A Global, Open-label Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Hemophilia B With Inhibitors (PRESent-3)

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	20
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Unicéntrico nacional, Multicéntrico internacional	profilaxis, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2022-502881-25-00 (CTIS)

Cod. Protocolo
AP-0103

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

Enfermedad investigada
Hemophilia B

Título Científico
A Global, Open-label Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Hemophilia B With Inhibitors (PRESent-3)

Justificación

Phase II a/b<br/

Objetivo Principal

To evaluate the efficacy and safety of prophylactic SerpinPC administered subcutaneously in subjects with hemophilia B (HemB) with inhibitors

Variables de Evaluación Primaria

Study Endpoint: Treated bleeds (expressed as annualized bleeding rate [ABR]) in the observation period and during the first 24 weeks under SerpinPC

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the tolerability of SerpinPC
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, during the first 48 weeks)
Treated spontaneous bleeds (expressed as ABR)
Treated spontaneous joint bleeds (expressed as ABR)
All bleeds requiring treatment (expressed as ABR; ie, all bleeds that would ordinarily be treated with factor concentrate/bypass agent if therapy were available)
Total coagulation factor and/or bypass product consumption during SerpinPC treatment
PK concentrations of SerpinPC throughout the study
Haemophilia Quality-of-Life Questionnaire for Adults (Haem-A-QoL) Physical Health scale in subjects aged 17 to 65 years

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male subjects 12 and 65 years of age at the time of informed consent
Adequate hematologic function, defined as a platelet count of 100,000/L (100 × 109/L) and hemoglobin level of 10 g/dL (100 g/L or 6.206 mmol/L) at Screening and Pre-dosing visits
Adequate hepatic function, defined as a total bilirubin level of 1.5 × upper level of normal (ULN, excluding Gilbert syndrome) and aspartate aminotransferase and/or alanine aminotransferase of 3 × ULN at Screening and Pre-dosing visits; no clinical signs or known laboratory or radiographic evidence consistent with cirrhosis of the liver
Adequate renal function, defined as a serum creatinine level of 2.0 × ULN at Screening and Pre-dosing visits
Able to use a diary to document bleeding events and medication usage (caregiver assistance allowed for adolescents)
Sexually active subjects with a partner who could become pregnant should agree to use effective contraception for the duration of the study Effective contraceptive measures include condom with or without spermicide, a combination of male condom with either cap, diaphragm, or sponge with spermicide (double barrier methods), vasectomy, partner using stable contraceptive measures (combined [estrogen and progestogen-containing] hormonal contraception or progestogen-only hormonal contraception initiated 2 or more menstrual cycles prior to screening, intrauterine device [IUD], intrauterine hormone-releasing system [IUS], bilateral tubal ligation), and/or sexual abstinence
Capable of providing written informed consent (adolescent assent and parental/guardian/legal representative consent when appropriate) for participation and having the opportunity to discuss the study with the Investigator or delegate
Historically documented HemB (defined as factor IX 0.05 IU/mL [5%])
Subjects who are currently in a prophylaxis program must be willing to stop prophylaxis (including episodic prophylaxis for sporting events) before the first dose of SerpinPC
Historical or ongoing factor IX inhibitor requiring current treatment with bypass agents based on medical records or laboratory reports
Documented ABR of 6 in the 12 months before screening (subjects not on prophylaxis regimen) or documented ABR of 2 for subjects on prophylaxis regimen
At least 12 weeks of prospective documentation of bleeding episodes in the AP-0105 non-interventional study before SerpinPC dosing, or willing to complete a 12-week observational period (at minimum) in AP-0103
No bleeding in the 7 days before Baseline (the prospective observation period can be extended if there is an ongoing active bleed)
D-dimer of 750 g/L; in cases where there is a resolving bleed, the exclusion threshold is 1750 mg/L at Screening and Pre-dosing visits

Criterios de Exclusión

Known severe thrombophilia (defined as antithrombin deficiency and/or protein S deficiency and/or protein C deficiency) Any major medical, psychological, or psychiatric condition that could cause the subject to be unsuitable for the study or could interfere with the interpretation of the study results History of or other evidence of recent alcohol or drug abuse as determined by the Investigator (in the 12 months before screening) Known HIV infection with CD4 count (or T-cell count) of <200 cells/L within 24 weeks before Screening and Pre-dosing visits. Patients with HIV infection who have CD4 > 200 and meet all other criteria are eligible Current or planned treatment with anticoagulant or antiplatelet drugs Is planning to donate/bank sperm during SerpinPC treatment AND within 30 days of last dose of SerpinPC Any other significant conditions or comorbidities that, in the opinion of the Investigator, would make the subject unsuitable for enrollment, or could interfere with participation in, or completion of the study Patient with previous factor IX inhibitor who responded to immune tolerance induction and remains on prophylactic factor concentrate Previous deep vein thrombosis (excluding line-associated thrombosis), pulmonary embolism, myocardial infarction, or stroke History of intolerance to SC injections Uncontrolled hypertension (systolic blood pressure >160 mm Hg; diastolic blood pressure >100 mm Hg) Weight >150 kg OR body mass index >40 kg/m2 Has active cancer and/or requires therapy for cancer, except for basal cell carcinoma Participation in another interventional clinical trial (except for AP-0105) during the 30 days before screening Ongoing, or planned treatment with gene therapy for HemB

