Fitusiran prophylaxis in male pediatric subjects aged 1 to less than 12 years with hemophilia A or B

Estado	Tipo de Participantes	Rangos de Edad
EC Finalizado	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Niños (2-11 años) , Lactantes y preescolar (28 días - 23 meses) , Recién nacidos (0-27 días)
Género	**Fases**	**Participantes esperados**
Hombres	Fase II , Fase III	32
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Multicéntrico internacional	profilaxis, tratamiento, seguridad, eficacia, farmacodinamica, dosis	NO

Información

Identificador
2024-512501-76-00 (CTIS)

Cod. Protocolo
EFC15467

Transicionado 2019-000679-18

Área terapéutica
Enfermedades [C] - Hematología [C15]|

Enfermedad investigada
Hemophilia

Título Científico
ATLAS-PEDS: An open-label, multinational study of fitusiran prophylaxis in pediatric subjects ages 1 to less than 12

years with hemophilia A or B

Justificación

No aportado

Objetivo Principal

To confirm appropriate dose levels of fitusiran when administered to male pediatric participants (ages 1 to <12 years of age) with severe hemophilia A or B

Variables de Evaluación Primaria

Plasma antithrombin (AT) activity levels

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To characterize the safety and tolerability
To determine fitusiran plasma concentrations at selected time points

Variables de Evaluación Secundaria

Number of participants reported with adverse events
Fitusiran plasma concentrations

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male, aged 1 to <12 years at the time of enrollment. Severe hemophilia A or B (Factor VIII (FVIII) <1% or Factor IX (FIX) 2%) Participants must have inhibitory antibodies to FVIII or FIX and must meet one of the following Nijmegen-modified Bethesda assay results criteria: - Inhibitor titer of 0.6 BU/mL at screening, OR - Inhibitor titer of <0.6 BU/mL at screening with medical record evidence of 2 consecutive titers 0.6 BU/mL, OR - Inhibitor titer of <0.6 BU/mL at

screening with medical record evidence of 1 inhibitor titer 0.6 BU/mL and a history of anamnestic response or severe allergic reaction (anaphylaxis or nephrotic syndrome) Adequate peripheral venous access, as determined by the Investigator, to allow the blood draws required by the study protocol Weight requirements at the time of enrollment: 8 to <45 kg Willing and able to comply with the study requirements and to provide signed written informed consent obtained from parent(s)/legal guardian (hereinafter the parent) and written or oral assent obtained from participant, per local and national requirements

Criterios de Exclusión

Known co-existing bleeding disorders other than hemophilia A or B Inadequate renal function History of multiple drug allergies or history of allergic reaction to an oligonucleotide or N-Acetylgalactosamine (GalNAc) Subjects with central or peripheral indwelling catheters, with history of venous access complications leading to hospitalization and/or systemic anticoagulation therapy. History of intolerance to subcutaneous (SC) injection(s) Use of emicizumab (Hemlibra®) within 6 months prior to screening Any other conditions or comorbidities that would make the patient unsuitable for enrollment or could interfere with participation in or completion of the study, per Investigator judgment Antithrombin (AT) activity <60% at Screening Co-existing thrombophilic disorder Clinically significant liver disease Active Hepatitis C virus infection Acute or chronic Hepatitis B virus infection Acute Hepatitis A or hepatitis E infection HIV positive with a CD4 count of <400 cells/L History of arterial or venous thromboembolism, unrelated to an indwelling venous access

Calendario

(Última actualización: 28/08/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
18/07/2019	28/07/2020	28/07/2020	30/10/2020	23/06/2021

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
31/03/2023	No aportado	No aportado	11/03/2026	04/08/2026

Promotor

Genzyme Corp. United States

450 Water Street

Contact Person

Global Regulatory Affairs

+8006331610

contact-us@sanofi.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Parc Tauli

Plaça de la Torre de lAigua, s/n 08208 Sabadell

ceic@tauli.cat

937458454-937458451

Centros

Activo (29/09/2019)

