsible person
Dr. Mohammad Barzegar

Position
Pediatric neurologist, Professor, Faculty Member

Latest degree
Subspecialist

Other areas of specialty/work
Neuroscience

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Pediatric Health Research Center, Children Hospital, Sheshgelan street, Tabriz, Iran.

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Person responsible for scientific inquiries

Contact

Name of organization / entity
Pediatric Health Research Center, Tabriz University of Medical Sciences

Full name of responsible person
Dr. Moahhamad Barzegar

Position
Pediatric Neurologist

Latest degree
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Other areas of specialty/work
Neuroscience

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Person responsible for updating data

Contact

Name of organization / entity
Tabriz Children Hospital

Full name of responsible person
Dr Hassan Bakhtiari

Position
Pediatric neurology fellowship

Latest degree
Subspecialist

Other areas of specialty/work
Neuroscience

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Sharing plan

Deidentified Individual Participant Data Set (IPD)
Undecided - It is not yet known if there will be a plan to make this available

Study Protocol
Undecided - It is not yet known if there will be a plan to make this available

Statistical Analysis Plan
Undecided - It is not yet known if there will be a plan to make this available

Informed Consent Form
Undecided - It is not yet known if there will be a plan to make this available

Clinical Study Report
Undecided - It is not yet known if there will be a plan to make this available

Analytic Code
Undecided - It is not yet known if there will be a plan to make this available

Data Dictionary
Undecided - It is not yet known if there will be a plan to make this available