Estudio de investigación sobre la eficacia de concizumab si tiene hemofilia A o B con o sin inhibidores (explorer10)

Estado	**Tipo de Participantes**	**Rangos de Edad**
Reclutando	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Hombres	Fase III	105
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética	NO

Información

Identificador
2023-506925-13-00 (CTIS)

Cod. Protocolo
NN7415-4616

Transicionado 2020-000504-11

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

Enfermedad investigada
Hemofilia A o B con o sin inhibidores

Título Científico
Estudio abierto para investigar la eficacia, la seguridad y la farmacocinética de la profilaxis con concizumab en niños menores de 12 años con hemofilia A o B con o sin inhibidores

Justificación

No aportado

Objetivo Principal

Establecer la superioridad de la profilaxis con concizumab en niños <12 años con hemofilia A o B con inhibidores que no han recibido tratamiento previamente con concizumab en comparación con su tratamiento a demanda previo en el número de episodios hemorrágicos espontáneos y traumáticos tratados. Establecer la superioridad de la profilaxis con concizumab en niños <12 años con hemofilia A o B sin inhibidores que no han recibido tratamiento previamente con concizumab en comparación con su tratamiento a demanda previo en el número de episodios hemorrágicos espontáneos y traumáticos tratados

Variables de Evaluación Primaria

Para pacientes con inhibidores que hayan recibido tratamiento a demanda durante al menos 26 semanas en las últimas 52 semanas previas a la inclusión: número de episodios hemorrágicos espontáneos y traumáticos tratados. Periodo: desde el inicio del tratamiento (semana 0) hasta el momento del corte para el análisis principal. Unidad: número de episodios.
Para pacientes sin inhibidores que hayan recibido tratamiento a demanda durante al menos las 52 semanas previas a la inclusión: número de episodios hemorrágicos espontáneos y traumáticos tratados. Periodo: desde el inicio del tratamiento (semana 0) hasta el momento del corte para el análisis principal. Unidad: número de episodios

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Evaluar la eficacia de la profilaxis con concizumab en niños <12 años con hemofilia A o B sin inhibidores que no han recibido tratamiento previamente con concizumab en comparación con su tratamiento profiláctico previo en el número de episodios hemorrágicos espontáneos y traumáticos tratados Evaluar la seguridad de concizumab administrado una vez al día en niños <12 años con hemofilia A o B con o sin inhibidores que no han recibido tratamiento previamente con concizumab Evaluar la seguridad de concizumab administrado una vez al día en pacientes que han recibido tratamiento previamente con concizumab mediante uso compasivo Evaluar la farmacocinética y farmacodinámica de concizumab administrado una vez al día en niños <12 años con hemofilia A o B con o sin inhibidores que no han recibido tratamiento previamente con concizuma

Variables de Evaluación Secundaria

Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks

prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand during at least the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand during at least the last 52 weeks prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand at least the last 52 weeks prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand at least the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated spontaneous and traumatic bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: Number of treatment emergent adverse events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Number of thromboembolic events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Number of hypersensitivity type reactions, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of reaction(s).
Secondary supportive: Number of injection site reactions, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of reaction(s).
Secondary supportive: Number of patients who develop antibodies to concizumab yes/no, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of patient(s).
Secondary supportive: Number of treatment emergent adverse events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Concizumab plasma concentrations prior to dosing, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Week 32. Unit: ng/mL
Secondary supportive: Peak thrombin generation prior to dosing, reported both separately for inhibitor and non-

inhibitor patients and combined. Time frame: Week 32. Unit: nM
Secondary supportive: Free TFPI concentration prior to dosing, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Week 32. Unit: ng/mL
Secondary supportive: Pre-dose (trough) concizumab plasma concentration (Ctrough), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Prior to the concizumab administration at week 20. Unit: ng/mL
Secondary supportive: Maximum concizumab plasma concentration (Cmax), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From 0 to 24 hours where 0 is the time of the concizumab dose at week 20. Unit: ng/mL
Secondary supportive: Area under the concizumab plasma concentration-time curve (AUC), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From 0 to 24 hours where 0 is the time of the concizumab dose at week 20. Unit: ng*hr/mL

