

Clinical Trial Protocol

Iranian Registry of Clinical Trials

27 Jul 2026

Efficacy of Everolimus in pediatric patients with tuberous sclerosis and subependymal giant cell astrocytoma

Protocol summary

Study aim

specify the effect of Everolimus on SEGA volume change before and after treatment and to determine the effect of this drug on renal angiomyolipoma (AML), skin lesions, and seizures in TSC patients.

Design

Sample size was calculated based on the two-proportion comparison (before-after) [McNemar's test], considering a proportion of 21.5% response to treatment

Settings and conduct

All patients with the diagnosis of tuberculosis and all the above criteria who had referred to Imam Reza (AS) pediatric oncology clinic were included in the study, and the examination and questionnaire were completed at specific time intervals.

Participants/Inclusion and exclusion criteria

Inclusion criteria: Patients who definitely diagnosed by tuberous sclerosis complex and medically stable.
Exclusion criteria: To require any surgery for subependymal giant cell astrocytomas, critical hydrocephalus or imminent cerebral herniation patients with severe hepatic impairment

Intervention groups

Everolimus will be administered orally at a starting dose of 1.0 mg/m² body surface area per day and adjusted to attain blood trough concentrations of 5-10 ng/mL considering toxic side effects.

Main outcome variables

Primary endpoint will be considered as the confirmed tumor response, defined as a reduction in the total volume of all target subependymal giant cell astrocytomas of 50% or more relative to baseline, in the absence of worsening of non-target subependymal giant cell astrocytomas.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20240112060688N1**

Registration date: **2024-12-23, 1403/10/03**

Registration timing: **registered_while_recruiting**

Last update: **2024-12-23, 1403/10/03**

Update count: **0**

Registration date

2024-12-23, 1403/10/03

Registrant information

Name

Hadi Mottaghipisheh

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 71 3830 3110

Email address

mottaghipisheh@sums.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2024-12-21, 1403/10/01

Expected recruitment end date

2025-12-22, 1404/10/01

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Efficacy of Everolimus in pediatric patients with tuberous sclerosis and subependymal giant cell astrocytoma

Public title

Everolimus in sub ependymal giant cell astrocytoma

Purpose
Treatment

Inclusion/Exclusion criteria
Inclusion criteria:
Patients who definitely diagnosed by tuberous sclerosis complex and medically stable children 1 to 18 years old
Exclusion criteria:
To require any surgery for subependymal giant cell astrocytomas, critical hydrocephalus or imminent cerebral herniation

Age
From **1 year** old to **18 years** old

Gender
Both

Phase
4

Groups that have been masked
No information

Sample size
Target sample size: **29**

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
research Ethics committees of school of medicine - shiraz university of medical sciences
Street address
farhangshahr station 11 Amir oncology hospital
City
shiraz
Province
Fars
Postal code
۷۱۸۷۹۱۵۹۹۸

Approval date
2024-09-28, 1403/07/07

Ethics committee reference number
IR.SUMS.MED.REC.1403.433

Health conditions studied

1

Description of health condition studied
tuberous sclerosis

ICD-10 code
Q85.1

ICD-10 code description
Tuberous sclerosis

Primary outcomes

1

Description
decreased size of sub ependymal giant cell astrocytoma

Timepoint
at the beginning and every 3 months till one year

Method of measurement
MRI findings

2

Description
Absolute change from baseline to ۲۴ weeks in seizure frequency per ۲۴ hours

Timepoint
at the beginning and every 3 months till one year

Method of measurement
Electroencephalography

3

Description
skin lesion response rate in patients with at least one skin lesion at baseline;

Timepoint
at the beginning and every 3 months till one year

Method of measurement
physical examination

4

Description
angiomyolipoma response rate

Timepoint
at the beginning and every 3 months till one year

Method of measurement
ultrasound sonography

Secondary outcomes

empty

Intervention groups

1

Description
Intervention group: eligible children (aged under ۱۸ years old) with a definite diagnosis of tuberous sclerosis complex will be included by convenience sampling method based on the inclusion exclusion criteria: Everolimus will be administered orally at a starting dose

of ۴.۵ mg/m^۲ body surface area per day for ۱۲ months duration and adjusted to attain blood trough concentrations of ۵-۱۵ ng/mL considering toxic side effects. All patients were monitored for adverse effects during everolimus treatment, like stomatitis, aphthous ulcer, flushing, hypertension, peripheral edema, hyperglycemia, abdominal pain and others included in questionnaire. In some patients because of unavailability of test for everolimus level we decreased ۲۰% of prescription dose after severe aphthous lesions or severe toxicity occurred. if patients do not tolerate everolimus, drug stopped and excluded from the study. Our patients followed for minimum one year after start of everolimus. Brain MRI will be done every ۳ months after initiation of the treatment and abdominal and pelvic sonography for angiomylipoma lesions in and reported in single specialized center and reported by a single expert radiologist. All patients will be completed an EEG at baseline and ۲۴ weeks (or end of treatment for those who discontinued). Skin lesions will be assessed every ۳ months by physician in clinic according to size, number, color and location. Blood will be drawn every visit starting at week ۲ for everolimus drug level, including hematology and blood chemistry, will be done every ۲ weeks for the first ۴ weeks, then monthly.

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Shiraz- Pediatric Oncology Imam Reza clinic

Full name of responsible person

Hadi Mottaghipisheh

Street address

Namazi square

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Shiraz University of Medical Sciences

Full name of responsible person

Mohammad Hashem Hashempoor

Street address

Zand St., Central Building of Shiraz University of Medical Sciences, 7th floor, Research and Technology

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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

Shiraz 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

Shiraz University of Medical Sciences

Full name of responsible person

Hadi Mottaghipisheh

Position

Assistant Professor

Latest degree

Subspecialist

Other areas of specialty/work

Oncology

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Person responsible for scientific inquiries

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Position

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Latest degree

Subspecialist

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Person responsible for updating data**Contact****Name of organization / entity**

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Latest degree

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Other areas of specialty/work

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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

all collected deidentified IPD

When the data will become available and for how long

starting after publication

To whom data/document is available

people working in academic institutions

Under which criteria data/document could be used

no any other criteria

From where data/document is obtainable

email

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

email

Comments