Caplacizumab and immunosuppressive therapy without first-line therapeutic plasma exchange in adults with immune-mediated thrombotic thrombocytopenic purpura

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	73
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Tiene resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Multicéntrico internacional	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador

2024-513262-19-00 (CTIS)

Cod. Protocolo

EFC16521

Transicionado 2022-001177-31

Área terapéutica

Enfermedades [C] - Hematología [C15]|

Enfermedad investigada

Thrombotic Thrombocytopenic Purpura

Título Científico

An open-label, single-arm, multicenter study to evaluate the efficacy and safety of caplacizumab and immunosuppressive therapy without first-line therapeutic plasma exchange in adults with immunemediated thrombotic thrombocytopenic purpura

Justificación

No aportado

Objetivo Principal

To evaluate the efficacy of caplacizumab in combination with immunosuppressive therapy (IST) without therapeutic plasma exchange (TPE) in adults with immune mediated thrombotic thrombocytopenic purpura (iTTP)

Variables de Evaluación Primaria

Proportion of participants achieving Remission without requiring therapeutic plasma exchange (TPE).

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the need for therapeutic plasma exchange in adult participants with an episode of iTTP treated with caplacizumab and IST.
To evaluate the safety of caplacizumab in combination with IST without first-line TPE in adults with iTTP
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinical response
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on restoring platelet counts
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on refractory disease
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinically relevant iTTP-related events consisting of iTTP-related mortality
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinically relevant iTTP-related events consisting of exacerbation of iTTP
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinically relevant iTTP-related events consisting of relapse of iTTP

Variables de Evaluación Secundaria

Proportion of participants achieving Remission
Proportion of participants who require TPE
The occurrence of adverse events (AEs), serious adverse events (SAEs), and adverse events of special interest (AESIs)
Proportion of participants achieving Clinical Response (On-treatment period from day 1 to day 84)
Proportion of participants achieving Clinical Response (Overall study period from day 1 to day 168)
Time to platelet count response
Proportion of participants refractory to therapy
Proportion of participants with TTP-related death (On-treatment period from day 1 to day 84)
Proportion of participants with TTP-related death (Overall study period from day 1 to day 168)

Proportion of participants with a clinical exacerbation of iTTP (On-treatment period from day 1 to day 84)
Proportion of participants with a clinical exacerbation of iTTP (Overall study period from day 1 to day 168)
Proportion of participants with a clinical relapse of iTTP (On-treatment period from day 1 to day 84)
Proportion of participants with a clinical relapse of iTTP (Overall study period from day 1 to day 168)

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

-Participants with a clinical diagnosis of iTTP (initial or recurrent), which includes thrombocytopenia, microangiopathic hemolytic anemia (eg, presence of schistocytes in peripheral blood smear) and relatively preserved renal function. The iTTP diagnosis should be confirmed by ADAMTS13 testing within 48 hours (2 days).
-Participants with a clinical diagnosis of iTTP and a French TMA score of 1 or 2.
-A female participant is eligible to participate if she is not pregnant or breastfeeding, and one of the following conditions applies: Is a woman of nonchildbearing potential (WONCBP), OR Is a woman of childbearing potential (WOCBP) and agrees to use an acceptable contraceptive method during the overall treatment period and for at least 2 months after the last study drug administration.
-Male participants with female partners of childbearing potential must agree to follow the contraceptive guidance as per protocol during the overall treatment period and for at least 2 months after last study drug administration.