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Obtención del consentimiento/asentimiento informado antes de realizar cualquier actividad relacionada con el estudio. Las actividades relacionadas con el estudio son todos aquellos procedimientos llevados a cabo como parte del estudio, incluidas las actividades para determinar si un individuo es idóneo para participar. Diagnóstico de hemofilia A congénita grave (FVIII <1 %), hemofilia B congénita moderada/grave (FIX ¿2 %), o hemofilia congénita con inhibidores. Para el grupo 1 solamente: hombres <12 años en el momento de la firma del consentimiento informado. Para el grupo 1 solamente: pacientes con inhibidores (HAcI o HBcI): 1) Pacientes con HAcI con antecedentes médicos de al menos 26 semanas de tratamiento a demanda(b) en total en las últimas 52 semanas previas a la inclusión(a). 2) Pacientes con HBcI con antecedentes médicos de al menos 26 semanas de tratamiento a demanda(b) en total en las últimas 52 semanas previas a la inclusión(a). 3) Pacientes con HBcI independientemente de la pauta y la duración del tratamiento previo para la hemofilia(b). (a) Para pacientes menores de 1 año a los que se les haya diagnosticado hemofilia <1 año antes de la inclusión, los antecedentes médicos desde el momento del diagnóstico serán suficientes, siempre y cuando haya disponibles antecedentes médicos de un total de al menos 26 semanas de tratamiento relevante. (b) El tratamiento a demanda o profiláctico que reúne los requisitos para poder usarse durante este estudio es únicamente el que sirva para tratar las hemorragias con medicamentos que contengan factor de coagulación intravenosa. Para el grupo 1 solamente: pacientes sin inhibidores (HAcI o HBcI): 1) Pacientes con antecedentes médicos de al menos 52 semanas de tratamiento a demanda(b),(c) durante el último año previo a la inclusión y con al menos 3 hemorragias tratadas documentadas(d) durante este periodo. 2) Pacientes con antecedentes médicos de al menos 26 semanas de tratamiento profiláctico(b) en total en las últimas 52 semanas previas a la inclusión. (a) Para los pacientes menores de 1 año a los que se les haya diagnosticado hemofilia <1 año antes de la inclusión, los antecedentes médicos desde el momento del diagnóstico serán suficientes, siempre y cuando haya disponibles antecedentes médicos de un total de al menos 26 semanas de tratamiento relevante. (b) El tratamiento a demanda o profiláctico que reúne los requisitos para poder usarse durante este estudio es únicamente el que sirva para tratar las hemorragias con medicamentos que contengan factor de coagulación intravenosa. (c) Tratamiento profiláctico a corto plazo o relacionado con la cirugía (en relación con una hemorragia grave). (d) En los participantes <2 años, no hay limitación en el número de hemorragias tratadas documentadas en los antecedentes médicos. Para el grupo 2 solamente: pacientes de sexo masculino (independientemente de la edad) que han recibido tratamiento previamente con concizumab mediante uso compasivo

Criterios de Exclusión

Hipersensibilidad conocida o sospechada al tratamiento del estudio o a fármacos relacionados.
Trastorno de coagulación hereditario o adquirido conocido y distinto de la hemofilia congénita.
Tratamiento de inducción de inmunotolerancia en curso o previsto.
Antecedentes de enfermedad tromboembólica(a). Signos clínicos actuales de enfermedad tromboembólica o tratamiento para esta. Pacientes que, a criterio del investigador, se consideran en alto riesgo de acontecimientos tromboembólicos(b).
(a) Incluye la trombosis arterial y venosa, como el infarto de miocardio, la embolia pulmonar, el infarto/la trombosis cerebral, la trombosis venosa profunda, otros acontecimientos tromboembólicos clínicamente significativos y la oclusión de las arterias periféricas. (b) Los factores de riesgo tromboembólicos podrían incluir, entre otros, hipercolesterolemia, diabetes mellitus, hipertensión, obesidad, tabaquismo, antecedentes familiares de acontecimientos tromboembólicos, arterioesclerosis y otras afecciones asociadas a un mayor riesgo de acontecimientos tromboembólicos

