

Clinical Trial Protocol

Iranian Registry of Clinical Trials

10 Jun 2026

Effectiveness of Tetrahydrobiopterin for improvement of dietary regimen of children with Phenylketonuria

Protocol summary

Summary

This study aims to evaluate the effects of Tetrahydrobiopterin (BH4) on the treatment and dietary regimen of children with phenylketonuria (PKU). In this study, 20 Children ranging from 1 to 10 years old with controlled PKU are enrolled. Patients are prescribed normal dietary regimen for three days. Afterward, phenylalanine (Phe) level will be measured and BH4 is administered orally with a daily dose of 20 mg/kg. After 24 and 48 hours, Phe level will be re-measured. Then patients with Phe level less than 600 $\mu\text{mol/L}$ or more than 1200 $\mu\text{mol/L}$ and those with intolerance to treatment will be eliminated and the rest of the patients continue treatment in the second phase of the study. In addition to BH4, these children are prescribed 50 mg of phenylalanine powder daily (in addition to the current Phe in dietary regimen). If by the end of the week, the level of Phe is normal, another 50 mg of Phe is added for next week and this process goes on weekly up to 3 months until Phe dose is twice as the initial dose and if tolerated, patients return to normal diet.

General information

Acronym

IRCT registration information

IRCT registration number: **IRCT2016100230082N1**

Registration date: **2017-06-01, 1396/03/11**

Registration timing: **retrospective**

Last update:

Update count: **0**

Registration date

2017-06-01, 1396/03/11

Registrant information

Name

Peyman Eshraghi

Name of organization / entity

Imam Reza hospital, Mashhad University of Medicine

Country

Iran (Islamic Republic of)

Phone

+98 38593045

Email address

eshraghip2@mums.ac.ir

Recruitment status

Recruitment complete

Funding source

Mashhad University of Medical Sciences

Expected recruitment start date

2015-01-01, 1393/10/11

Expected recruitment end date

2016-03-09, 1394/12/19

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Effectiveness of Tetrahydrobiopterin for improvement of dietary regimen of children with Phenylketonuria

Public title

Effect of kuvan on relaxation of diet in phenylketonuria

Purpose

Supportive

Inclusion/Exclusion criteria

Inclusion criteria: patients with controlled PKU; age range of 1-10 years; lack of underlying disorders (e.g. malnutrition, hepatic and renal diseases). Exclusion criteria: BH4 deficiency; Phe levels less than 600 micromoles/liter or more than 1200 micromoles/liter; presence of fever or acute illness; intolerance to BH4 therapy.

Age

From **1 year** old to **10 years** old

Gender

Both

Phase

N/A

Groups that have been masked

No information

Sample size

Target sample size: **20**

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

Central Building of Mashhad University of Medical Sciences (Ghorshi), Daneshgah 16, Daneshgah Street

City

Mashhad

Postal code**Approval date**

2013-11-20, 1392/08/29

Ethics committee reference number

920734

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

mild phenylketonuria

ICD-10 code

E70.1

ICD-10 code description

Other hyperphenylalaninaemias

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

Serum levels of phenylalanine

Timepoint

In first phases, after three days of free diet, then 24 and 48 hours after prescription of BH4. In second phase, measured weekly

Method of measurement

HPLC

Secondary outcomes

empty

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

Kuvan, 100 mg oral tablet, daily, 20 mg/kg.

Phenylalanine, orally powder, daily, 50 mg (additive phenylalanine of dietary regime), If level of Phe is normal, another 50 mg of Phe is added for next week and this process goes on until Phe dose is twice as the initial dose and if tolerated, they return to normal diet

Category

Treatment - Drugs

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

Imam Reza Hospital

Full name of responsible person

Dr Vajihe Chalak

Street address

Department Of Pediatric Endocrinology, Imam Reza Hospital, Imam Reza Square

City

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Sponsors / Funding sources**1****Sponsor****Name of organization / entity**

Mashhad University of Medical Sciences, Vice Chancellor for Research

Full name of responsible person

Dr Mohsen Tafaghodi

Street address

Central Building of Mashhad University of Medical Sciences (Ghorshi), Daneshgah 16, Daneshgah Street

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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, Vice Chancellor

for Research

Proportion provided by this source

100

Public or private sector

empty

Domestic or foreign origin

empty

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

empty

Person responsible for general inquiries

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Dr Vajihe Chalak

Position

Pediatric Assistant

Other areas of specialty/work

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Person responsible for scientific inquiries

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Associate Proffesor of Pediatric Endocrinology

Other areas of specialty/work

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Person responsible for updating data

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Sharing plan

Deidentified Individual Participant Data Set (IPD)

empty

Study Protocol

empty

Statistical Analysis Plan

empty

Informed Consent Form

empty

Clinical Study Report

empty

Analytic Code

empty

Data Dictionary

empty