A study to assess the Efficacy and Safety of Efgartigimod IV in Adult Participants with Primary Immune Thrombocytopenia

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	17
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2024-515451-38-00 (CTIS)

Cod. Protocolo
ARGX-113-2402

Área terapéutica
Enfermedades [C] - Patologías del sistema inmunitario [C20]|

Enfermedad investigada
Primary Immune thrombocytopenia (ITP)

Título Científico
A Phase 3, Multicenter, Randomized, Double-Blinded, Placebo-Controlled, Parallel-Arm Study Followed by an Open-Label Arm to Evaluate the Efficacy and Safety of Efgartigimod IV in Adult Participants With Primary Immune Thrombocytopenia

Justificación

No aportado

Objetivo Principal

To evaluate the efficacy of efgartigimod IV compared with placebo IV in the extent of disease control

Variables de Evaluación Primaria

Extent of disease control, defined as the number of cumulative weeks during the Double-Blinded Treatment Period with platelet counts of at least 50×10^9/L.

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the efficacy of efgartigimod IV compared with placebo IV in achieving sustained platelet count response
To evaluate the efficacy of efgartigimod IV compared with placebo IV in overall platelet count response
To evaluate the incidence and severity of bleeding in participants treated with efgartigimod IV compared with participants treated with placebo IV
To evaluate the use of rescue ITP therapy in participants treated with efgartigimod IV compared with participants treated with placebo IV
To evaluate the long-term efficacy of efgartigimod IV (including the OLTP1)
To evaluate the tolerability and safety of efgartigimod IV
To assess the immunogenicity of efgartigimod IV
To assess the pharmacokinetics (PK) and pharmacodynamics (PD) of efgartigimod IV

Variables de Evaluación Secundaria

Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 4 of the 6 study visits between study weeks 19 and 24 of the DBTP Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 6 of the 8 study visits between study weeks 17 and 24 of the DBTP Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 8 of the 12 study visits between weeks 13 and 24 of the DBTP Proportion of participants achieving a platelet count of at least 50×10^9/L on at least 4 occasions at any time until study week 12 during the DBTP Time to response, defined as the time to achieve 2 consecutive platelet counts of at least 50×10^9/L at any time during the DBTP Proportion of participants with an International Working Group (IWG) response during the DBTP Proportion of participants with International Working Group (IWG) complete response during the DBTP Proportion of participants with initial response, defined as a platelet count of at least 30×10^9/L and a 2-fold increase from baseline in platelet count at study week 5 of the DBTP Time to achieve a platelet count of at least 30×10^9/L and a 2-fold increase from baseline in platelet count during the DBTP Number of cumulative weeks during the DBTP with platelet counts of at least 30×10^9/L and 20×10^9/L above baseline Number of cumulative

weeks during the DBTP with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline in participants with baseline platelet counts of <15×10^9/L Extent of disease control, defined as the percentage of weeks with platelet counts of at least 50×10^9/L In participants receiving placebo IV in the DBTP, the extent of disease control, defined as the number of cumulative weeks with platelet counts of at least 50×10^9/L, between weeks 13 and 24 of the OLTP1 Overall platelet count response, defined as the proportion of participants achieving a platelet count of at least 50×10^9/L on at least 4 occasions during the first 52 weeks of treatment with efgartigimod IV. Mean change from baseline in platelet count at each study visit The percentage of weeks with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline In participants with baseline platelet counts <15×10^9/L, the percentage of weeks with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline Proportion of participants who achieve a sustained platelet count response, defined as achieving platelet counts of at least 50×10^9/L for at least 4 occasions during 6-week intervals. In participants receiving placebo IV in the DBTP, the proportion of participants achieving platelet counts of at least 50×10^9/L for at least 8 of the 12 study visits between weeks 13 and 24 of the OLTP1 In participants receiving placebo IV in the DBTP, the proportion of participants who achieve a sustained platelet count response, defined as achieving platelet counts of at least 50×10^9/L for at least 6 of the 8 study visits between weeks 17 and 24 of the OLTP1 In participants receiving placebo IV in the DBTP, the extent of disease control, defined as the number of cumulative weeks with platelet counts of at least 50×10^9/L during the first 24 weeks of treatment with efgartigimod IV Occurrence rate of rescue ITP therapy Proportion of participants with reduction in concurrent ITP therapy during the OLTP1 Incidence and severity of bleeding, assessed by the ITP Bleeding Scale (IBLS) Incidence and severity of adverse events (AEs) (including AEs of clinical interest) and serious AEs. Vital signs, laboratory safety measurements, physical examination Incidence and prevalence of antidrug antibodies (ADA) against efgartigimod in serum over time Incidence and prevalence of NAb against efgartigimod in serum over time Efgartigimod Cmax and Ctrough over time Percent change from baseline in total IgG levels in serum over time

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Is at least 18 years of age and the local legal age of consent for clinical studies when signing the informed consent form (ICF). Has documented baseline mean platelet count of <30 x 10^9/L before randomization Has a documented duration of primary immune thrombocytopenia (ITP) of more than 12 months on the date of informed consent form (ICF) signature Has documented prior ITP treatment with at least 1 of the following treatments: corticosteroids, intravenous immunoglobulin (IVIg), anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive), thrombopoietin receptor agonist (TPO-RAs), or rituximab Has documented insufficient response to a prior ITP treatment with corticosteroids, IVIg, anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive), TPO-RAs, rituximab (the specific criteria can be found in the protocol). Has documented prior response defined as 1 platelet count of 50×10^9/L to at least 1 of the following ITP treatments in the 3 years before the date of ICF signature: prednisone, dexamethasone, other or nonspecified corticosteroids, IVIg, or anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive).

