Protocol Title: Phase 3, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480 (Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C1%)

Estado	**Tipo de Participantes**	**Rangos de Edad**
Fin Reclutamiento	No aportado	Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Hombres	Fase III	58
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Unicéntrico nacional	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica, farmacogenética	Terapia génica

Información

Identificador
2024-512075-12-00 (CTIS)

Cod. Protocolo
C3731003

Transicionado 2019-004451-37

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

Enfermedad investigada
hemophilia A

Título Científico
C3731003 Phase 3, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480

(Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C1%)

Justificación

No aportado

Objetivo Principal

Evaluate the efficacy of a single infusion of PF-07055480 in participants18 and <65 years of age with moderately severe to severe hemophilia A (FVIII C 1%).

Variables de Evaluación Primaria

Total annualized bleeding rate (ABR, spontaneous and traumatic bleedings, treated and untreated) from Week 12 through at least 15 months following PF-07055480 infusion versus Total ABR on prior Factor VIII (FVIII) prophylaxis replacement regimen.

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To demonstrate that the use of exogenous FVIII is significantly reduced post PF-07055480 infusion.
To assess additional efficacy parameters post PF-07055480 infusion including FVIII activity level, use of exogenous FVIII, information on bleeding events and PROs.
Estimate the durability of efficacy up to 5 years after PF-07055480 infusion.
To estimate the safety and tolerability of PF-07055480, including immunogenicity, for the study duration of 5 years after PF-07055480 infusion.

Variables de Evaluación Secundaria

Factor VIII (FVIII) activity level >5% at 15 months following infusion of PF-07055480. ABR (spontaneous and traumatic treated bleedings) from Week 12 through at least 15 months following PF-07055480 infusion versus ABR on prior FVIII prophylaxis replacement regimen. Annualized infusion rate (AIR) of exogenous FVIII from Week 12 through at least 15 months following infusion of PF-07055480 versus AIR on prior FVIII prophylaxis replacement regimen. FVIII activity level from Week 12 through 15 months following infusion of PF-07055480. The following secondary parameters will be assessed from Week 12 through at least 15 months after PF-07055480 infusion and compared with prior FVIII prophylaxis replacement regimen: Annualized FVIII consumption. Annualized bleeding rate (ABR) of specific type: o by cause (spontaneous or traumatic) o by location (in joints, in target joints, or in soft tissue). Total ABR by cause and by location. Percentage of participants without bleeds. The following secondary parameters will be assessed by visit after PF- 07055480 infusion: FVIII activity level. Change from baseline in joint

health as measured by the HJHS instrument. Change from baseline in the following patient-reported outcome (PRO) endpoints: o Haemophilia Quality of Life Questionnaire for Adults (Haem-AQoL) o Haemophilia Activities List (HAL). The following parameters will be analysed yearly or by visit as appropriate: ABR. FVIII activity level. AIR of exogenous FVIII. Annualized FVIII consumption. ABR of specific type: o by cause (spontaneous or traumatic). o by location (in joint, in target joints, or in soft tissue). Total ABR. Total ABR by cause and by location. Percentage of participants without bleeds. The following parameters will be analysed yearly or by visit as appropriate: Change from baseline in joint health as measured by the HJHS instrument. Change from baseline in PRO endpoints: Haem-A-QoL and HAL. In addition, ABR, Total ABR, and AIR will be analyzed throughout the 5 year study period. - Incidence and severity of adverse events (AEs). - Events of special interest (such as hypersensitivity reactions, clinically reported thrombotic events, and malignancy). - Immunogenicity: Antibodies against adeno-associated virus 6 (AAV6) capsid protein (neutralizing antibodies [nAbs] and anti-drug antibodies [ADAs]). T-cell responses against AAV6 capsid and against the transgene. FVIII inhibitors.

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Males who have been followed on routine Factor VIII prophylaxis therapy in the lead-in study (C0371004) and have 150 documented exposure days to a Factor VIII protein product
Moderately severe to severe hemophilia A (Factor VIII activity 1%)
Suspension of FVIII prophylaxis therapy post study drug infusion

Criterios de Exclusión

Anti-AAV6 neutralizing antibodies History of inhibitor to Factor VIII Laboratory values at screening visit that are abnormal or outside acceptable study limits Significant and/or unstable liver disease, biliary disease, significant liver fibrosis Planned surgical procedure requiring Factor VIII surgical prophylactic factor treatment 12 months from screening visit Active hepatitis B or C Serological evidence of human immunodeficiency virus HIV-1 or HIV-2 with either Cluster of Differentiation 4 positive (CD4+) cell count 200 mm3 or viral load >20 copies/mL

Calendario

(Última actualización: 26/09/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
11/12/2020	14/06/2021	20/09/2021	21/03/2022	27/07/2022

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
31/07/2023	No aportado	No aportado	No aportado	No aportado

Promotor

Pfizer Inc. United States

66 Hudson Boulevard East

Contact Person

Clinical Medical Lead

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ClinicalTrials.gov_Inquiries@pfizer.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

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Centros

Cerrado (01/04/2025)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Servicio de Hematologia