Calendario

(Última actualización: 06/08/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
23/03/2022	01/07/2022	09/01/2023	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
No aportado	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

No iniciado (02/06/2023)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

Murcia

MURCIA

Activo (01/07/2022)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Activo (23/01/2023)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Activo (16/09/2022)

HOSPITAL UNIVERSITARIO REGIONAL DE MALAGA

Málaga

MÁLAGA

No iniciado (--/--/----)

Vall D'hebron Institut De Recerca

Barcelona

BARCELONA

N/A

Medicamentos

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Principios Activos: N/A

Experimental

Concizumab C 10 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

Sin resultados

A research study on how well concizumab works for you if you have haemophilia A or B with or without inhibitors (explorer10)

State	Type of participants	Age Ranges
Recruiting	Incapable subjects of giving consent	Less than 18 , Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Men	Phase III	105
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics	NO

Information

Identifier
2023-506925-13-00 (CTIS)

Protocol Code
NN7415-4616

Transitioned 2020-000504-11

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

Investigated Disease
Haemophilia A or B with or without inhibitors

Scientific Title
Open-label study investigating efficacy, safety and pharmacokinetics of concizumab prophylaxis in children below 12 years with haemophilia A or B with or without inhibitors

Rationale

Not provided

Main Objective

To establish superiority of concizumab prophylaxis in concizumab-naïve children <12 years with haemophilia A or B with inhibitors compared to their previous on-demand treatment on the number of treated spontaneous and traumatic bleeding episodes. To establish superiority of concizumab prophylaxis in concizumab-naïve children <12 years with haemophilia A or B without inhibitors compared to their previous on-demand treatment on the number of treated spontaneous and traumatic bleeding episodes.

Primary Endpoints

For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of treated spontaneous and traumatic bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
For non-inhibitor patients treated on demand during at least the last 52 weeks prior enrolment: Number of treated spontaneous and traumatic bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the efficacy of concizumab prophylaxis in concizumab-naïve children <12 years with haemophilia A or B without inhibitors compared to their previous PPX treatment on the number of treated spontaneous and traumatic bleeding episodes To evaluate the safety of concizumab administered once-daily in concizumab-naïve children <12 years with haemophilia A or B with or without inhibitors To evaluate the safety of concizumab administered once-daily in patients previously treated with concizumab via compassionate use To evaluate pharmacokinetics and pharmacodynamics of concizumab administered once-daily in concizumab-naïve children <12 years with haemophilia A or B with or without inhibitors

Secondary Endpoints

Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks

prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For inhibitor patients with at least 26 weeks on-demand treatment during the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand during at least the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand during at least the last 52 weeks prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand at least the last 52 weeks prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Supportive secondary: For non-inhibitor patients treated on-demand at least the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated spontaneous and traumatic bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of all bleeding episodes (spontaneous and traumatic). Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated spontaneous bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated joint bleeding episodes. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: For non-inhibitor patients with at least 26 weeks PPX treatment during the last 52 weeks prior enrolment: Number of treated bleeding episodes in baseline target joints. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of episode(s).
Secondary supportive: Number of treatment emergent adverse events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Number of thromboembolic events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Number of hypersensitivity type reactions, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of reaction(s).
Secondary supportive: Number of injection site reactions, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of reaction(s).
Secondary supportive: Number of patients who develop antibodies to concizumab yes/no, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of patient(s).
Secondary supportive: Number of treatment emergent adverse events, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From start of treatment (week 0) up until the primary analysis cut-off. Unit: Count of event(s).
Secondary supportive: Concizumab plasma concentrations prior to dosing, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Week 32. Unit: ng/mL
Secondary supportive: Peak thrombin generation prior to dosing, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Week 32. Unit: nM
Secondary supportive: Free TFPI concentration prior to dosing, reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Week 32. Unit: ng/mL

