Clinical Trial Protocol
Iranian Registry of Clinical Trials

27 Oct 2026

Follow-up of Severe Congenital Neutropenia Patients Receiving PegGCSF (PD lasta); Study of Early and Late Drug Complications

Protocol summary

Study aim
Evaluation the advantages and disadvantages of PDlasta in Severe Congenital Neutropenia Patients with age older than 12 years

Design
After sign the inform consent form 10 Patients older than 12 years old that treated with PDlgrastim already and have all of the inclusion criteria enroll the study

Settings and conduct
Patients receive the PDlasta for free and treat with PDlasta according to protocol and visit weekly till 3 months and then monthly to evaluate clinical status, CBC and ANC (Absolute Neutrophil Count), PDlasta side effects and dosage adjustment

Participants/Inclusion and exclusion criteria
Inclusion criteria: All patients with severe congenital neutropenia older than 12 years old and treated with Filgrastim in past, arrest in myeloid maturation, without mutation in GCSF receptor gene ;Exclusion criteria: neutropenia due to other diseases, huge splenomegaly, coexistent disorders

Intervention groups
Patients receive the PDlasta and we evaluate the clinical sign and symptoms and also absolute neutrophil count

Main outcome variables
Increase in absolute neutrophil count and decrease in hospitalization due to infectious diseases.

General information

Reason for update

Acronym

IRCT registration information
IRCT registration number: **IRCT20150125020786N3**
Registration date: **2018-07-08, 1397/04/17**
Registration timing: **prospective**

Last update: **2018-07-08, 1397/04/17**
Update count: **0**

Registration date
2018-07-08, 1397/04/17

Registrant information

Name
Marzieh Maddah

Name of organization / entity
Immunology, Asthma & Allergy Research Institute

Country
Iran (Islamic Republic of)

Phone
+98 21 6693 5855

Email address
pourpakz@tums.ac.ir

Recruitment status
Recruitment complete

Funding source
Pouyesh Darou

Expected recruitment start date
2018-07-23, 1397/05/01

Expected recruitment end date
2019-07-23, 1398/05/01

Actual recruitment start date
empty

Actual recruitment end date
empty

Trial completion date
empty

Scientific title
Follow-up of Severe Congenital Neutropenia Patients Receiving PegGCSF (PD lasta); Study of Early and Late Drug Complications

Public title
Follow-up of Severe Congenital Neutropenia Patients Receiving PegGCSF (PD lasta); Study of Early and Late Drug Complications

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Adult patients and children with severe congenital neutropenia over 12 years Severe Congenital Neutropenia Patients Myeloid maturation arrest in bone marrow Already successfully treated with Filgrastim Negative mutation in GCSF receptor Normal spleen and liver in abdominal sonography Consent to Participate in study and sign inform consent Cooperative patient for close follow-up

Exclusion criteria:

Secondary neutropenia due to chemotherapy or other diseases Life-threatening complications such as anaphylaxis or splenic rupture due to Filgrastim Uncooperative patient for close follow-up Severe Spleenomegaly patients with other chronic diseases such as heart or hepatic disease or ...

Age

From **12 years** old

Gender

Both

Phase

1-2

Groups that have been masked

No information

Sample size

Target sample size: **10**

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 Immunology , Asthma and Allergy Research Institiute

Street address

4th Floor, Building No. 3, Tehran - Children's Hospital Medical Center, Keshavarz Blvd. Qarib Street

City

Tehran

Province

Tehran

Postal code

1419733151

Approval date

2014-01-04, 1392/10/14

Ethics committee reference number

182412

Health conditions studied

1

Description of health condition studied

Congenital Neutropenia

ICD-10 code

D70

ICD-10 code description

Neutropenia

Primary outcomes

1

Description

Absolute Neutrophil Count

Timepoint

first day of study , 7th day, 14th day, 21st day, 28th day, 35th day, 42nd day, 49th day, 56th day, 3th day, 70th day, 77th day, 84th day, 91st day then every month until reciving the drug

Method of measurement

Blood Sample

Secondary outcomes

1

Description

Infection

Timepoint

Monthly

Method of measurement

Clinical questionnare

2

Description

Hospitalization rate

Timepoint

Monthly

Method of measurement

Clinical questionnare

Intervention groups

1

Description

PegGCSF produced by Pouyesh Darou Prescribed weekly or every 2 weeks and the dose will be adjusted based on response to drug

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center
Immunology , Asthma and Allergy Research Institute

Full name of responsible person
Marzieh Maddah

Street address
4th Floor, Building No. 3, Children's Hospital Medical Center, Qarib Street, Keshavarz Blvd.

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity
Pouyesh Darou biopharmaceutical Co.

Full name of responsible person
Dr Tabatabai

Street address
No. 13, 5th Ave., Fatemi St.

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123456798

Phone
+98 21 8899 7248

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info@pooyeshdarou.com

Grant name

Grant code / Reference number

Is the source of funding the same sponsor organization/entity?
Yes

Title of funding source
Pouyesh Darou biopharmaceutical Co.

Proportion provided by this source
50

Public or private sector
Private

Domestic or foreign origin
Domestic

Category of foreign source of funding
empty

Country of origin

Type of organization providing the funding
Industry

2

Sponsor

Name of organization / entity
Immunology, Asthma and Allergy research Institute

Full name of responsible person
Zahra Pourpak

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4th Floor, Building No. 3, Children's Hospital Medical Center, Keshavarz Blvd., Qarib Street

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Email
pourpakz@tums.ac.ir

Grant name

Grant code / Reference number

Is the source of funding the same sponsor organization/entity?
No

Title of funding source
Immunology, Asthma and Allergy research Institute

Proportion provided by this source
50

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
Immunology, Asthma and Allergy Research Institute

Full name of responsible person
Marzieh Maddah

Position
Researcher and MD , specialist

Latest degree
Specialist

Other areas of specialty/work
Pediatrics

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Person responsible for scientific inquiries

Contact

Name of organization / entity

Immunology, Asthma and Allergy Research Institute

Full name of responsible person

Mostafa Moin

Position

Professor, pediatrician, Immunology, Asthma and Allergy subspecilist

Latest degree

Subspecialist

Other areas of specialty/work

Immunology

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Person responsible for updating data

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Name of organization / entity

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Position

researcher

Latest degree

Specialist

Other areas of specialty/work

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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

Patients recruitment will be before January 1st 2019.

Study Protocol

Undecided - It is not yet known if there will be a plan to make this available

Statistical Analysis Plan

Not applicable

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

Not applicable