Activo (15/06/2021)

HOSPITAL UNIVERSITARIO DEL RIO HORTEGA

Valladolid

VALLADOLID

Servicio de hematología

Medicamentos

giroctocogene fitelparvovec

SOLUTION FOR INFUSION

SOLUTION FOR INFUSION

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Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

Protocol Title: Phase 3, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480 (Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C1%)

State	Type of participants	Age Ranges
End of recruitment	Not provided	Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Men	Phase III	58
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
National One-center	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics, pharmacogenetics	Gene therapy

Information

Identifier
2024-512075-12-00 (CTIS)

Protocol Code
C3731003

Transitioned 2019-004451-37

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

Investigated Disease
hemophilia A

Scientific Title
C3731003 Phase 3, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480

(Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C1%)

Rationale

Not provided

Main Objective

Evaluate the efficacy of a single infusion of PF-07055480 in participants18 and <65 years of age with moderately severe to severe hemophilia A (FVIII C 1%).

Primary Endpoints

Total annualized bleeding rate (ABR, spontaneous and traumatic bleedings, treated and untreated) from Week 12 through at least 15 months following PF-07055480 infusion versus Total ABR on prior Factor VIII (FVIII) prophylaxis replacement regimen.

Temporary moments of secondary assessment

Not provided

Secondary Objective

To demonstrate that the use of exogenous FVIII is significantly reduced post PF-07055480 infusion.
To assess additional efficacy parameters post PF-07055480 infusion including FVIII activity level, use of exogenous FVIII, information on bleeding events and PROs.
Estimate the durability of efficacy up to 5 years after PF-07055480 infusion.
To estimate the safety and tolerability of PF-07055480, including immunogenicity, for the study duration of 5 years after PF-07055480 infusion.

Secondary Endpoints

Factor VIII (FVIII) activity level >5% at 15 months following infusion of PF-07055480. ABR (spontaneous and traumatic treated bleedings) from Week 12 through at least 15 months following PF-07055480 infusion versus ABR on prior FVIII prophylaxis replacement regimen. Annualized infusion rate (AIR) of exogenous FVIII from Week 12 through at least 15 months following infusion of PF-07055480 versus AIR on prior FVIII prophylaxis replacement regimen. FVIII activity level from Week 12 through 15 months following infusion of PF-07055480. The following secondary parameters will be assessed from Week 12 through at least 15 months after PF-07055480 infusion and compared with prior FVIII prophylaxis replacement regimen: Annualized FVIII consumption. Annualized bleeding rate (ABR) of specific type: o by cause (spontaneous or traumatic) o by location (in joints, in target joints, or in soft tissue). Total ABR by cause and by location. Percentage of participants without bleeds. The following secondary parameters will be assessed by visit after PF- 07055480 infusion: FVIII activity level. Change from baseline in joint

health as measured by the HJHS instrument. Change from baseline in the following patient-reported outcome (PRO) endpoints: o Haemophilia Quality of Life Questionnaire for Adults (Haem-AQoL) o Haemophilia Activities List (HAL). The following parameters will be analysed yearly or by visit as appropriate: ABR. FVIII activity level. AIR of exogenous FVIII. Annualized FVIII consumption. ABR of specific type: o by cause (spontaneous or traumatic). o by location (in joint, in target joints, or in soft tissue). Total ABR. Total ABR by cause and by location. Percentage of participants without bleeds. The following parameters will be analysed yearly or by visit as appropriate: Change from baseline in joint health as measured by the HJHS instrument. Change from baseline in PRO endpoints: Haem-A-QoL and HAL. In addition, ABR, Total ABR, and AIR will be analyzed throughout the 5 year study period. - Incidence and severity of adverse events (AEs). - Events of special interest (such as hypersensitivity reactions, clinically reported thrombotic events, and malignancy). - Immunogenicity: Antibodies against adeno-associated virus 6 (AAV6) capsid protein (neutralizing antibodies [nAbs] and anti-drug antibodies [ADAs]). T-cell responses against AAV6 capsid and against the transgene. FVIII inhibitors.

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Males who have been followed on routine Factor VIII prophylaxis therapy in the lead-in study (C0371004) and have 150 documented exposure days to a Factor VIII protein product
Moderately severe to severe hemophilia A (Factor VIII activity 1%)
Suspension of FVIII prophylaxis therapy post study drug infusion

Exclusion criteria

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Calendar

(Last Update: 26/09/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
11/12/2020	14/06/2021	20/09/2021	21/03/2022	27/07/2022

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
31/07/2023	Not aported	Not aported	Not aported	Not aported

Sponsor

Pfizer Inc. United States

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Contact Person

Clinical Medical Lead

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Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

Closed (01/04/2025)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Servicio de Hematologia

Active (15/06/2021)

HOSPITAL UNIVERSITARIO DEL RIO HORTEGA

Valladolid

VALLADOLID

Servicio de hematología

Medication

giroctocogene fitelparvovec

SOLUTION FOR INFUSION

SOLUTION FOR INFUSION

-

Active Principles: N/A

Orphan **Experimental**

No results