A Global, Open-label, Adaptive Design Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Severe Hemophilia A or Moderately Severe to Severe Hemophilia B (PRESent-2)

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Adultos (18-64 años) , Adolescentes (12-17 años)
Género	**Fases**	**Participantes esperados**
Hombres	Fase II	154
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Multicéntrico internacional	profilaxis, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2022-502880-39-00 (CTIS)

Cod. Protocolo
AP-0102

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

Enfermedad investigada
Severe Hemophilia A or Moderately Severe to Severe Hemophilia B

Título Científico
A Global, Open-label, Adaptive Design Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Severe Hemophilia A or Moderately Severe to Severe Hemophilia B (PRESent-2)

Justificación

Phase IIb<br/

Objetivo Principal

To evaluate the efficacy and safety of prophylactic SerpinPC administered subcutaneously (SC) in subjects with moderately severe to severe hemophilia B (HemB)

Variables de Evaluación Primaria

Study Endpoint: Treated bleeds (expressed as annualized bleeding rate [ABR]) in the observation period and under SerpinPC at the end of Part 2 Week 24 in subjects with HemB previously receiving on-demand treatment

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the tolerability of SerpinPC
To evaluate the efficacy and safety of prophylactic SerpinPC in subjects with hemophilia
To further characterize the PK profile of SerpinPC

Variables de Evaluación Secundaria

Treated bleeds (expressed as ABR) other than those defined by the primary endpoint (eg, bleeds during the first 48 weeks, and in those subjects receiving prior prophylaxis treatment)
Treated spontaneous bleeds (expressed as ABR)
Treated spontaneous joint bleeds (expressed as ABR)
All bleeds requiring treatment (expressed as ABR, ie, all treated bleeds and all bleeds that would ordinarily be treated with factor concentrate/bypass agent if therapy were available)
Total coagulation factor and/or bypass product consumption
PK concentrations of SerpinPC throughout the study
Haemophilia-specific QoL Instrument for Adults (Haem-A-QoL) Physical Health scale in subjects aged 17 to 65 years with hemophilia and the end of Part 3 Week 24).

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male subjects 12 and 65 years of age with severe HemA, with or without inhibitors, or moderately severe to severe HemB, without high titer inhibitor (high titer inhibitor defined as 5 Bethesda Units [BU]/mL) and not requiring current treatment with bypass agents.
Subject is currently included in a prophylaxis program OR subject is undergoing an on-demand treatment regimen; if the latter, must have had 6 documented acute bleeding episodes that required treatment during the 6 months before Screening
At least 12 (Part 1) or 24 (Part 2) weeks of prospective documentation of bleeding episodes in the AP-0105 noninterventional study before dosing, or willing to complete the observation period in AP-0102
No bleeding in the 7 days before Baseline (observation period can be extended if actively bleeding)
Adequate hematologic, hepatic, and renal function, and D-dimer of 750 g/L

Criterios de Exclusión

Known severe thrombophilia, previous deep vein thrombosis (excluding line-associated thrombosis), pulmonary embolism, myocardial infarction, stroke, uncontrolled hypertension (>160/100 mmHg) or has active cancer and/or requires therapy for cancer, except for basal cell carcinoma Subject with previous factor VIII or factor IX inhibitor who responded to immune tolerance induction and remains on prophylactic factor concentrate Body weight < 30 kg OR >150 kg OR body mass index >40 kg/m2 Use of emicizumab in the 24 weeks before Baseline (Day 0) / Ongoing, or planned treatment with gene therapy for hemophilia / Current or planned treatment with anticoagulant or antiplatelet drugs

Calendario

(Última actualización: 20/03/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
25/07/2023	14/11/2023	23/11/2023	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

Apcintex Limited United Kingdom

3rd Floor1 Ashley Road

Contact Person

Chief Medical Officer

+442039206789

info@apcintex.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Regional de la Comunidad de Madrid