Criterios de Exclusión

Participants are excluded from the study if any of the following criteria apply: -Platelet count 100x10^9/L. -Clinical condition other than that associated with TTP, with life expectancy <6 months, such as end-stage malignancy -Known chronic treatment with anticoagulants and anti-platelet drugs that cannot be stopped (interrupted) safely, including but not limited to: vitamin K antagonists direct-acting oral anticoagulants heparin or low molecular weight heparin (LMWH) non-steroidal anti-inflammatory molecules other than acetyl salicylic acid -Participants who were previously enrolled in this clinical study (study EFC16521). -Participants who received an investigational drug, or device, other than caplacizumab, within 30 days of anticipated IMP administration or 5 half-lives of the previous investigational drug, whichever is longer. -Positive result on COVID test. -Serum creatinine level >2.26 mg/dL (200 mol/L) in case platelet count is > 30x10^9/L (to exclude possible cases of atypical HUS) -Known other causes of thrombocytopenia including but not limited to: Clinical evidence of enteric infection with E. coli 0157 or related organism Atypical HUS Hematopoietic stem cell, bone marrow or solid organ transplantation-associated thrombotic microangiopathy Known or suspected sepsis Diagnosis of disseminated intravascular coagulation -Congenital TTP (known at the time of study entry) -Clinically significant active bleeding or known co-morbidities associated with high risk of bleeding (excluding thrombocytopenia) -Inherited or acquired coagulation disorders -Malignant arterial hypertension -Participants requiring or expected to require invasive procedures immediately (eg, stroke requiring thrombolytic therapy, those who need mechanical ventilation, etc.) -Those presenting with severe neurological or cardiac disease

Calendario

(Última actualización: 09/04/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
21/10/2022	16/02/2023	No aportado	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
28/06/2024	No aportado	No aportado	29/05/2024	26/12/2024

Promotor

Sanofi-Aventis Recherche & Developpement France

1 Avenue Pierre Brossolette

Contact Person

Clinical Sciences and Operations

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contact-us@sanofi.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital General Universitario Gregorio Marañón

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915867007

Centros

Cerrado (02/09/2024)

HOSPITAL CLINIC DE BARCELONA

Barcelona

BARCELONA

Servicio de Hematología

Cerrado (25/03/2025)

HOSPITAL GENERAL UNIVERSITARIO GREGORIO MARAÑON

Madrid

MADRID

Servicio de Hematología

Cerrado (18/09/2024)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Servicio de Hematología

Cerrado (17/09/2024)

HOSPITAL UNIVERSITARIO Y POLITECNICO LA FE

València

VALENCIA

Servicio de Hematología

Medicamentos

Cablivi 10 mg powder and solvent for solution for injection

SOLUTION FOR INJECTION

INTRAVENOUS (IV) OR SUBCUTANEOUS (SC)

Código ATC: B01AX07 - CAPLACIZUMAB

Principios Activos: N/A

Huérfano **Experimental**

Cablivi 10 mg powder and solvent for solution for injection

SOLUTION FOR INJECTION

INTRAVENOUS (IV) OR SUBCUTANEOUS (SC)

Código ATC: B01AX07 - CAPLACIZUMAB

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Principios Activos: N/A

Huérfano **Experimental**

Tiene resultados

Caplacizumab and immunosuppressive therapy without first-line therapeutic plasma exchange in adults with immune-mediated thrombotic thrombocytopenic purpura

State	Type of participants	Age Ranges
Finished CT	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	73
Results	**Low level of intervention**	**Rare disease**
Has results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
International multicenter	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2024-513262-19-00 (CTIS)

Protocol Code
EFC16521

Transitioned 2022-001177-31

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

Investigated Disease
Thrombotic Thrombocytopenic Purpura

Scientific Title
An open-label, single-arm, multicenter study to evaluate the efficacy and safety of caplacizumab and immunosuppressive therapy without first-line therapeutic plasma exchange in adults with immunemediated thrombotic thrombocytopenic purpura

Rationale

Not provided

Main Objective

To evaluate the efficacy of caplacizumab in combination with immunosuppressive therapy (IST) without therapeutic plasma exchange (TPE) in adults with immune mediated thrombotic thrombocytopenic purpura (iTTP)

Primary Endpoints

Proportion of participants achieving Remission without requiring therapeutic plasma exchange (TPE).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the need for therapeutic plasma exchange in adult participants with an episode of iTTP treated with caplacizumab and IST.
To evaluate the safety of caplacizumab in combination with IST without first-line TPE in adults with iTTP
To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinical response
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To evaluate the effect of treatment with caplacizumab and IST without first-line TPE on clinically relevant iTTP-related events consisting of relapse of iTTP

