Clinical Trial Protocol
Iranian Registry of Clinical Trials

10 Jun 2026

Assessing the effectiveness of simvastatin in the treatment of patients with pachyonychia congenita: A crossover randomized controlled trial.

Protocol summary

Study aim

The efficacy of simvastatin in reducing signs and symptoms in patients with pachyonychia congenita.

Design

A multicenter concealed, randomized, blinded, sham-controlled, crossover clinical trial of 20 patients, enrolled between January 2019 and September 2019 and followed for 14 months.

Settings and conduct

The patients will be randomly assigned into two groups. The participants in the case group will receive a single dose of simvastatin 40 mg per day (20 mg in individuals aged 10-18) for six months followed by a washout period of 2 months. Then they will crossover to the control group. The participants in the control arm will receive placebo for six months. After a washout period of two months, they will crossover to the case group. Patients will be evaluated throughout the study for clearance of palmoplantar and nail lesions, and overall improvement in quality of life. Histologic specimens will be obtained at the beginning and the end of each phase for investigating the molecular effects of simvastatin.

Participants/Inclusion and exclusion criteria

Inclusion Criteria: Age between10 to 65 years. Weight equal or more than 32kg. Mentally able to respond to questions. Exclusion criteria: Comorbidities such as cardiovascular, renal or hepatic impairment or diabetes. Drug-related concerns such as a previous history of myopathy, hypersensitivity to statins, pregnancy, and breastfeeding. Confounding factors such as cutaneous infection, previous consumption of statins, and drug/alcohol abuse.

Intervention groups

Patients with genetically confirmed diagnosis of pachyonychia congenita are randomly assigned in two groups. The control group will receive placebo and the intervention group will receive simvastatin for 6 months. After a washout period, the groups will cross over.

Main outcome variables

Hyperkeratotic lesion thickness, Nail thickness, Skin pathological changes, Pain, Quality of life.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20160320027109N3**
Registration date: **2019-01-09, 1397/10/19**
Registration timing: **registered_while_recruiting**

Last update: **2019-01-09, 1397/10/19**
Update count: **0**

Registration date

2019-01-09, 1397/10/19

Registrant information

Name

Fahimeh Abdollahimajd

Name of organization / entity

Skin Research Center

Country

Iran (Islamic Republic of)

Phone

+98 21 2274 1507

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fabdollahimajd@sbmu.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2019-01-01, 1397/10/11

Expected recruitment end date

2019-09-02, 1398/06/11

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Assessing the effectiveness of simvastatin in the treatment of patients with pachyonychia congenita: A crossover randomized controlled trial.

Public title

Effect of simvastatin in treatment of pachyonychia congenita

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

.Weight equal or more than 32kg Mentally healthy and able to clearly understand, assess, and respond questions.

Exclusion criteria:

Cardiovascular disease. Renal impairment. Hepatic impairment or severe gastrointestinal disease. Diabetes. History of myopathy. Active fingernail and cutaneous cyst infection. If there is an infection in skin or fingernail, it must be treated before starting the trial. Pregnant or breastfeeding. History of hypersensitivity to statins. Previous consumption of statins for other reasons. History of drug or alcohol abuse.

Age

From **10 years** old to **65 years** old

Gender

Both

Phase

2-3

Groups that have been masked

- Participant
- Care provider
- Investigator
- Outcome assessor
- Data analyser

Sample size

Target sample size: **20**

Randomization (investigator's opinion)

Randomized

Randomization description

Recruited patients are randomly allocated to either treatment or control arm. Both arms are in equal size. Randomization sequence is generated by Random Allocation Software version 1.0 May 2004, using a simple random method. It generates a randomization code for each participant (individual randomization). Randomization is run just one time at the beginning of the study. Then participants crossover based on the first randomized allocation. Sequentially numbered sealed opaque envelops are used to conceal the allocation. Each participant receives one envelope containing the randomization code.

Blinding (investigator's opinion)

Triple blinded

Blinding description

Blinding of the participants is achieved via administration of placebo which has the same package, figure, color, taste, smell, size, dosage, and route of administration as the drug used in the case group (simvastatin and the placebo are provided by the same company). In order to blind the outcome assessors, the researchers, and the data analyzers, simvastatin and placebo are referred to as A and B throughout the investigation.

