

Clinical Trial Protocol

Iranian Registry of Clinical Trials

23 Jun 2026

Evaluation of response rate to hydroxycobalamin treatment in patients with methylmalonic acidemia

Protocol summary

Study aim

evaluation of response to hydroxycobalamin in patients with methylmalonic acidemia

Design

all patients with isolated methylmalonic acidemia or methylmalonic acidemia with homocystinuria confirmed on ms/ms and urine organic acid or genetic that are about 15 patients. this study no need to randomization and it is phase 3 clinical trial.

Settings and conduct

patients with isolated methylmalonic acidemia or methylmalonic acidemia with homocystinuria confirmed on ms/ms and urine organic acid or genetic in mashhad city at 1400 that are about 15 patients. after obtaining informed consent, they will enter the study and asked them if they received hydroxycobalamin, discontinue this drug about 1 week before test. then we received 5cc urine for mma, then in stable patients we administered hydroxycobalamin acetate amp from sterop company 1mg im daily for 3 consecutive days. in unstable patients we administered hydroxycobalamin 2mg iv for 7 days. the day after injections we will repeat urine mma. this study no need to blinding.

Participants/Inclusion and exclusion criteria

entry condition: patients with isolated methylmalonic acidemia or methylmalonic acidemia with homocystinuria confirmed on ms/ms and urine organic acid or genetic in mashhad city at 1400 that are about 15 patients. no entry condition: patients that are suspicious to this disease and haven't certain diagnose or poor controlled patients that haven't enough cooperation.

Intervention groups

patients with methylmalonic acidemia

Main outcome variables

urine methylmalonic acid

General information

Reason for update

Acronym

MMA

IRCT registration information

IRCT registration number: **IRCT20211207053311N1**

Registration date: **2022-03-07, 1400/12/16**

Registration timing: **registered_while_recruiting**

Last update: **2022-03-07, 1400/12/16**

Update count: **0**

Registration date

2022-03-07, 1400/12/16

Registrant information

Name

Afsane Nazari

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 51 3501 2620

Email address

nazari.afsane@gmail.com

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2022-02-04, 1400/11/15

Expected recruitment end date

2022-06-05, 1401/03/15

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Evaluation of response rate to hydroxycobalamin treatment in patients with methylmalonic acidemia

Public title

Evaluation of response rate to hydroxycobalamin treatment in patients with methylmalonic acidemia

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

patients with isolated methylmalonic acidemia or methylmalonic acidemia with hemocystinuria confirmed in MS/MS and urine organic acid or genetic

Exclusion criteria:

suspicious patients that have not certain diagnosis and poor control patients that have not cooperation

Age

To **18 years** old

Gender

Both

Phase

3

Groups that have been masked

No information

Sample size

Target sample size: **15**

More than 1 sample in each individual

Number of samples in each individual: **2**
urine methylmalonic acid

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

Daneshgah Ave, mashhad university of medical sciences

City

mashhad

Province

Razavi Khorasan

Postal code

9138813944

Approval date

2022-01-25, 1400/11/05

Ethics committee reference number

IR.MUMS.MEDICAL.REC.1400.828

Health conditions studied

1

Description of health condition studied

methylmalonic acidemia

ICD-10 code

E71.120

ICD-10 code description

E71.120

Primary outcomes

1

Description

decrease more than 50 percent in urine methylmalonic acid considered positive and the patient is responsible to hydroxycobalamin.

Timepoint

urine methylmalonic acid before starting the intervention and then 3 days after received hydroxycobalamin in stable patients and after 7 days in unstable patients.

Method of measurement

evaluation of urine methylmalonic acid

Secondary outcomes

empty

Intervention groups

1

Description

Intervention group:

Category

Treatment - Other

Recruitment centers

1

Recruitment center

Name of recruitment center

Akbar hospital

Full name of responsible person

Afsane Nazari

Street address

Kaveh Blvd, Akbar hospital

City

mashhad

Province

Razavi Khorasan

Postal code

9177897157

Phone

+98 51 3871 3803

Fax

+98 51 3870 9201

Email

ak.pr@mums.ac.ir

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

majid ghayour mobarhan

Street address

Daneshgah ave, mashhad university of medical sciences

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Email

vcresraech@mums.ac.ir

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

afsane nazari

Position

fellowship

Latest degree

Specialist

Other areas of specialty/work

Pediatrics

Street address

No.18, pajuhesh 1 Ave, vakil abad 79 Blvd, mashhad, Iran

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+98 51 3501 2620

Email

nazari.afsane@gmail.com

Person responsible for scientific inquiries

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

afsane nazari

Position

fellowship

Latest degree

Specialist

Other areas of specialty/work

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Person responsible for updating data

Contact

Name of organization / entity

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afsane nazari

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Email

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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