A study to investigate the efficacy and safety of fitusiran prophylaxis in male participants aged 1 to less than 12 years with hemophilia A or B.

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Adolescentes (12-17 años) , Niños (2-11 años) , Lactantes y preescolar (28 días - 23 meses) , Recién nacidos (0-27 días)
Género	**Fases**	**Participantes esperados**
Hombres	Fase III	61
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	profilaxis, seguridad, eficacia, farmacocinética, farmacodinamica, dosis	NO

Información

Identificador
2025-521858-42-00 (CTIS)

Cod. Protocolo
EFC17905

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

Enfermedad investigada
Hemophilia

Título Científico
An open-label, parallel, Phase 3, two-arm study to investigate the efficacy and safety of fitusiran prophylaxis in male participants aged 1 to less than 12 years with hemophilia A or B with or without inhibitory antibodies to Factors VIII or

IX

Justificación

No aportado

Objetivo Principal

To evaluate treatment efficacy during fitusiran prophylaxis and SOC periods in the fitusiran- naïve arm.

Variables de Evaluación Primaria

Annualized treated bleeding rate (ABR) in the fitusiran primary efficacy period and in the SOC period.

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To characterize the following during the fitusiran prophylaxis and SOC periods in the fitusiran- naïve arm: - the frequency of treated spontaneous bleeding episodes - the frequency of treated joint bleeding episodes
To characterize the frequency of treated bleeding episodes during the fitusiran treatment period in both arms
To characterize the effect of fitusiran prophylaxis on health-related quality of life (HRQoL) outcomes in the fitusiran-naïve arm
To characterize the safety of fitusiran in both arms
To characterize the effect of fitusiran prophylaxis on joint health outcomes in the fitusiran-naïve arm

Variables de Evaluación Secundaria

Annualized spontaneous bleeding rate (AsBR) in the fitusiran primary efficacy period and in the SOC period.
Annualized joint bleeding rate (AjBR) in the fitusiran primary efficacy period and in the SOC period
ABR in the fitusiran treatment period (160 weeks) for fitusiran-naïve participants.
ABR in the fitusiran treatment period (60 weeks) for rolled-over participants
Change in physical activity
Change in pain intensity
Change in HRQoL
Incidence, severity, seriousness, and relatedness of adverse events (AEs)
Change in total score and domain scores
Target joints resolution

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Participant must be 1 to <12 years of age at the time of enrollment Participants must have severe hemophilia A or B (FVIII <1% or FIX 2%) as evidenced by a central laboratory measurement at screening or documented medical record evidence. -Participants must meet inhibitor or non-inhibitor status as defined below: Inhibitor: Requiring use of BPA for prophylaxis or BPA as on-demand therapy for any bleeding episodes for at least the last 3 months prior to screening, and 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 (eg, anaphylaxis) or nephrotic syndrome Non-inhibitor: Requiring use of clotting factor concentrates (CFCs) for prophylaxis or CFCs as on- demand therapy for any bleeding episodes for at least the last 3 months prior to screening, and meet each of the following criterion: . Nijmegen-modified Bethesda assay inhibitor titer of <0.6 BU/mL at screening, AND . No use of BPA to treat bleeding episodes for at least the last 3 months prior to screening -Participants must have adequate peripheral venous access, as determined by the Investigator, to allow the blood draws required by the study protocol. -Male: There are no contraceptive requirements for this study except where required by local regulations. -Capable of giving signed informed consent/assent. A signed written informed consent must be obtained from parent(s)/legal guardian (hereafter referred to as the parent), as well as a 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. History of intolerance to SC injection(s). Current participation in ITI therapy. -The use of emicizumab (Hemlibra®) or any non-factor bleed management treatment within 6 months prior to screening Prior gene therapy Current or future participation in another clinical study, scheduled to occur during this study, involving an investigational product other than fitusiran or an investigational device. AT activity <60% at screening, as determined by central laboratory analysis. Co-existing thrombophilic disorder. -Presence of an active Hepatitis C virus infection Presence of acute hepatitis A or Hepatitis E virus infection. Presence of acute or chronic hepatitis B virus infection. Presence of clinically significant liver disease. Platelet count 100 000/L. Presence of acute infection at screening. Human immunodeficiency virus (HIV) positive with a CD4 count of <400 cells/L. Estimated glomerular filtration rate 45 mL/min/1.73 m2 (using the Schwartz formula). History of antiphospholipid antibody syndrome. History of arterial or venous thromboembolism, unrelated to an indwelling venous access. -Any condition (eg, medical concern), which in the opinion of the Investigator, would make the participant unsuitable for dosing or which could interfere with the study compliance, the participant's safety and/or the participant's participation in the completion of the treatment period of the study. History of multiple drug allergies or history of allergic reaction to an oligonucleotide or GalNAc. -Subjects with a central or peripheral indwelling catheter, with a history of venous access complications (such as infections, thrombosis) leading to hospitalization and/or systemic anticoagulation therapy in the last 12 months. At screening, anticipated need of surgery during the study or planned surgery scheduled to occur during the study. -Completion of a surgical procedure within 14 days prior to screening, or currently receiving additional BPA infusion for postoperative hemostasis.

