

Clinical Trial Protocol

Iranian Registry of Clinical Trials

16 Jul 2026

Investigation of the therapeutic effects of Trehalose in lysosomal storage diseases (Niemann-Pick types A & B, and Mucopolysaccharidosis type III/Sanfilippo syndrome)

Protocol summary

2020-03-03, 1398/12/13

Study aim

Reduction of Symptoms in LSD patients by Trehalose therapeutic effect

Design

This study has been designed as a non randomized controlled trial without control group and placebo.

Settings and conduct

Endocrinology and metabolism research institute, Imam Reza hospital

Participants/Inclusion and exclusion criteria

Patients with definitive diagnosis of Nieman-Pick and Sanfilippo syndrome

Intervention groups

Intravenous (IV) administration of Trehalose for 12 weeks in the intervention group

Main outcome variables

1-Psychopathology and behavioral assessment. 2-biochemical assays to evaluate the levels of serum enzymes such as sphingomyelinase. 3-serum and urinary concentrations of glycosaminoglycans (GAGs) such as heparan sulfate. 4-Sonographic assessment of liver and spleen size

General information

Reason for update

Acronym

Trehalose Therapeutic Effects in Lysosomal Storage Diseases (TTE LSD)

IRCT registration information

IRCT registration number: **IRCT20130829014521N16**

Registration date: **2020-03-03, 1398/12/13**

Registration timing: **prospective**

Last update: **2021-01-09, 1399/10/20**

Update count: **1**

Registration date

Registrant information

Name

Amirhossein Sahebkar

Name of organization / entity

Mashhad University of Medical Sciences

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Iran (Islamic Republic of)

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Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2020-06-20, 1399/03/31

Expected recruitment end date

2021-02-18, 1399/11/30

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Investigation of the therapeutic effects of Trehalose in lysosomal storage diseases (Niemann-Pick types A & B, and Mucopolysaccharidosis type III/Sanfilippo syndrome)

Public title

Trehalose and lysosomal storage diseases

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Patients with lysosomal storage disorders (Niemann-Pick type A & B and Sanfilippo syndrome)

Exclusion criteria:

Age

No age limit

Gender

Both

Phase

3

Groups that have been masked

No information

Sample size

Target sample size: 10

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

Ethics Committee of Mashhad University of Medical Sciences

Street address

Vice Chancellor for Research, Mashhad University of Medical Sciences, Ghoreishi building, Daneshgah Street

City

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Province

Razavi Khorasan

Postal code

9138813944

Approval date

2019-12-06, 1398/09/15

Ethics committee reference number

IR.MUMS.REC.1398.257

Health conditions studied

1

Description of health condition studied

Mucopolysaccharidosis type III/Sanfilippo syndrome

ICD-10 code

E76.22

ICD-10 code description

Sanfilippo mucopolysaccharidoses

2

Description of health condition studied

Niemann-Pick types A & B

ICD-10 code

E75.24

ICD-10 code description

Niemann-Pick disease

Primary outcomes

1

Description

Assessment of liver and spleen size

Timepoint

A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12))

Method of measurement

Liver and spleen size can be evaluated with sonography

2

Description

Psychopathology and behavioral assessment

Timepoint

A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12))

Method of measurement

Filling out the questionnaire

Secondary outcomes

1

Description

Evaluation of plasma sphingomyelin levels

Timepoint

A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12))

Method of measurement

Sphingomyelin assay kit

2

Description

Serum concentrations of heparan sulfate and glycosaminoglycans (GAGs)

Timepoint

A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12))

Method of measurement

Sphingomyelin and glycosaminoglycans (GAGs) assay kit

Intervention groups

1

Description

Intervention group: Intravenous (IV) administration of

Trehalose for 12 weeks
Category
Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center
Endocrinology and metabolism research institute,
Akbar hospital
Full name of responsible person
Dr.Amirhossein Sahebkar
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Sponsors / Funding sources

1

Sponsor

Name of organization / entity
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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
Mashhad 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
Mashhad University of Medical Sciences
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Dr.Amirhossein Sahebkar
Position
Associate professor
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Other areas of specialty/work
Pharmaceutical biotechnology
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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

"There is no further information"

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

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

No - There is not a plan to make this available