Research study to look at how well the drug concizumab works in your body if you have haemophilia with inhibitors

Estado	**Tipo de Participantes**	**Rangos de Edad**
Fin Reclutamiento	No aportado	Mayores de 64 , Adultos (18-64 años) , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Hombres	Fase III	91
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia	NO

Información

Identificador
2023-506832-33-00 (CTIS)

Cod. Protocolo
NN7415-4311

Transicionado 2018-004889-34

Área terapéutica
Enfermedades [C] - Anormalidades congénitas, hereditarias y neonatología [C16]|

Enfermedad investigada
haemophilia A (HA) with inhibitors and haemophilia B (HB) with inhibitors

Título Científico
Efficacy and Safety of Concizumab prophylaxis in patients with haemophilia A or B with inhibitors

Justificación

No aportado

Objetivo Principal

To compare the effect of concizumab prophylaxis to no prophylaxis (on-demand treatment with bypassing agents) in reducing the number of bleeding episodes in adult and adolescent patients with haemophilia A or B with inhibitors

Variables de Evaluación Primaria

The number of treated spontaneous and traumatic bleeding episodes. On demand (arm 1) From randomisation (week 0) up until start of concizumab treatment (at least 24 weeks) Concizumab (arm 2) From start of the new concizumab dosing regimen (week 0) up until the primary analysis cut-off (at least 32 weeks)

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To compare the patient-reported outcomes (PROs) after treatment with concizumab prophylaxis vs no prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors
To investigate the safety of concizumab prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors
To investigate the PK and PD parameters of concizumab prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors

Variables de Evaluación Secundaria

Change in SF36v2 bodily pain from start of treatment (week 0) until week 24
Change in SF36v2 physical functioning From start of treatment (week 0) until week 24
Number of treated spontaneous bleeding episodes On demand (arm 1) From randomisation (week 0) up until start of concizumab treatment (at least 24 weeks) Concizumab (arm 2) From start of the new concizumab dosing regimen (week 0) up until the primary analysis cut-off (at least 32 weeks)
Number of treated spontaneous and traumatic joint bleeds On demand (arm 1) From randomisation (week 0) up until start of concizumab treatment (at least 24 weeks) Concizumab (arm 2) From start of the new concizumab dosing regimen (week 0) up until the primary analysis cut-off (at least 32 weeks)
Number of treated spontaneous and traumatic target joint bleeds On demand (arm 1) From randomisation (week 0) up until start of concizumab treatment (at least 24 weeks) Concizumab (arm 2) From start of the new concizumab dosing regimen (week 0) up until the primary analysis cut-off (at least 32 weeks)
Number of thromboembolic events On demand (arm 1 main part) From randomisation to on demand treatment up until start of concizumab treatment Concizumab (arms 2-4) Before the pause: From start of concizumab treatment

(week 0) up until 7 weeks after the treatment was paused as well as After the pause: From start of concizumab treatment (week 0) up until the primary analysis cut-off (at least 32 weeks) Concizumab (arm 1 extension part) From start of the new concizumab dosing regimen (visit 9a) up
Number of thromboembolic events Concizumab Before the pause: From start of treatment (week 0) up until 7 weeks after the treatment was paused as well as After the pause: From start of concizumab treatment up until the end of trial (up to 339 weeks)
Number of hypersensitivity type reactions On demand (arm 1 main part) From randomisation to on demand treatment up until start of concizumab treatment Concizumab (arms 2-4) Before the pause: From start of concizumab treatment (week 0) up until 7 weeks after the treatment was paused as well as After the pause: From start of concizumab treatment (week 0) up until the primary analysis cut-off (at least 32 weeks) Concizumab (arm 1 extension part) From start of the new concizumab dosing regimen (visi
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Number of injection site reactions On demand (arm 1 main part) From randomisation to on demand treatment up until start of concizumab treatment Concizumab (arms 2-4) Before the pause: From start of concizumab treatment (week 0b) up until 7 weeks after the treatment was paused as well as After the pause: From start of concizumab treatment (week 0a) up until the primary analysis cut-off (at least 32 weeks) Concizumab (arm 1 extension part) From start of the new concizumab dosing regimen (visit 9a)
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Pre-dose (trough) concizumab plasma concentration (Ctrough) Prior to the concizumab administration at week 24 (after restart)
Pre-dose thrombin peak Prior to the concizumab administration at week 24 (after restart)
Pre-dose free TFPI concentration Prior to the concizumab administration at week 24 (after restart)
Maximum concizumab plasma concentration (Cmax) From 0 to 24 hours where 0 is time of the concizumab dose at week 24 (after restart)
Area under the concizumab plasma concentration-time curve (AUC) From 0 to 24 hours where 0 is time of the concizumab dose at week 24 (after restart)

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Informed consent obtained before any trial-related activities. Trial-related activities are any procedures that are carried out as part of the trial, including activities to determine suitability for the trial Male aged 12 years at the time of signing informed consent. Body weight >25 kg at screening Congenital Haemophilia A or B of any severity with documented history of inhibitor (0.6 BU). Patient has been prescribed, or in need of, treatment with bypassing agents in the last 24 weeks prior to screening (for patients not previously enrolled in NN7415-4310).