Calendario

(Última actualización: 09/07/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
16/01/2026	16/03/2026	11/03/2026	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

Sanofi-Aventis Recherche & Developpement France

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Contact Person

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Monetary support: N/A

Tipo de promotor: Comercial

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Centros

Activo (16/02/2026)

Hospital Sant Joan De Deu Barcelona

Esplugues De Llobregat

BARCELONA

Servicio de Hematología Pediátrica

Activo (03/02/2026)

Hospital Universitario La Paz

Madrid

MADRID

Servicio de Hematología

Activo (02/02/2026)

Hospital Universitario Miguel Servet

Zaragoza

ZARAGOZA

Servicio de Hematología

Medicamentos

SAR439774
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Principios Activos: N/A

Huérfano Experimental

SAR439774
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Principios Activos: N/A

Huérfano Experimental

Sin resultados

A study to investigate the efficacy and safety of fitusiran prophylaxis in male participants aged 1 to less than 12 years with hemophilia A or B.

State	Type of participants	Age Ranges
Recruiting	Incapable subjects of giving consent	Less than 18 , Teens (12-17 years) , Children (2-11 years) , Infants and preschool (28 days - 23 months) , Newborn infants (0-27 days)
Gender	**Phases**	**Expected Participants**
Men	Phase III	61
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	prophylaxis, safety, effectiveness, pharmacokinetics, pharmacodynamics, dose	NO

Information

Identifier
2025-521858-42-00 (CTIS)

Protocol Code
EFC17905

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

Investigated Disease
Hemophilia

Scientific Title
An open-label, parallel, Phase 3, two-arm study to investigate the efficacy and safety of fitusiran prophylaxis in male participants aged 1 to less than 12 years with hemophilia A or B with or without inhibitory antibodies to Factors VIII or

IX

Rationale

Not provided

Main Objective

To evaluate treatment efficacy during fitusiran prophylaxis and SOC periods in the fitusiran- naïve arm.

Primary Endpoints

Annualized treated bleeding rate (ABR) in the fitusiran primary efficacy period and in the SOC period.

Temporary moments of secondary assessment

Not provided

Secondary Objective

To characterize the following during the fitusiran prophylaxis and SOC periods in the fitusiran- naïve arm: - the frequency of treated spontaneous bleeding episodes - the frequency of treated joint bleeding episodes
To characterize the frequency of treated bleeding episodes during the fitusiran treatment period in both arms
To characterize the effect of fitusiran prophylaxis on health-related quality of life (HRQoL) outcomes in the fitusiran-naïve arm
To characterize the safety of fitusiran in both arms
To characterize the effect of fitusiran prophylaxis on joint health outcomes in the fitusiran-naïve arm