Placebo

Used

Assignment

Crossover

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Ethics committee of Skin Research Center Shahid Beheshti University of Medical Sciences

Street address

The SBMU SRC (Skin Research Center),Shohada-E-Tajrish Educational Hospital,Qods Sq. ,Tehran, Iran

City

Tehran

Province

Tehran

Postal code

1989934148

Approval date

2018-12-09, 1397/09/18

Ethics committee reference number

IR.SBMU.SRC.REC.1397.019

Health conditions studied

1

Description of health condition studied

Pachyonychia congenita

ICD-10 code

Q84.5

ICD-10 code description

Enlarged and hypertrophic nails

Primary outcomes

1

Description

Area occupied by heperkeratotic lesions on palms and soles

Timepoint

At the beginning and end of each study phase

Method of measurement

Photography, imagej software

2

Description
Plantar lesions maximum thickness
Timepoint
At the beginning and end of each study phase
Method of measurement
20 MHz B mode Ultrasonography

3

Description
Maximum nail thickness
Timepoint
At the beginning and end of each study phase
Method of measurement
20 MHz B mode Ultrasonography

4

Description
Self-report nail grooming need
Timepoint
At the beginning and end of each study phase
Method of measurement
Likert scale

5

Description
Self-report hyperkeratotic lesion grooming need
Timepoint
At the beginning and end of each study phase
Method of measurement
Likert scale

6

Description
Physicians satisfaction with patients response
Timepoint
At the beginning and end of each study phase
Method of measurement
Likert scale

7

Description
Patients mobility,treadmill tolerance test
Timepoint
At the beginning and end of each study phase
Method of measurement
The maximum time a patient could tolerate walking a on a treadmill

8

Description
Mcgill pain questionnaire
Timepoint
At the beginning and end of each study phase
Method of measurement
Questionnaire

9

Description
Dermatology Life Quality Index (DLQI)
Timepoint
At the beginning and end of each study phase
Method of measurement
Questionnaire

10

Description
Keratin gene expression. KRT 6a, 6b, 6c, 16, 17
Timepoint
At the beginning and end of each study phase
Method of measurement
Histophatologic evluation

Secondary outcomes

1

Description
Simvastatin side-effects
Timepoint
Throughout the treatment.
Method of measurement
Any exacerbation of clinical signs or laboratory changes

2

Description
Patient compliance with medication
Timepoint
Throughout the treatment.
Method of measurement
According to physicians estimates, Likert scale.

Intervention groups

1

Description
Intervention group: Participants will be treated with simvastatin, given as a single oral dose of 40 mg in the evening. In patients aged 10 to 18 years, the treatment will be initiated with 20 mg of simvastatin. Before the beginning of the treatment and after 6 months on treatment, patients will be evaluated for the change in area and thickness of the lesions and indicators related to pain and quality of life. A biopsy specimen will be obtained both before and after the treatment to evaluate gene expression profiles. After a washout period of two months without treatment, patients will be assigned to the control group.
Category
Treatment - Drugs

2

Description
Control group:Participants will receive a single oral dose in the evening. Before the beginning of the treatment

and after 6 months on treatment, patients will be evaluated for the change in area and thickness of the lesions and indicators related to pain and quality of life. A biopsy specimen will be obtained both before and after the treatment to evaluate gene expression profiles. After a washout period of two months without treatment, patients will be assigned to the control group.

Category
Treatment - Drugs

Recruitment centers

1

Recruitment center
Name of recruitment center
Dermatology clinic, Shohada-e Tajrish Hospital
Full name of responsible person
Fahimeh Abdollahimajd
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2

Recruitment center
Name of recruitment center
Department of Dermatology and Cutaneous Biology, Thomas Jefferson University
Full name of responsible person
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Web page address

Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Shahid Beheshti University of Medical Sciences
Full name of responsible person
Dr. Hamideh Moravvej
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Skin Research Center, Shohada-e Tajrish Hospital, Qods sq, Tehran, Iran
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1989934148
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Email
fabdollahimajd@sbmu.ac.ir
Grant name
Grant code / Reference number
Is the source of funding the same sponsor organization/entity?
Yes
Title of funding source
Shahid Beheshti University of Medical Sciences
Proportion provided by this source
40
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
Shahid Beheshti University of Medical Sciences
Full name of responsible person
Fateme Rajabi
Position
Dermatology resident
Latest degree
Medical doctor
Other areas of specialty/work
Dermatology
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Person responsible for scientific inquiries

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Person responsible for updating data

Contact
Name of organization / entity
Thomas Jefferson University, Dermatology & cutaneous biology
Full name of responsible person
Masoomeh Faghankhani
Position
Postdoc research fellow
Latest degree
Medical doctor
Other areas of specialty/work
Dermatology
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Sharing plan

Deidentified Individual Participant Data Set (IPD)
Yes - There is 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
Yes - There is 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
Title and more details about the data/document
It is not yet known if there will be a plan to make this available
When the data will become available and for how long
It is not yet known if there will be a plan to make this available
To whom data/document is available
It is not yet known if there will be a plan to make this available
Under which criteria data/document could be used
It is not yet known if there will be a plan to make this available
From where data/document is obtainable
It is not yet known if there will be a plan to make this available
What processes are involved for a request to access data/document
It is not yet known if there will be a plan to make this available
Comments