

Clinical Trial Protocol

Iranian Registry of Clinical Trials

09 Jun 2026

Efficacy of enzyme replacement therapy with alpha-glucosidase in patient with late-onset Pompe disease

Protocol summary

Study aim

The therapeutic effect and adverse event of enzyme replacement therapy

Design

In this research, late-onset Pompe patients with clinical symptoms starting at adolescence, and initially as skeletal muscle weakness, and then as a result of conflicts in the heart and respiratory system, are investigated

Settings and conduct

20 mg of alpha-glucosidase enzyme every 14 days for 24 months

Participants/Inclusion and exclusion criteria

Inclusion criteria: 1. Latent pump failure 2. No enzymatic treatment Exclusion criteria: 1. Pregnancy 2. Unwillingness to participate in the study 3. Advanced liver disease and renal failure

Intervention groups

In this research, late-onset Pompe patients with clinical symptoms starting at adolescence are investigated

Main outcome variables

Muscular Enzymes and Respiratory function

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT2017111120981N2**

Registration date: **2017-11-19, 1396/08/28**

Registration timing: **retrospective**

Last update: **2018-06-11, 1397/03/21**

Update count: **1**

Registration date

2017-11-19, 1396/08/28

Registrant information

Name

Khadijeh Hajinaghitehrani

Name of organization / entity

Azad Medical University

Country

Iran (Islamic Republic of)

Phone

+98 21 2257 1228

Email address

khtehrani@iautmu.ac.ir

Recruitment status

Recruitment complete

Funding source

Deputy of Research of Islamic Azad University, Tehran Medical Sciences Branch

Expected recruitment start date

2014-03-05, 1392/12/14

Expected recruitment end date

2015-03-05, 1393/12/14

Actual recruitment start date

2014-03-11, 1392/12/20

Actual recruitment end date

2015-02-20, 1393/12/01

Trial completion date

empty

Scientific title

Efficacy of enzyme replacement therapy with alpha-glucosidase in patient with late-onset Pompe disease

Public title

Efficacy of enzyme replacement therapy with alpha-glucosidase in patient with late-onset Pompe disease

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Late-onset Pompe disease No received enzyme replacement therapy

Exclusion criteria:

Pregnancy Unwillingness to participate in the study
Advanced liver disease and renal failure

Age

No age limit

Gender

Both

Phase

N/A

Groups that have been masked

No information

Sample size

Target sample size: 2

Actual sample size reached: 2

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 on Research Islamic Azad
University, Tehran Medical Branch

Street address

Khaghani Ave, Islamic Azad University, Tehran
Medical Branch

City

Tehran

Province

Tehran

Postal code

19395-1495

Approval date

2016-02-24, 1394/12/05

Ethics committee reference number

IR.IAU.TMU.REC.1394.70

Health conditions studied**1****Description of health condition studied**

Pompe

ICD-10 code

E74.0

ICD-10 code description

Glycogen storage disease

Primary outcomes**1****Description**

Respiratory function

Timepoint

Every 6 months

Method of measurement

spirometry

2**Description**

Muscular force

Timepoint

evry 6 months

Method of measurement

physical exam

Secondary outcomes**1****Description**

Muscular Enzymes

Timepoint

evry 6 months

Method of measurement

lab data

Intervention groups**1****Description**

20 mg of alpha-glucosidase enzyme every 14 days

Category

Treatment - Drugs

Recruitment centers**1****Recruitment center****Name of recruitment center**

Bu-ali hospital

Full name of responsible person

Dr. Khadijeh Hajinaghi Tehrani

Street address

Damavand Ave

City

Tehran

Province

Tehran

Postal code

19395-1495

Phone

+98 21 2200 6660

Email

dr_tehrani10@yahoo.com

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Research Deputy of Islamic Azad University, Tehran
Medical Branch

Full name of responsible person

Elham Kohbor

Street address

Khaghani Ave, Islamic Azad University, Tehran
Medical Branch

City

Tehran

Province

Tehran

Postal code

19395-1495

Phone

+98 21 2200 6660

Email

dr_tehrani10@yahoo.com

Grant name**Grant code / Reference number****Is the source of funding the same sponsor organization/entity?**

Yes

Title of funding source

Research Deputy of Islamic Azad University, Tehran
Medical Branch

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**

Bu-ali Hospital

Full name of responsible person

Khadijeh Hajinaghi Tehrani

Position

Neurologist / Plan Designer

Latest degree

Specialist

Other areas of specialty/work

Neurology

Street address

Bu-ali Hospital

City

Tehran

Province

Tehran

Postal code

19395-1495

Phone

+98 912 149 3324

Fax**Email**

Khtehrani@iautmu.ac.ir

Web page address

Person responsible for scientific inquiries

Contact**Name of organization / entity**

Bu-ali Hospital

Full name of responsible person

Khadijeh Hajinaghi Tehrani

Position

Neurologist

Latest degree

Specialist

Other areas of specialty/work

Neurology

Street address

Bu-ali Hospital

City

Tehran

Province

Tehran

Postal code

19395-1495

Phone

+98 21 3334 8036

Fax**Email**

Khtehrani@iautmu.ac.ir

Web page address

Person responsible for updating data

Contact**Name of organization / entity**

Bu-ali Hospital

Full name of responsible person

Ehsan Jangholi

Position

MD

Latest degree

Medical doctor

Other areas of specialty/work

General Practitioner

Street address

Bu-ali Hospital

City

Tehran

Province

Tehran

Postal code

19395-1495

Phone

+98 21 3334 8036

Fax

Email

ehsanjangholi@yahoo.com

Web page address**Sharing plan****Deidentified Individual Participant Data Set (IPD)**

Yes - There is a plan to make this available

Study Protocol

Yes - There is a plan to make this available

Statistical Analysis Plan

Yes - There is a plan to make this available

Informed Consent Form

Yes - There is 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

Title and more details about the data/document

The only some part of data will be shared.

When the data will become available and for how long

5 months after publish

To whom data/document is available

only academic researcher

Under which criteria data/document could be used

By official permission

From where data/document is obtainable

by the corresponding author

What processes are involved for a request to access data/document

by the official request to the corresponding author, if she/he agrees to it.

Comments