

Clinical Trial Protocol

Iranian Registry of Clinical Trials

16 Sep 2026

Investigating the effectiveness and side effects of Spinaraza and Risidiplam on the motor performance of patients with spinal muscular atrophy: a non-controlled trial

Protocol summary

Study aim

Investigating the effectiveness and side effects of Spinaraza and Risidiplam on the motor performance of patients with spinal muscular atrophy

Design

An interventional Study to investigate the effectiveness and side effects of 2 FDA-approved drugs for SMA patients, without a control group, non-randomized, no blinding

Settings and conduct

Suspected cases of SMA are called from Hamadan and Kermanshah, and the confirmed cases with informed consent are entered

Participants/Inclusion and exclusion criteria

Patients with a final diagnosis of SMA by a pediatric neurologist chosen for the research project in Hamadan and Kermanshah provinces, Patients who themselves or their legal guardians have informed consent to inject drugs and participate in the study

Intervention groups

Patients who receive Spinraza Patients who receive Risidiplam

Main outcome variables

The score of motor function tests (Hammersmith and HINE)

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20120821010634N4**

Registration date: **2023-05-27, 1402/03/06**

Registration timing: **registered_while_recruiting**

Last update: **2023-05-27, 1402/03/06**

Update count: **0**

Registration date

2023-05-27, 1402/03/06

Registrant information

Name

Afshin Fayazi

Name of organization / entity

Hamedan University of Medical Sciences

Country

Iran (Islamic Republic of)

Phone

+98 81 1264 0075

Email address

afshin_fay@yahoo.com

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2023-04-21, 1402/02/01

Expected recruitment end date

2024-09-19, 1403/06/29

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Investigating the effectiveness and side effects of Spinaraza and Risidiplam on the motor performance of patients with spinal muscular atrophy: a non-controlled trial

Public title

effectiveness and side effects of Spinaraza and Risidiplam on the motor performance of patients with spinal muscular atrophy

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

SMA clinical diagnosis by pediatric neurologist SMA diagnosis according to genetic test

Exclusion criteria:

The patient do not have a consent

Age

No age limit

Gender

Both

Phase

N/A

Groups that have been masked

No information

Sample size

Target sample size: 30

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

Ethics committee of Hamadan University of Medical Sciences

Street address

Deputy of research, Hamadan University of Medical Sciences, Shahid Fahmideh Street

City

Hamadan

Province

Hamadan

Postal code

6517838678

Approval date

2023-03-04, 1401/12/13

Ethics committee reference number

IR.UMSHA.REC.1401.1046

Health conditions studied

1

Description of health condition studied

Spinal Muscular Atrophy

ICD-10 code

G12

ICD-10 code description

Spinal muscular atrophy and related syndromes

Primary outcomes

1

Description

Assessment of Motor Function

Timepoint

one month, 3 months, and 6 months after drug initiation

Method of measurement

Assessment of motor function based on Hammersmith and HINE scales

2

Description

Clinical and laboratory side effects of drugs

Timepoint

one month, 3 months, and 6 month after drug initiation

Method of measurement

fulfilling check list according to patient reports, requesting CBC, liver and renal function tests

Secondary outcomes

empty

Intervention groups

1

Description

Intervention group: patients that receive Spinraza. intrathecal injection of 5 ml (12mg) of the drug after drainage of 5 ml from CSF. Injections are planned for days 0, 14,28, 63, and every 4 months after that. (manufactured by Biogen and Alcyone Therapeutics)

Category

Treatment - Drugs

2

Description

Intervention group: patients that receive Risdiplam, this drug is prescribed as an oral daily dosage according to age and weight. (under 2 months: 0.15 mg/kg, two months to two years: 0.2 mg/kg, more than two years and less than 20 kg: 0.25 mg/kg, more than two years and more than 20 kg: 5 mg)

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center
Besat Hospital (Hamadan)
Full name of responsible person
Afshin Fayazi
Street address
Motahari Blvd
City
Hamadan
Province
Hamadan
Postal code
6514845411
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+98 81 3264 0030
Email
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Full name of responsible person
Afshin Fayazi
Position
Associate professor
Latest degree
Subspecialist
Other areas of specialty/work
Pediatrics
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6514845411
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afshin_fay@yahoo.com

Sponsors / Funding sources

1

Sponsor

Name of organization / entity
Hamedan University of Medical Sciences
Full name of responsible person
Reza Shokouhi
Street address
Shaid Fahmideh street
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6517838678
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+98 81 3131 0000
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info.research@umsha.ac.ir

Grant name

Grant code / Reference number

Is the source of funding the same sponsor organization/entity?

Yes

Title of funding source

Hamedan 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
Hamedan University of Medical Sciences

Person responsible for scientific inquiries

Contact

Name of organization / entity
Hamedan University of Medical Sciences
Full name of responsible person
Afshin Fayazi
Position
Associate professor
Latest degree
Subspecialist
Other areas of specialty/work
Pediatrics
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Person responsible for updating data

Contact

Name of organization / entity
Hamedan University of Medical Sciences
Full name of responsible person
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Sharing plan

Deidentified Individual Participant Data Set (IPD)

No - There is not a plan to make this available

Justification/reason for indecision/not sharing IPD

Respecting the privacy and personal data of participants

Study Protocol

No - There is not a plan to make this available

Statistical Analysis Plan

No - There is not a plan to make this available

Informed Consent Form

No - There is not a plan to make this available

Clinical Study Report

No - There is not a plan to make this available

Analytic Code

No - There is not a plan to make this available

Data Dictionary

No - There is not a plan to make this available