Secondary Endpoints

Annualized spontaneous bleeding rate (AsBR) in the fitusiran primary efficacy period and in the SOC period.
Annualized joint bleeding rate (AjBR) in the fitusiran primary efficacy period and in the SOC period
ABR in the fitusiran treatment period (160 weeks) for fitusiran-naïve participants.
ABR in the fitusiran treatment period (60 weeks) for rolled-over participants
Change in physical activity
Change in pain intensity
Change in HRQoL
Incidence, severity, seriousness, and relatedness of adverse events (AEs)
Change in total score and domain scores
Target joints resolution

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Participant must be 1 to <12 years of age at the time of enrollment Participants must have severe hemophilia A or B (FVIII <1% or FIX 2%) as evidenced by a central laboratory measurement at screening or documented medical record evidence. -Participants must meet inhibitor or non-inhibitor status as defined below: Inhibitor: Requiring use of BPA for prophylaxis or BPA as on-demand therapy for any bleeding episodes for at least the last 3 months prior to screening, and 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 (eg, anaphylaxis) or nephrotic syndrome Non-inhibitor: Requiring use of clotting factor concentrates (CFCs) for prophylaxis or CFCs as on- demand therapy for any bleeding episodes for at least the last 3 months prior to screening, and meet each of the following criterion: . Nijmegen-modified Bethesda assay inhibitor titer of <0.6 BU/mL at screening, AND . No use of BPA to treat bleeding episodes for at least the last 3 months prior to screening -Participants must have adequate peripheral venous access, as determined by the Investigator, to allow the blood draws required by the study protocol. -Male: There are no contraceptive requirements for this study except where required by local regulations. -Capable of giving signed informed consent/assent. A signed written informed consent must be obtained from parent(s)/legal guardian (hereafter referred to as the parent), as well as a written or oral assent obtained from participant, per local and national requirements.

Exclusion criteria

Known co-existing bleeding disorders other than hemophilia A or B. History of intolerance to SC injection(s). Current participation in ITI therapy. -The use of emicizumab (Hemlibra®) or any non-factor bleed management treatment within 6 months prior to screening Prior gene therapy Current or future participation in another clinical study, scheduled to occur during this study, involving an investigational product other than fitusiran or an investigational device. AT activity <60% at screening, as determined by central laboratory analysis. Co-existing thrombophilic disorder. -Presence of an active Hepatitis C virus infection Presence of acute hepatitis A or Hepatitis E virus infection. Presence of acute or chronic hepatitis B virus infection. Presence of clinically significant liver disease. Platelet count 100 000/L. Presence of acute infection at screening. Human immunodeficiency virus (HIV) positive with a CD4 count of <400 cells/L. Estimated glomerular filtration rate 45 mL/min/1.73 m2 (using the Schwartz formula). History of antiphospholipid antibody syndrome. History of arterial or venous thromboembolism, unrelated to an indwelling venous access. -Any condition (eg, medical concern), which in the opinion of the Investigator, would make the participant unsuitable for dosing or which could interfere with the study compliance, the participant's safety and/or the participant's participation in the completion of the treatment period of the study. History of multiple drug allergies or history of allergic reaction to an oligonucleotide or GalNAc. -Subjects with a central or peripheral indwelling catheter, with a history of venous access complications (such as infections, thrombosis) leading to hospitalization and/or systemic anticoagulation therapy in the last 12 months. At screening, anticipated need of surgery during the study or planned surgery scheduled to occur during the study. -Completion of a surgical procedure within 14 days prior to screening, or currently receiving additional BPA infusion for postoperative hemostasis.

Calendar

(Last Update: 09/07/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
16/01/2026	16/03/2026	11/03/2026	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

Sanofi-Aventis Recherche & Developpement France

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Contact Person

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Monetary support: N/A

Sponsor type: Commercial

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Sites

Active (16/02/2026)

Hospital Sant Joan De Deu Barcelona

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Servicio de Hematología Pediátrica

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MADRID

Servicio de Hematología

Active (02/02/2026)

Hospital Universitario Miguel Servet

Zaragoza

ZARAGOZA

Servicio de Hematología

Medication

SAR439774
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Active Principles: N/A

Orphan **Experimental**

SAR439774
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Active Principles: N/A

Orphan **Experimental**

No results