A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of NXT007 in Persons with Severe or Moderate Hemophilia A

Estado	**Tipo de Participantes**	**Rangos de Edad**
Reclutando	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Adultos (18-64 años) , Adolescentes (12-17 años) , Niños (2-11 años) , Lactantes y preescolar (28 días - 23 meses)
Género	**Fases**	**Participantes esperados**
Hombres	Fase I , Fase II	4
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2023-503906-35-00 (CTIS)

Cod. Protocolo
WP44714

Área terapéutica
·Enfermedades [C] - Anormalidades congénitas, hereditarias y neonatología [C16]
·Enfermedades [C] - Hematología [C15]
·Procesos fisiológicos [G] - Fenómenos químicos [G02]

Enfermedad investigada
Severe or Moderate Hemophilia A with or without factor VIII inhibitors

Título Científico

A Phase I/II Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of NXT007 in Persons with Severe or Moderate Hemophilia A

Justificación

No aportado

Objetivo Principal

To evaluate the safety of multiple doses of NXT007

Variables de Evaluación Primaria

1. Incidence and severity of adverse events with severity determined according to National Cancer Institute Common Terminology Criteria for Adverse Events version 5 .0 (NCI CTCAE v5.0)
2. Change from baseline in selected clinical laboratory test results
3. Change from baseline in vital signs and electrocardiogram (ECG) parameters

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To characterize the pharmacokinetics of NXT007 following multiple doses of NXT007
To evaluate the immunogenicity of multiple doses of NXT007
To evaluate the recurrence of or de novo factor VIII (FVIII) inhibitor occurrence, and change to FVIII inhibitor titer over time
To explore the efficacy of multiple doses of NXT007

Variables de Evaluación Secundaria

1. Plasma concentration of NXT007 at specified timepoints and relevant pharmacokinetic (PK) parameters 2. Prevalence of anti-drug antibodies (ADAs) at baseline and incidence of ADAs during the study 3. Number and proportion of participants with anti-FVIII inhibitors (titer >= 0.6 BU/mL) at specified timepoints 4. Number of bleeding events over time 5. Annualized bleeding rate (ABR) for treated bleeds, all bleeds, treated spontaneous bleeds, and treated joint bleeds

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male (based on sex at birth) Diagnosis of severe (FVIII:C <1 IU/dL) or moderate [FVIII coagulant activity (FVIII:C) between >= 1 IU/dL and <= 5 IU/dL)] congenital hemophilia A (HA) with or without inhibitors against FVIII Participants with FVIII inhibitors: participants using recombinant activated factor VII (rFVIIa) or willing to switch to rFVIIa as primary bypassing agent for the treatment of breakthrough bleeds, trauma, or procedures Historic local FVIII inhibitor test results being available during screening to confirm any previous inhibitor history and current status Participants who previously successfully completed immune tolerance induction (ITI) must have done so at least 5 years before screening and must have no evidence of inhibitor recurrence (permanent or temporary) since. FVIII tolerance defined as < 0.6 bethesda unit (BU)/mL (< 1.0 BU/mL only for laboratories with an historical sensitivity cutoff for inhibitor detection of 1.0 BU/mL) and in vivo recovery (IVR) > 66%

Criterios de Exclusión

Inherited or acquired bleeding disorders other than congenital HA Protein C activity, protein S free antigen, or anti-thrombin III activity levels below the lower limit of the reference range at screening At high risk for thrombotic microangiopathy (TMA), including past personal or family history of TMA, in the investigators judgment Previous or current treatment for thromboembolic disease (with the exception of previous catheter-associated thrombosis for which anti-thrombotic treatment is not currently ongoing) or signs of thromboembolic disease Personal history of ischemic heart disease, cerebrovascular disease, or diabetes mellitus (for participants =12 and <60 years) History of severe allergic or anaphylactic reactions to monoclonal antibody therapy and to chimeric or humanized antibodies or fusion proteins

Calendario

(Última actualización: 05/06/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
15/09/2023	11/10/2023	03/11/2023	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

F. Hoffmann-La Roche AG Switzerland

Grenzacherstrasse 124

Contact Person

Trial Information System - TISL

41616881111

global.eudract@roche.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 (--/--/----)

Hospital Universitario La Paz

Madrid

MADRID

Hematology

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

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Hematology

Medicamentos

NXT007
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Principios Activos: N/A

