

Clinical Trial Protocol

Iranian Registry of Clinical Trials

09 Jun 2026

Investigation of the effects of trehalose on quality of life status and paraclinical findings of patients with lysosomal storage disorders (Niemann-Pick and mucopolysaccharidosis type III (Sanfilippo))

Protocol summary

Study aim

Investigation of the effects of trehalose on quality of life status and paraclinical findings of patients with lysosomal storage disorders (Niemann-Pick and mucopolysaccharidosis type III (Sanfilippo))

Design

Single-arm clinical trial (without control group), phase 2, on 40 patients

Settings and conduct

Patients with Niemann-Pick or mucopolysaccharidosis type III (Sanfilippo) with mentioned inclusion criteria, will be enrolled to this study. The Trehalose (15 gr per week) will be administered intravenous (IV) for 12 weeks. The patients will be assessed before and after the intervention.

Participants/Inclusion and exclusion criteria

Inclusion Criteria: Patients with Niemann-Pick or mucopolysaccharidosis type III (Sanfilippo) that sign informed consent (For children, their parents or legal guardians should sign informed consent).

Intervention groups

Intervention group: IV Trehalose 15 gr per week for 12 weeks made by Dr. Rajabi Pharmaceutical Co.

Main outcome variables

Primary Outcome: Quality of life (by Peds.QL in <18 and SF-36 for those over the age of 18). Secondary outcomes: The size of liver and spleen, Plasma Sphingomyelin Level, Serum heparan sulfate level, Heparan sulfate and glycosaminoglycans levels in the urine, Lung HRCT, CBC, Liver enzymes (AST, ALT), LDH, Creatinine.

General information

Reason for update

Due to the rarity of the disease and death of some of previously identified patients (suffering from San Filippo

or Niemann-Pick syndrome), the sample size is reduced. All types of Niemann-Pick and mucopolysaccharidosis type III (Sanfilippo) will be entered to this trial.

Acronym

IRCT registration information

IRCT registration number: **IRCT20130829014521N23**

Registration date: **2023-10-27, 1402/08/05**

Registration timing: **prospective**

Last update: **2024-02-04, 1402/11/15**

Update count: **1**

Registration date

2023-10-27, 1402/08/05

Registrant information

Name

Amirhossein Sahebkar

Name of organization / entity

Mashhad University of Medical Sciences

Country

Iran (Islamic Republic of)

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+98 51 1882 9260

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Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2024-04-08, 1403/01/20

Expected recruitment end date

2026-04-09, 1405/01/20

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Investigation of the effects of trehalose on quality of life status and paraclinical findings of patients with lysosomal storage disorders (Niemann-Pick and mucopolysaccharidosis type III (Sanfilippo))

Public title

Investigation of the effects of trehalose on quality of life of patients with lysosomal storage disorders

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Patients with Niemann-Pick and mucopolysaccharidosis type III Informed consents of the patients or their parents(for children)

Exclusion criteria:

Special medical conditions

Age

No age limit

Gender

Both

Phase

2

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

Single

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Research Ethics Committees of Mashhad University of Medical Sciences

Street address

Ghoreshi Building, Daneshgah Street

City

Mashhad

Province

Razavi Khorasan

Postal code

9138813944

Approval date

2023-09-23, 1402/07/01

Ethics committee reference number

IR.MUMS.REC.1402.213

Health conditions studied

1

Description of health condition studied

Niemann-Pick

ICD-10 code

E75.2

ICD-10 code description

Other sphingolipidosis

2

Description of health condition studied

Mucopolysaccharidosis type III (Sanfilippo)

ICD-10 code

E76.2

ICD-10 code description

Other mucopolysaccharidoses

Primary outcomes

1

Description

Quality of life

Timepoint

Baseline and after 12 weeks

Method of measurement

Peds-ql questionnaire and SF-36 will be fulfilled for those under and over 18, respectively.

Secondary outcomes

1

Description

The size of liver and spleen

Timepoint

Baseline and after 12 weeks

Method of measurement

Sonography

2

Description

Plasma Sphingomyelin Level

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

3

Description

Serum heparan sulfate level

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

4

Description

Heparan sulfate and glycosaminoglycans levels in the urine

Timepoint

Baseline and after 12 weeks

Method of measurement

Urine Analysis

5

Description

Lung HRCT (High-resolution computed tomography)

Timepoint

Baseline and after 12 weeks

Method of measurement

HRCT

6

Description

Complete Blood Count (CBC)

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

7

Description

Liver enzymes including glutamic-oxaloacetic transaminase (SGOT) and Serum glutamic pyruvic transaminase (SGPT)

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

8

Description

Creatinine

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

9

Description

Lactate dehydrogenase (LDH)

Timepoint

Baseline and after 12 weeks

Method of measurement

Blood test

Intervention groups

1

Description

Intervention group: IV Trehalose 15 gr per week for 12 weeks

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Emam Hossein Children`s Hospital

Full name of responsible person

Amirhossein Sahebkar

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Emam Hossein Children`s Hospital, Km 10 Imam Khomeini Street

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Vice-chancellor of research and technology of the Ministry of health and medical education

Full name of responsible person

Yunes Panahi

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Ministry of Health and Medical Education, Eivanak Blvd., Farahzadi Blvd., Qods Town

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Email

info@hbi.ir

Web page address

<https://research.behdasht.gov.ir/>

Grant name

Grant code / Reference number

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

Yes

Title of funding source

Vice-chancellor of research and technology of the
Ministry of health and medical education

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**

Esfahan University of Medical Sciences

Full name of responsible person

Mohammad Baghernia

Position

Assistant Professor

Latest degree

Ph.D.

Other areas of specialty/work

Nutrition

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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