

Clinical Trial Protocol

Iranian Registry of Clinical Trials

10 Jul 2026

Evaluation of the efficacy and safety of Risdiplam and Nusinersen in patients with Spinal Muscular Atrophy (SMA)

Protocol summary

Study aim

1-Comparison of the efficacy of risdiplam and nusinersen in SMA patients 2-Comparison of side effects of risdiplam and nusinersen in SMA patients 3-Comparison of the amount of NCS changes in these two groups

Design

Clinical trial without control group, with parallel groups, not blinded, not randomized, phase 1 on 40 patients.

Settings and conduct

Before starting the medication, full clinical examinations of the patients will be performed and the growth indices, laboratory findings and scoring of the patients' motor abilities based on the criteria of CHOP INTEND, RULM, HFMSE and SMAIS will be used. The mentioned performance will be re-evaluated and recorded during the study period with intervals of 0, 1, 3 and 6 months and if possible 12 months. Also, unwanted side effects of drugs will be investigated. The initial NCV is performed at the time of entering the study and is repeated for patients with SMA type 1 every 3 months during the study period and for type 2 and 3 patients with 6 month intervals. Also, laboratory tests is repeated at the end of the 4th week and the 6th month.

Participants/Inclusion and exclusion criteria

Genetically confirmed SMA T 1, 2 and 3 patients with SMN2 copy number between 2 and 4 and age less than 10 years

Intervention groups

Spinraza Group and Risdiplam Group

Main outcome variables

Assessment of Motor Function, Amplitude changes in NCS and drugs side effects

General information

Reason for update

Acronym

Spinal Muscular Atrophy(SMA), Survival motor neuron2 (SMN2), Central nervous system (CNS), Cerebrospinal

Fluid (CSF), Homozygous Gene Mutation or Deletion (5q SMA), Children's hospital of philadelphia infant test of neuromuscular disorders (CHOP INT

IRCT registration information

IRCT registration number: **IRCT20131012014988N8**

Registration date: **2023-10-22, 1402/07/30**

Registration timing: **prospective**

Last update: **2023-10-22, 1402/07/30**

Update count: **0**

Registration date

2023-10-22, 1402/07/30

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

Email address

phrc_mb@tbzmed.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2023-10-23, 1402/08/01

Expected recruitment end date

2024-10-22, 1403/08/01

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Evaluation of the efficacy and safety of Risdiplam and Nusinersen in patients with Spinal Muscular Atrophy (SMA)

Public title

Evaluation of the efficacy and safety of Risdiplam and Nusinersen in patients with Spinal Muscular Atrophy (SMA)

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Definitive genetic diagnosis of homozygous gene mutation or deletion (5q SMA) Patients with equal or less than 10 years old SMN2 copy number is 2 to 4

Exclusion criteria:

Active infection requiring systemic antibiotic or antiviral treatment up to 2 weeks after recovery Existence of concomitant incurable diseases (history of asphyxia, seizures, severe liver, cardiovascular, and endocrine involvement) Liver failure (due to the possibility of increased drug levels) (Child Pugh class C) receiving metformin (for risdiplam) Lactation and pregnancy History of recent eye diseases (uncontrolled glaucoma) (for Risdiplam) Existence of severe kyphoscoliosis or corrective surgery (for Nusinersen) Having a CNS shunt and CSF catheter (for Nusinersen) History of bacterial meningitis (for nusinersen)

Age

To 10 years old

Gender

Both

Phase

N/A

Groups that have been masked

No information

Sample size

Target sample size: 40

Randomization (investigator's opinion)

N/A

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 Tabriz University of Medical

Sciences

Street address

Golgasht Ave, medicine faculty

City

Tabriz

Province

East Azarbaijan

Postal code

5157610144

Approval date

2023-08-13, 1402/05/22

Ethics committee reference number

IR.TBZMED.REC.1402.402

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, three months, and six months, and if possible, one year after starting the drug

Method of measurement

At the end of the 1st, 3rd, and 6th months from the start of treatment, a pediatric neurologist or a pediatric neurology fellowship will visit and perform CHOP INTENT, HFMSE, RULM, and SMAIS tests for patients.

2

Description

Amplitude changes in NCS

Timepoint

Every three months for SMA-1 and every six months for SMA-2,3

Method of measurement

A physical medicine and rehabilitation specialist (a permanent member of the research group) will evaluate the CMAP amplitude changes of a single nerve.

3

Description

Drug side effects evaluation

Timepoint

Every 2 weeks in the first month and then monthly by asking the patients themselves or their parents

Method of measurement

by asking the patients themselves or their parents

Secondary outcomes

empty

Intervention groups

1

Description

Intervention group: spinraza group

Category

Treatment - Drugs

2

Description

Intervention group: risdiplam group

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

zahra mardani azari children hospital

Full name of responsible person

Dr mohammad baezegar

Street address

Mashhouri st. Mardani Azari St. Khavaran Town

City

tabriz

Province

East Azarbaijan

Postal code

5143377505

Phone

+98 41 3159 2105

Email

mardaniazar@tbzmed.ac.ir

Web page address

<https://mardaniazari.tbzmed.ac.ir/>

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Tabriz University of Medical Sciences

Full name of responsible person

Dr. Parviz Shahabi

Street address

Golgasht Street

City

Tabriz

Province

East Azarbaijan

Postal code

5143377505

Phone

+98 41 3335 7310

Email

dabirkhanecent@tbzmed.ac.ir

Grant name

Grant code / Reference number

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

No

Title of funding source

Board of Trustees of the Ministry of Health

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

Tabriz University of Medical Sciences

Full name of responsible person

Dr. Mohammad Barzegar

Position

professor

Latest degree

Subspecialist

Other areas of specialty/work

Pediatrics

Street address

No 3, maryam street, Golbad street, tabriz

City

tabriz

Province

East Azarbaijan

Postal code

5143377505

Phone

+98 914 117 0492

Fax

+98 41 3159 2105

Email

mm_barzegar@yahoo.com

Person responsible for scientific inquiries

Contact

Name of organization / entity

Tabriz University of Medical Sciences

Full name of responsible person

Dr. Mohammad Barzegar

Position

professor

Latest degree

Subspecialist

Other areas of specialty/work

Pediatrics

Street address

No 3, maryam street, Golbad street, tabriz

City

tabriz

Province

East Azarbaijan

Postal code

5143377505

Phone

+98 914 117 0492

Email

mm_barzegar@yahoo.com

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

Tabriz University of Medical Sciences

Full name of responsible person

Dr. Bita poorshiri

Position

assistant professor

Latest degree

Subspecialist

Other areas of specialty/work

Pediatrics

Street address

aras building, tavanir street, tabriz

City

tabriz

Province

East Azarbaijan

Postal code

5138933171

Phone

+98 914 301 3061

Email

bpskam@yahoo.com

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