Clinical Trial Protocol
Iranian Registry of Clinical Trials

10 Jun 2026

Investigation of the therapeutic effects of Trehalose in a patient witn multiple sulfatase deficiency (MSD)

Protocol summary

Study aim
Reduction of Symptoms in MSD 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
Akbar children's hospital/ Faculty of medicine/ Faculty of pharmacy Patients will be selected from Akbar hospital and will receive trehalose intravenously (15 g/week) for 12 weeks, then related parameters will be measured at the end of the study.
Participants/Inclusion and exclusion criteria
Patient with definite diagnose of MSD
Intervention groups
Trehalose solution 15% (C12H22O11) that is a nonreducing disaccharide consisting two glucose units are linked in an α,α-1,1-glycosidic linkage
Main outcome variables
Assessment of health-related quality of life with Quality of Life (TAPQOL) questionnaire

General information

Reason for update
Acronym
TTEMSD
IRCT registration information
IRCT registration number: **IRCT20130829014521N17**
Registration date: **2021-03-15, 1399/12/25**
Registration timing: **registered_while_recruiting**

Last update: **2021-03-15, 1399/12/25**
Update count: **0**
Registration date
2021-03-15, 1399/12/25

Registrant information

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Amirhossein Sahebkar
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Recruitment status
Recruitment complete
Funding source

Expected recruitment start date
2021-02-19, 1399/12/01
Expected recruitment end date
2021-03-20, 1399/12/30
Actual recruitment start date
empty
Actual recruitment end date
empty
Trial completion date
empty

Scientific title
Investigation of the therapeutic effects of Trehalose in a patient witn multiple sulfatase deficiency (MSD)

Public title
Trehalose and multiple sulfatase deficiency (MSD)
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Patients with definitive diagnosis of MSD
Exclusion criteria:
Age
No age limit
Gender
Both

Phase
2
Groups that have been masked
No information
Sample size
Target sample size: **1**
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
کمیته اخلاق دانشگاه علوم پزشکی مشهد
Street address
Vice Chancellor for Research, Mashhad University of Medical Sciences, Ghoreishi building, Daneshgah Street
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9138813944
Approval date
2021-01-30, 1399/11/11
Ethics committee reference number
IR.MUMS.REC.1399.619

Health conditions studied

1

Description of health condition studied
Multiple sulfatase deficiency
ICD-10 code
E75.2
ICD-10 code description
Multiple sulfatase deficiency (also known as 'Austin disease', and 'mucosulfatidosis') is a very rare autosomal recessive lysosomal storage disease caused by a deficiency in multiple sulfatase enzymes, or in formylglycine-generating enzyme, which activate

Primary outcomes

1

Description
Assessment of health-related quality of life
Timepoint
A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12)
Method of measurement
The TAPQOL is a 43 item questionnaire consisting of 12multi-item scales that cover the domains physical, social, cognitive, and emotional functioning.

Secondary outcomes

1

Description
1- Sonographic 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
2-biochemical assays to evaluate the levels of serum enzymes such as ALT and AST
Timepoint
A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12)
Method of measurement
Enzymatic assay

3

Description
Evaluation of brain lesions
Timepoint
A before-and-after study (At the beginning and end of the intervention trial (Day 0 and week 12)
Method of measurement
Brain imaging methods (scan-MRI)

Intervention groups

1

Description
Intervention group: Trehalose solution 15% (C12H22O11) that is a nonreducing disaccharide consisting two glucose units are linked in an α,α-1,1-glycosidic linkage.
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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Shahid Fakouri blvd.

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Sponsors / Funding sources

1

Sponsor

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Mashhad University of Medical Sciences

Full name of responsible person
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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

Full name of responsible person
Amirhossein Sahebkar

Position
Associate professor

Latest degree
Ph.D.

Other areas of specialty/work
Medical Pharmacy

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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
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