COMPLEJO ASISTENCIAL SON ESPASES (*)

Palma de Mallorca

BALEARES

Hematología

Activo (06/08/2019)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Hematología

Activo (23/07/2019)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Hematología

Medicamentos

SAR439774

SOLUTION FOR INJECTION

SUBCUTANEOUS

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

Fitusiran prophylaxis in male pediatric subjects aged 1 to less than 12 years with hemophilia A or B

State	Type of participants	Age Ranges
Finished CT	Incapable subjects of giving consent	Less than 18 , Children (2-11 years) , Infants and preschool (28 days - 23 months) , Newborn infants (0-27 days)
Gender	**Phases**	**Expected Participants**
Men	Phase II , Phase III	32
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
International multicenter	prophylaxis, treatment, safety, effectiveness, pharmacodynamics, dose	NO

Information

Identifier
2024-512501-76-00 (CTIS)

Protocol Code
EFC15467

Transitioned 2019-000679-18

Therapeutic area
Diseases [C] - Hemic and Lymphatic Diseases [C15]|

Investigated Disease
Hemophilia

Scientific Title
ATLAS-PEDS: An open-label, multinational study of fitusiran prophylaxis in pediatric subjects ages 1 to less than 12

years with hemophilia A or B

Rationale

Not provided

Main Objective

To confirm appropriate dose levels of fitusiran when administered to male pediatric participants (ages 1 to <12 years of age) with severe hemophilia A or B

Primary Endpoints

Plasma antithrombin (AT) activity levels

Temporary moments of secondary assessment

Not provided

Secondary Objective

To characterize the safety and tolerability
To determine fitusiran plasma concentrations at selected time points

Secondary Endpoints

Number of participants reported with adverse events
Fitusiran plasma concentrations

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male, aged 1 to <12 years at the time of enrollment. Severe hemophilia A or B (Factor VIII (FVIII) <1% or Factor IX (FIX) 2%) Participants must have inhibitory antibodies to FVIII or FIX and must meet one of the following Nijmegen-modified Bethesda assay results criteria: - Inhibitor titer of 0.6 BU/mL at screening, OR - Inhibitor titer of <0.6 BU/mL at screening with medical record evidence of 2 consecutive titers 0.6 BU/mL, OR - Inhibitor titer of <0.6 BU/mL at

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Exclusion criteria

Known co-existing bleeding disorders other than hemophilia A or B Inadequate renal function History of multiple drug allergies or history of allergic reaction to an oligonucleotide or N-Acetylgalactosamine (GalNAc) Subjects with central or peripheral indwelling catheters, with history of venous access complications leading to hospitalization and/or systemic anticoagulation therapy. History of intolerance to subcutaneous (SC) injection(s) Use of emicizumab (Hemlibra®) within 6 months prior to screening Any other conditions or comorbidities that would make the patient unsuitable for enrollment or could interfere with participation in or completion of the study, per Investigator judgment Antithrombin (AT) activity <60% at Screening Co-existing thrombophilic disorder Clinically significant liver disease Active Hepatitis C virus infection Acute or chronic Hepatitis B virus infection Acute Hepatitis A or hepatitis E infection HIV positive with a CD4 count of <400 cells/L History of arterial or venous thromboembolism, unrelated to an indwelling venous access

Calendar

(Last Update: 28/08/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
18/07/2019	28/07/2020	28/07/2020	30/10/2020	23/06/2021

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
31/03/2023	Not aported	Not aported	11/03/2026	04/08/2026

Sponsor

Genzyme Corp. United States

450 Water Street

Contact Person

Global Regulatory Affairs

+8006331610

contact-us@sanofi.com

Monetary support: N/A

Sponsor type: Commercial

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Sites

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Madrid

MADRID

Hematología

Medication

SAR439774

SOLUTION FOR INJECTION

SUBCUTANEOUS

-

Active Principles: N/A

Orphan **Experimental**

No results