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Centros

Cerrado (13/12/2024)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Haemostasis

Cerrado (19/03/2025)

Hospital Universitario La Paz

Madrid

MADRID

Haemostasis

Cerrado (12/02/2025)

Hospital Universitario Regional De Malaga

Malaga

MÁLAGA

Haemostasis

Cerrado (03/02/2025)

Hospital Unviersitario Miguel Servet

Zaragoza

ZARAGOZA

Haemostasis

Cerrado (12/03/2025)

University Clinical Hospital Virgen De La Arrixaca

Murcia

MURCIA

Haemostasis

Medicamentos

SerpinPC
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Principios Activos: N/A

Huérfano Experimental

Sin resultados

A Global, Open-label, Adaptive Design Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Severe Hemophilia A or Moderately Severe to Severe Hemophilia B (PRESent-2)

State	Type of participants	Age Ranges
Recruiting	Incapable subjects of giving consent	Less than 18 , Adults (18-64 years) , Teens (12-17 years)
Gender	**Phases**	**Expected Participants**
Men	Phase II	154
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
International multicenter	prophylaxis, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2022-502880-39-00 (CTIS)

Protocol Code
AP-0102

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

Investigated Disease
Severe Hemophilia A or Moderately Severe to Severe Hemophilia B

Scientific Title
A Global, Open-label, Adaptive Design Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Severe Hemophilia A or Moderately Severe to Severe Hemophilia B (PRESent-2)

Rationale

Phase IIb<br/

Main Objective

To evaluate the efficacy and safety of prophylactic SerpinPC administered subcutaneously (SC) in subjects with moderately severe to severe hemophilia B (HemB)

Primary Endpoints

Study Endpoint: Treated bleeds (expressed as annualized bleeding rate [ABR]) in the observation period and under SerpinPC at the end of Part 2 Week 24 in subjects with HemB previously receiving on-demand treatment

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the tolerability of SerpinPC
To evaluate the efficacy and safety of prophylactic SerpinPC in subjects with hemophilia
To further characterize the PK profile of SerpinPC

Secondary Endpoints

Treated bleeds (expressed as ABR) other than those defined by the primary endpoint (eg, bleeds during the first 48 weeks, and in those subjects receiving prior prophylaxis treatment)
Treated spontaneous bleeds (expressed as ABR)
Treated spontaneous joint bleeds (expressed as ABR)
All bleeds requiring treatment (expressed as ABR, ie, all treated bleeds and all bleeds that would ordinarily be treated with factor concentrate/bypass agent if therapy were available)
Total coagulation factor and/or bypass product consumption
PK concentrations of SerpinPC throughout the study
Haemophilia-specific QoL Instrument for Adults (Haem-A-QoL) Physical Health scale in subjects aged 17 to 65 years with hemophilia and the end of Part 3 Week 24).

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male subjects 12 and 65 years of age with severe HemA, with or without inhibitors, or moderately severe to severe HemB, without high titer inhibitor (high titer inhibitor defined as 5 Bethesda Units [BU]/mL) and not requiring current treatment with bypass agents.
Subject is currently included in a prophylaxis program OR subject is undergoing an on-demand treatment regimen; if the latter, must have had 6 documented acute bleeding episodes that required treatment during the 6 months before Screening
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Calendar

(Last Update: 20/03/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
25/07/2023	14/11/2023	23/11/2023	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

Apcintex Limited United Kingdom

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Contact Person

Chief Medical Officer

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info@apcintex.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

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Malaga

MÁLAGA

Haemostasis

Closed (03/02/2025)

Hospital Unviersitario Miguel Servet

Zaragoza

ZARAGOZA

Haemostasis

Closed (12/03/2025)

University Clinical Hospital Virgen De La Arrixaca

Murcia

MURCIA

Haemostasis

Medication

SerpinPC
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Active Principles: N/A

Orphan Experimental

No results