

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

12 Jun 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 Splenomegaly 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 Institute

##### 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 questionnaire

### 2

#### Description

Hospitalization rate

#### Timepoint

Monthly

#### Method of measurement

Clinical questionnaire

## 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.

**City**

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**Province**

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**Postal code**

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**Phone**

+98 21 6691 9589

**Email**

marzieh.maddah@gmail.com

## 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.

**City**

Tehran

**Province**

Tehran

**Postal code**

123456798

**Phone**

+98 21 8899 7248

**Email**

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

**Street address**

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

**City**

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**Phone**

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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 subspecialist  
**Latest degree**  
Subspecialist  
**Other areas of specialty/work**  
Immunology  
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4th Floor, Building No. 3, Children's Hospital Medical Center, Qarib Street  
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mmoin@tums.ac.ir  
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## Person responsible for updating data

### Contact

**Name of organization / entity**  
Immunology, Asthma and Allergy Research Institute  
**Full name of responsible person**  
Marzieh Maddah

**Position**  
researcher  
**Latest degree**  
Specialist  
**Other areas of specialty/work**  
Pediatrics  
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**Web page address**

## 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