Safety and Efficacy of turoctocog alfa pegol (N8-GP) in Prophylaxis and Treatment of Bleeds in Previously N8-GP Treated Patients with Severe Haemophilia A

Published: 21-02-2018
Last updated: 10-04-2024

Primary objective:To investigate the safety of turoctocog alfa pegol during continuous use for prevention and treatment of bleeding episodes of previously turoctocog alfa pegol treated severe haemophilia A patients.Secondary objectives:To...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Coagulopathies and bleeding diatheses (excl thrombocytopenic)
Study type	Interventional

Summary

ID

NL-OMON46773

Source
ToetsingOnline

Brief title
Pathfinder8

Condition

- Coagulopathies and bleeding diatheses (excl thrombocytopenic)

Synonym
blood clothing disorder, Haemophilia A

Research involving
Human

Sponsors and support

Primary sponsor: Novo Nordisk
Source(s) of monetary or material Support: Novo Nordisk

Intervention

Keyword: Haemophilia A, Prophylaxis, Turotocog alfa pegol (N8-GP)

Outcome measures

Primary outcome

Primary endpoint:

Number of adverse events reported

Secondary outcome

Key secondary endpoints:

* Incidence of FVIII inhibitors *0.6 BU

* Number of bleeding episodes on prophylaxis

* Number of spontaneous bleeding episodes on prophylaxis

* Haemostatic effect of turoctocog alfa pegol when used for treatment of

bleeding episodes assessed as: Excellent, Good, Moderate, or None

* Number of turoctocog alfa pegol injections required per bleeding episode

Study description

Background summary

The rationale for performing this trial is to allow the continued evaluation of the safety and efficacy of turoctocog alfa pegol in order to obtain additional data on long-term use. Introducing a twice or three times weekly prophylactic dosing regimen to the majority of patients is intended to show potential improvement in clinical outcomes by converting patients to a milder bleeding phenotype. Joint health and target joints will be assessed and evaluated at

inclusion and at end of trial.

Study objective

Primary objective:
To investigate the safety of turoctocog alfa pegol during continuous use for prevention and treatment of bleeding episodes of previously turoctocog alfa pegol treated severe haemophilia A patients.

Secondary objectives:
To investigate the following in severe haemophilia A patients previously treated with turoctocog alfa pegol:
* Development of FVIII inhibitors
* Efficacy of turoctocog alfa pegol prophylaxis
* Haemostatic efficacy of turoctocog alfa pegol when used for treatment of bleeds

Study design

This phase 3 trial is a multi-centre, multi-national, open-label, non-randomised trial evaluating safety and efficacy of turoctocog alfa pegol during prophylaxis treatment and treatment of bleeds. There will be three turoctocog alfa pegol treatment arms (dosing once weekly, twice weekly, and three times weekly) and no comparator.

Intervention

Intravenous injection of turoctocog alfa pegol once, twice or three times weekly in order to prevent bleeding episodes. Additionally, breakthrough bleeds will also be treated with an intravenous injection of turoctocog alfa pegol.

Study burden and risks

The trial will provide assessments of longer-term safety of N8-GP and establish information on joint health and target joints.
Currently available clinical data from the pathfinder programme support that N8*GP has the intended haemostatic potential. The key risks associated with N8-GP administration are inhibitor development and allergic/hypersensitivity reactions, which are well-known class effects for FVIII products.

Contacts

Public
Novo Nordisk

Flemingweg 18
Alphen a/d Rijn 2408 AV
NL
Scientific
Novo Nordisk

Flemingweg 18
Alphen a/d Rijn 2408 AV
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1. Male patients of all ages with the diagnosis of severe congenital haemophilia A (FVIII activity <1%) based on medical records
2. On-going participation in NN7088-3859 (pathfinder2), or NN7088-3885 (pathfinder5) at the time of transfer

Exclusion criteria

1. Known or suspected hypersensitivity to trial product including allergy to hamster protein or related products
2. Any disorder, except for conditions associated with haemophilia, which in the investigator's opinion might jeopardise patient's safety or compliance with the protocol
3. Current participation in any clinical trial (except NN7088-3859 (pathfinder2) or NN7088-3885 (pathfinder5)) of an approved or nonapproved investigational medicinal product

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	26-06-2018
Enrollment:	4
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Nog niet bekend
Generic name:	turoctocog alfa pegol (N8-GP)

Ethics review

Approved WMO

Date:	21-02-2018
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Approved WMO

Date:	24-04-2018
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Approved WMO

Date:	10-07-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	30-07-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	05-10-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	06-11-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	31-07-2019
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	06-08-2019
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	08-11-2019
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	02-07-2020
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam

	(Rotterdam)
Approved WMO Date:	29-09-2020
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	22-10-2020
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2017-003788-36-NL
CCMO	NL64362.078.18