Secondary Endpoints

Proportion of participants achieving Remission
Proportion of participants who require TPE
The occurrence of adverse events (AEs), serious adverse events (SAEs), and adverse events of special interest (AESIs)
Proportion of participants achieving Clinical Response (On-treatment period from day 1 to day 84)
Proportion of participants achieving Clinical Response (Overall study period from day 1 to day 168)
Time to platelet count response
Proportion of participants refractory to therapy
Proportion of participants with TTP-related death (On-treatment period from day 1 to day 84)
Proportion of participants with TTP-related death (Overall study period from day 1 to day 168)

Proportion of participants with a clinical exacerbation of iTTP (On-treatment period from day 1 to day 84)
Proportion of participants with a clinical exacerbation of iTTP (Overall study period from day 1 to day 168)
Proportion of participants with a clinical relapse of iTTP (On-treatment period from day 1 to day 84)
Proportion of participants with a clinical relapse of iTTP (Overall study period from day 1 to day 168)

Temporary moments of secondary assessment

Not provided

Inclusion criteria

-Participants with a clinical diagnosis of iTTP (initial or recurrent), which includes thrombocytopenia, microangiopathic hemolytic anemia (eg, presence of schistocytes in peripheral blood smear) and relatively preserved renal function. The iTTP diagnosis should be confirmed by ADAMTS13 testing within 48 hours (2 days).
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-Male participants with female partners of childbearing potential must agree to follow the contraceptive guidance as per protocol during the overall treatment period and for at least 2 months after last study drug administration.

Exclusion criteria

Participants are excluded from the study if any of the following criteria apply: -Platelet count 100x10^9/L. -Clinical condition other than that associated with TTP, with life expectancy <6 months, such as end-stage malignancy -Known chronic treatment with anticoagulants and anti-platelet drugs that cannot be stopped (interrupted) safely, including but not limited to: vitamin K antagonists direct-acting oral anticoagulants heparin or low molecular weight heparin (LMWH) non-steroidal anti-inflammatory molecules other than acetyl salicylic acid -Participants who were previously enrolled in this clinical study (study EFC16521). -Participants who received an investigational drug, or device, other than caplacizumab, within 30 days of anticipated IMP administration or 5 half-lives of the previous investigational drug, whichever is longer. -Positive result on COVID test. -Serum creatinine level >2.26 mg/dL (200 mol/L) in case platelet count is > 30x10^9/L (to exclude possible cases of atypical HUS) -Known other causes of thrombocytopenia including but not limited to: Clinical evidence of enteric infection with E. coli 0157 or related organism Atypical HUS Hematopoietic stem cell, bone marrow or solid organ transplantation-associated thrombotic microangiopathy Known or suspected sepsis Diagnosis of disseminated intravascular coagulation -Congenital TTP (known at the time of study entry) -Clinically significant active bleeding or known co-morbidities associated with high risk of bleeding (excluding thrombocytopenia) -Inherited or acquired coagulation disorders -Malignant arterial hypertension -Participants requiring or expected to require invasive procedures immediately (eg, stroke requiring thrombolytic therapy, those who need mechanical ventilation, etc.) -Those presenting with severe neurological or cardiac disease

Calendar

(Last Update: 09/04/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
21/10/2022	16/02/2023	Not aported	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
28/06/2024	Not aported	Not aported	29/05/2024	26/12/2024

Sponsor

Sanofi-Aventis Recherche & Developpement France

1 Avenue Pierre Brossolette

Contact Person

Clinical Sciences and Operations

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Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

Closed (02/09/2024)

HOSPITAL CLINIC DE BARCELONA

Barcelona

BARCELONA

Servicio de Hematología

Closed (25/03/2025)

HOSPITAL GENERAL UNIVERSITARIO GREGORIO MARAÑON

Madrid

MADRID

Servicio de Hematología

Closed (18/09/2024)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Servicio de Hematología

Closed (17/09/2024)

HOSPITAL UNIVERSITARIO Y POLITECNICO LA FE

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ATC code: B01AX07 - CAPLACIZUMAB

Active Principles: N/A

Orphan Experimental

Has results