Secondary supportive: Pre-dose (trough) concizumab plasma concentration (Ctrough), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: Prior to the concizumab administration at week 20. Unit: ng/mL
Secondary supportive: Maximum concizumab plasma concentration (Cmax), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From 0 to 24 hours where 0 is the time of the concizumab dose at week 20. Unit: ng/mL
Secondary supportive: Area under the concizumab plasma concentration-time curve (AUC), reported both separately for inhibitor and non-inhibitor patients and combined. Time frame: From 0 to 24 hours where 0 is the time of the concizumab dose at week 20. Unit: ng*hr/mL

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Informed consent/assent obtained before any study-related activities. Study-related activities are any procedures that are carried out as part of the study, including activities to determine suitability for the study. Diagnosis of congenital severe haemophilia A (FVIII <1%) or moderate/severe congenital haemophilia B (FIX 2%), or congenital haemophilia with inhibitors. For arm 1 only: Male aged <12 years of age at the time of signing informed consent. For arm 1 only: Patients with inhibitors (HAwI or HBwI): 1) Patients with HAwI with historical medical records of a total of at least 26 weeks of on-demand treatment(b) within the last 52 weeks prior to enrolment(a). 2) Patients with HBwI with historical medical records of a total of at least 26 weeks of on-demand treatment(b) within the last 52 weeks prior to enrolment(a). 3) Patients with HBwI regardless of the regimen and duration of previous haemophilia treatment(b). (a) For patients below 1 year of age that have been diagnosed with haemophilia <1 year prior to enrolment, historical medical records from time of diagnosis will suffice as long as medical records of a total of at least 26 weeks of relevant treatment is available. (b) On-demand or PPX treatment qualifying for this study is understood as patient-treatment solely for bleeds with intravenous coagulation factor-containing products. For arm 1 only: Patients without inhibitors (HA or HB): 1) Patients with historical medical records of at least 52 weeks of on-demand treatment(b),(c) during the last year prior to enrolment and with at least 3 documented treated bleeds(d) during this period. 2) Patients with historical medical records of a total of at least 26 weeks of PPX treatment(b) within the last 52 weeks prior to enrolment(a). (a) For patients below 1 year of age that have been diagnosed with haemophilia <1 year prior to enrolment, historical medical records from time of diagnosis will suffice as long as medical records of a total of at least 26 weeks of relevant treatment is available. (b) On-demand or PPX treatment qualifying for this study is understood as patient-treatment solely for bleeds with intravenous coagulation factor-containing products. (c) Surgery related PPX or short-term PPX (e.g., in relation to a severe bleed) is not allowed. (d) For participants <2 years of age there is no limitation for number of documented treated bleeds in the medical history. For arm 2 only: Male patients (regardless of age) previously treated with concizumab via compassionate use.

Exclusion criteria

Known or suspected hypersensitivity to study intervention or related products.
Known inherited or acquired coagulation disorder other than congenital haemophilia.
Ongoing or planned Immune Tolerance Induction treatment.
History of thromboembolic disease(a). 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(b). (a) Includes arterial and venous thrombosis including myocardial infarction, pulmonary embolism, cerebral infarction/thrombosis, deep vein thrombosis, other clinically significant thromboembolic events and peripheral artery occlusion. (b) Thromboembolic risk factors could include, but are not limited to, hypercholesterolemia, diabetes mellitus,

hypertension, obesity, smoking, family history of thromboembolic events, arteriosclerosis, other conditions associated with increased risk of thromboembolic events.

Calendar

(Last Update: 06/08/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
23/03/2022	01/07/2022	09/01/2023	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
Not aported	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

not initialized (02/06/2023)

HOSPITAL CLINICO UNIVERSITARIO VIRGEN DE LA ARRIXACA

Murcia

MURCIA

Active (01/07/2022)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Active (23/01/2023)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Active (16/09/2022)

HOSPITAL UNIVERSITARIO REGIONAL DE MALAGA

Málaga

MÁLAGA

not initialized (--/--/----)

Vall D'hebron Institut De Recerca

Barcelona

BARCELONA

N/A

Medication

Concizumab C 100 mg/mL PDS290
SOLUTION FOR INJECTION
SUBCUTANEOUS

-

Active Principles: N/A

Experimental

Concizumab C 10 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

No results