Calendario

(Última actualización: 25/11/2024)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
08/08/2023	16/11/2023	No aportado	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

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Contact Person

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Monetary support: N/A

Tipo de promotor: Comercial

Ceim

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Centros

Cerrado (21/11/2024)

Hospital Universitario La Paz

Madrid

MADRID

Haemostasis

Medicamentos

SerpinPC

SOLUTION FOR INJECTION

SUBCUTANEOUS INJECTION

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

A Global, Open-label Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Hemophilia B With Inhibitors (PRESent-3)

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	20
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
National One-center , International multicenter	prophylaxis, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2022-502881-25-00 (CTIS)

Protocol Code
AP-0103

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

Investigated Disease
Hemophilia B

Scientific Title
A Global, Open-label Study to Investigate the Efficacy and Safety of SerpinPC in Subjects With Hemophilia B With Inhibitors (PRESent-3)

Rationale

Phase II a/b<br/

Main Objective

To evaluate the efficacy and safety of prophylactic SerpinPC administered subcutaneously in subjects with hemophilia B (HemB) with inhibitors

Primary Endpoints

Study Endpoint: Treated bleeds (expressed as annualized bleeding rate [ABR]) in the observation period and during the first 24 weeks under SerpinPC

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the tolerability of SerpinPC
To further characterize the PK profile of SerpinPC

Secondary Endpoints

Treated bleeds (expressed as ABR) other than those defined by the primary endpoint (eg, during the first 48 weeks)
Treated spontaneous bleeds (expressed as ABR)
Treated spontaneous joint bleeds (expressed as ABR)
All bleeds requiring treatment (expressed as ABR; ie, all bleeds that would ordinarily be treated with factor concentrate/bypass agent if therapy were available)
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PK concentrations of SerpinPC throughout the study
Haemophilia Quality-of-Life Questionnaire for Adults (Haem-A-QoL) Physical Health scale in subjects aged 17 to 65 years

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male subjects 12 and 65 years of age at the time of informed consent
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D-dimer of 750 g/L; in cases where there is a resolving bleed, the exclusion threshold is 1750 mg/L at Screening and Pre-dosing visits

Exclusion criteria

Known severe thrombophilia (defined as antithrombin deficiency and/or protein S deficiency and/or protein C deficiency) Any major medical, psychological, or psychiatric condition that could cause the subject to be unsuitable for the study or could interfere with the interpretation of the study results History of or other evidence of recent alcohol or drug abuse as determined by the Investigator (in the 12 months before screening) Known HIV infection with CD4 count (or T-cell count) of <200 cells/L within 24 weeks before Screening and Pre-dosing visits. Patients with HIV infection who have CD4 > 200 and meet all other criteria are eligible Current or planned treatment with anticoagulant or antiplatelet drugs Is planning to donate/bank sperm during SerpinPC treatment AND within 30 days of last dose of SerpinPC Any other significant conditions or comorbidities that, in the opinion of the Investigator, would make the subject unsuitable for enrollment, or could interfere with participation in, or completion of the study Patient with previous factor IX inhibitor who responded to immune tolerance induction and remains on prophylactic factor concentrate Previous deep vein thrombosis (excluding line-associated thrombosis), pulmonary embolism, myocardial infarction, or stroke History of intolerance to SC injections Uncontrolled hypertension (systolic blood pressure >160 mm Hg; diastolic blood pressure >100 mm Hg) Weight >150 kg OR body mass index >40 kg/m2 Has active cancer and/or requires therapy for cancer, except for basal cell carcinoma Participation in another interventional clinical trial (except for AP-0105) during the 30 days before screening Ongoing, or planned treatment with gene therapy for HemB

Calendar

(Last Update: 25/11/2024)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
08/08/2023	16/11/2023	Not aported	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

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Monetary support: N/A

Sponsor type: Commercial

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Sites

Closed (21/11/2024)

Hospital Universitario La Paz

Madrid

MADRID

Haemostasis

Medication

SerpinPC
SOLUTION FOR INJECTION
SUBCUTANEOUS INJECTION

-

Active Principles: N/A

Orphan **Experimental**

No results