Experimental

Sin resultados

A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of NXT007 in Persons with Severe or Moderate Hemophilia A

State	Type of participants	Age Ranges
Recruiting	Incapable subjects of giving consent	Less than 18 , Adults (18-64 years) , Teens (12-17 years) , Children (2-11 years) , Infants and preschool (28 days - 23 months)
Gender	**Phases**	**Expected Participants**
Men	Phase I , Phase II	4
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2023-503906-35-00 (CTIS)

Protocol Code
WP44714

Therapeutic area
·Diseases [C] - Congenital, Hereditary, and Neonatal Diseases and Abnormalities [C16]
·Diseases [C] - Hemic and Lymphatic Diseases [C15]
·Body processes [G] - Chemical Phenomena [G02]

Investigated Disease
Severe or Moderate Hemophilia A with or without factor VIII inhibitors

Scientific Title

A Phase I/II Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics, and Efficacy of NXT007 in Persons with Severe or Moderate Hemophilia A

Rationale

Not provided

Main Objective

To evaluate the safety of multiple doses of NXT007

Primary Endpoints

1. Incidence and severity of adverse events with severity determined according to National Cancer Institute Common Terminology Criteria for Adverse Events version 5 .0 (NCI CTCAE v5.0)
2. Change from baseline in selected clinical laboratory test results
3. Change from baseline in vital signs and electrocardiogram (ECG) parameters

Temporary moments of secondary assessment

Not provided

Secondary Objective

To characterize the pharmacokinetics of NXT007 following multiple doses of NXT007
To evaluate the immunogenicity of multiple doses of NXT007
To evaluate the recurrence of or de novo factor VIII (FVIII) inhibitor occurrence, and change to FVIII inhibitor titer over time
To explore the efficacy of multiple doses of NXT007

Secondary Endpoints

1. Plasma concentration of NXT007 at specified timepoints and relevant pharmacokinetic (PK) parameters 2. Prevalence of anti-drug antibodies (ADAs) at baseline and incidence of ADAs during the study 3. Number and proportion of participants with anti-FVIII inhibitors (titer >= 0.6 BU/mL) at specified timepoints 4. Number of bleeding events over time 5. Annualized bleeding rate (ABR) for treated bleeds, all bleeds, treated spontaneous bleeds, and treated joint bleeds

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male (based on sex at birth) Diagnosis of severe (FVIII:C <1 IU/dL) or moderate [FVIII coagulant activity (FVIII:C) between >= 1 IU/dL and <= 5 IU/dL)] congenital hemophilia A (HA) with or without inhibitors against FVIII Participants with FVIII inhibitors: participants using recombinant activated factor VII (rFVIIa) or willing to switch to rFVIIa as primary bypassing agent for the treatment of breakthrough bleeds, trauma, or procedures Historic local FVIII inhibitor test results being available during screening to confirm any previous inhibitor history and current status Participants who previously successfully completed immune tolerance induction (ITI) must have done so at least 5 years before screening and must have no evidence of inhibitor recurrence (permanent or temporary) since. FVIII tolerance defined as < 0.6 bethesda unit (BU)/mL (< 1.0 BU/mL only for laboratories with an historical sensitivity cutoff for inhibitor detection of 1.0 BU/mL) and in vivo recovery (IVR) > 66%

Exclusion criteria

Inherited or acquired bleeding disorders other than congenital HA Protein C activity, protein S free antigen, or anti-thrombin III activity levels below the lower limit of the reference range at screening At high risk for thrombotic microangiopathy (TMA), including past personal or family history of TMA, in the investigators judgment Previous or current treatment for thromboembolic disease (with the exception of previous catheter-associated thrombosis for which anti-thrombotic treatment is not currently ongoing) or signs of thromboembolic disease Personal history of ischemic heart disease, cerebrovascular disease, or diabetes mellitus (for participants =12 and <60 years) History of severe allergic or anaphylactic reactions to monoclonal antibody therapy and to chimeric or humanized antibodies or fusion proteins

Calendar

(Last Update: 05/06/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
15/09/2023	11/10/2023	03/11/2023	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

F. Hoffmann-La Roche AG Switzerland

Grenzacherstrasse 124

Contact Person

Trial Information System - TISL

41616881111

global.eudract@roche.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

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

Hospital Universitario La Paz

Madrid

MADRID

Hematology

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

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Hematology

Medication

NXT007
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Active Principles: N/A

Experimental

No results