Criterios de Exclusión

Known or suspected hypersensitivity to any constituent of the trial product or related products A known systemic inflammatory condition requiring systemic treatment at screening Treatment with emicizumab within 180 days before screening. Ongoing or planned Immune Tolerance Induction treatment Any disorder, except for conditions associated with haemophilia, which in the investigators opinion might jeopardise patients safety or compliance with the protocol Previous participation in this trial. Participation is defined as signed informed consent. However, this is not applicable for patients who were screen failed at Sponsors decision due to the treatment pause. Participation in any clinical trial of an approved or non-approved investigational medicinal product within 5 half-lives or 30 days from screening, whichever is longer (not applicable for patients from NN7415-4310). Platelets 100x109/L at screening Fibrinogen below laboratory lower normal limit at screening Hepatic dysfunction defined as AST and/or ALT > 3 times the upper limit combined with total bilirubin > 1,5 times the upper limit at screening Renal impairment defined as estimated Glomerular Filtration Rate (eGFR) 30 ml/min/1.73 m2 for serum creatinine measured at screening Known inherited or acquired coagulation disorder other than congenital haemophilia History of thromboembolic disease. Current clinical signs of or treatment for thromboembolic disease. Patients who in the judgement of the investigator are considered at high risk of thromboembolic events c

Calendario

(Última actualización: 04/09/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
12/09/2019	15/11/2019	19/11/2019	16/03/2020	06/08/2020

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
21/01/2021	No aportado	No aportado	No aportado	No aportado

Promotor

Novo Nordisk A/S Denmark

Novo Alle 1

Contact Person

EU Submission Hub

+4544448888

EUSUBHUB@novonordisk.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Centros

Cerrado (05/09/2023)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Activo (15/11/2019)

HOSPITAL UNIVERSITARIO CENTRAL DE ASTURIAS

Oviedo

ASTURIAS

Servicio de Hematología y Hemoterapia

Activo (04/12/2019)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Unidad de Coagulopatías-Servicio de Hematología

Activo (21/01/2020)

HOSPITAL UNIVERSITARIO REGIONAL DE MÁLAGA

Málaga

MÁLAGA

Servicio de Hematología y Hemoterapía

Activo (27/11/2019)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCÍO

Sevilla

SEVILLA

Servicio de Hematología y Hemoterapia - Unidad de Ensayos Clínicos

Medicamentos

Concizumab C 40 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Principios Activos: N/A

Experimental

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Principios Activos: N/A

Experimental

Concizumab C 40 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Principios Activos: N/A

Experimental

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Principios Activos: N/A

Experimental

Sin resultados

Research study to look at how well the drug concizumab works in your body if you have haemophilia with inhibitors

State	Type of participants	Age Ranges
End of recruitment	Not provided	Older than 64 , Adults (18-64 years) , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Men	Phase III	91
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness	NO

Information

Identifier
2023-506832-33-00 (CTIS)

Protocol Code
NN7415-4311

Transitioned 2018-004889-34

Therapeutic area
Diseases [C] - Congenital, Hereditary, and Neonatal Diseases and Abnormalities [C16]|

Investigated Disease
haemophilia A (HA) with inhibitors and haemophilia B (HB) with inhibitors

Scientific Title
Efficacy and Safety of Concizumab prophylaxis in patients with haemophilia A or B with inhibitors

Rationale

Not provided

Main Objective

To compare the effect of concizumab prophylaxis to no prophylaxis (on-demand treatment with bypassing agents) in reducing the number of bleeding episodes in adult and adolescent patients with haemophilia A or B with inhibitors

Primary Endpoints

The number of treated spontaneous and traumatic bleeding episodes. On demand (arm 1) From randomisation (week 0) up until start of concizumab treatment (at least 24 weeks) Concizumab (arm 2) From start of the new concizumab dosing regimen (week 0) up until the primary analysis cut-off (at least 32 weeks)

Temporary moments of secondary assessment

Not provided

Secondary Objective

To compare the patient-reported outcomes (PROs) after treatment with concizumab prophylaxis vs no prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors
To investigate the safety of concizumab prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors
To investigate the PK and PD parameters of concizumab prophylaxis in adult and adolescent patients with haemophilia A or B with inhibitors

Secondary Endpoints

Change in SF36v2 bodily pain from start of treatment (week 0) until week 24
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Temporary moments of secondary assessment

Not provided

Inclusion criteria

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Calendar

(Last Update: 04/09/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
12/09/2019	15/11/2019	19/11/2019	16/03/2020	06/08/2020

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
21/01/2021	Not aported	Not aported	Not aported	Not aported

Sponsor

Novo Nordisk A/S Denmark

Novo Alle 1

Contact Person

EU Submission Hub

+4544448888

EUSUBHUB@novonordisk.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Sites

Closed (05/09/2023)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Active (15/11/2019)

HOSPITAL UNIVERSITARIO CENTRAL DE ASTURIAS

Oviedo

ASTURIAS

Servicio de Hematología y Hemoterapia

Active (04/12/2019)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Unidad de Coagulopatías-Servicio de Hematología

Active (21/01/2020)

HOSPITAL UNIVERSITARIO REGIONAL DE MÁLAGA

Málaga

MÁLAGA

Servicio de Hematología y Hemoterapía

Active (27/11/2019)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCÍO

Sevilla

SEVILLA

Servicio de Hematología y Hemoterapia - Unidad de Ensayos Clínicos

Medication

Concizumab C 40 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Active Principles: N/A

Experimental

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Active Principles: N/A

Experimental

Concizumab C 40 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Active Principles: N/A

Experimental

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Active Principles: N/A

Experimental

No results