Clinical Trial Protocol
Iranian Registry of Clinical Trials

10 Jun 2026

Efficacy of Everolimus in pediatric patients with tuberous sclerosis and sub ependymal 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 ۲۸.۵% 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 ۴.۵ mg/m۲ body surface area per day and adjusted to attain blood trough concentrations of ۵-۱۵ 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 ۵۰% 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)

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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 sub ependymal 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/m۲ 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 angiomyolipima 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

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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

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

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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

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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