Clinical Trial Protocol
Iranian Registry of Clinical Trials

16 Sep 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)

Phone
+98 51 1882 9260

Email address
sahebkara@mums.ac.ir

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
Street address
Emam Hossein Children`s Hospital, Km 10 Imam Khomeini Street
City
Isfahan
Province
Isfehan
Postal code
8195163381
Phone
+98 31 3386 6266
Email
sahebkara@mums.ac.ir

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
Street address
Ministry of Health and Medical Education, Eivanak Blvd., Farahzadi Blvd., Qods Town
City
Tehran
Province
Tehran
Postal code
1467664961
Phone
+98 21 8145 5211
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

Street address

Department of Community Nutrition, School of Nutrition and Food Sciences, Isfahan University of Medical Sciences, Hezar Jerib Avenue

City

Isfahan

Province

Isfehan

Postal code

8174673461

Phone

+98 31 3792 3154

Email

Bagherniya@yahoo.com

Person responsible for scientific inquiries

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Amirhossein Sahebkar

Position

Professor

Latest degree

Ph.D.

Other areas of specialty/work

Pharmaceutical biotechnology

Street address

School of Pharmacy, University Campus, Azadi Square

City

Mashhad

Province

Razavi Khorasan

Postal code

9177948954

Phone

0098513180110

Email

sahebkara@mums.ac.ir

Person responsible for updating data

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Amirhossein Sahebkar

Position

Professor

Latest degree

Ph.D.

Other areas of specialty/work

Pharmaceutical biotechnology

Street address

School of Pharmacy, University Campus, Azadi Square

City

Mashhad

Province

Razavi Khorasan

Postal code

9177948954

Phone

+98 51 3180 1100

Email

sahebkara@mums.ac.ir

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