Criterios de Exclusión

Other than the indication under study, known autoimmune disease or any medical condition that would interfere with an accurate assessment of clinical symptoms of ITP, confound the results of the study or put the participant at undue risk.
Secondary ITP
Nonimmune thrombocytopenia

Autoimmune hemolytic anemia
ITP-associated critical or severe bleeding

Calendario

(Última actualización: 13/08/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
24/01/2025	27/02/2025	03/04/2025	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

Argenx Belgium

Industriepark-Zwijnaarde 7

Contact Person

Vice President Clinical Development

+3293103400

ClinicalTrials@argenx.com

Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Parc de Salut Mar

Doctor Aiguader, 88 Edificio PRBB 08003 Barcelona

ceic-psmar@researchmar.net

933160679

Centros

Activo (27/03/2025)

Complexo Hospitalario Universitario A Coruna

A Coruna

CORUÑA

Hematology

Activo (28/02/2025)

Hospital Clinico Universitario De Valencia

Valencia

VALENCIA

Hematology

Activo (27/02/2025)

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology

No iniciado (--/--/----)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Activo (20/03/2025)

Hospital Universitario Quironsalud Madrid

Pozuelo De Alarcon

MADRID

Hematology

Activo (19/03/2025)

Hospital Universitario Ramon Y Cajal

Madrid

MADRID

Hematology

No iniciado (--/--/----)

Institut Catala D´oncologia

L'hospitalet De Llobregat

BARCELONA

Hematology

Medicamentos

Vyvgart 20 mg/mL concentrate for solution for infusion
SOLUTION FOR INFUSION
INTRAVENOUS USE

Código ATC: L04AA58 - EFGARTIGIMOD ALFA
Principios Activos: N/A

Huérfano Experimental

ARGX-113
SOLUTION FOR INFUSION
INTRAVENOUS USE

-

Principios Activos: N/A

Huérfano Experimental

Sin resultados

A study to assess the Efficacy and Safety of Efgartigimod IV in Adult Participants with Primary Immune Thrombocytopenia

State	Type of participants	Age Ranges
Recruiting	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	17
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2024-515451-38-00 (CTIS)

Protocol Code
ARGX-113-2402

Therapeutic area
Diseases [C] - Immune System Diseases [C20]|

Investigated Disease
Primary Immune thrombocytopenia (ITP)

Scientific Title
A Phase 3, Multicenter, Randomized, Double-Blinded, Placebo-Controlled, Parallel-Arm Study Followed by an Open-Label Arm to Evaluate the Efficacy and Safety of Efgartigimod IV in Adult Participants With Primary Immune Thrombocytopenia

Rationale

Not provided

Main Objective

To evaluate the efficacy of efgartigimod IV compared with placebo IV in the extent of disease control

Primary Endpoints

Extent of disease control, defined as the number of cumulative weeks during the Double-Blinded Treatment Period with platelet counts of at least 50×10^9/L.

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the efficacy of efgartigimod IV compared with placebo IV in achieving sustained platelet count response
To evaluate the efficacy of efgartigimod IV compared with placebo IV in overall platelet count response
To evaluate the incidence and severity of bleeding in participants treated with efgartigimod IV compared with participants treated with placebo IV
To evaluate the use of rescue ITP therapy in participants treated with efgartigimod IV compared with participants treated with placebo IV
To evaluate the long-term efficacy of efgartigimod IV (including the OLTP1)
To evaluate the tolerability and safety of efgartigimod IV
To assess the immunogenicity of efgartigimod IV
To assess the pharmacokinetics (PK) and pharmacodynamics (PD) of efgartigimod IV

Secondary Endpoints

Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 4 of the 6 study visits between study weeks 19 and 24 of the DBTP Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 6 of the 8 study visits between study weeks 17 and 24 of the DBTP Proportion of participants achieving platelet counts of at least 50×10^9/L for at least 8 of the 12 study visits between weeks 13 and 24 of the DBTP Proportion of participants achieving a platelet count of at least 50×10^9/L on at least 4 occasions at any time until study week 12 during the DBTP Time to response, defined as the time to achieve 2 consecutive platelet counts of at least 50×10^9/L at any time during the DBTP Proportion of participants with an International Working Group (IWG) response during the DBTP Proportion of participants with International Working Group (IWG) complete response during the DBTP Proportion of participants with initial response, defined as a platelet count of at least 30×10^9/L and a 2-fold increase from baseline in platelet count at study week 5 of the DBTP Time to achieve a platelet count of at least 30×10^9/L and a 2-fold increase from baseline in platelet count during the DBTP Number of cumulative weeks during the DBTP with platelet counts of at least 30×10^9/L and 20×10^9/L above baseline Number of cumulative

weeks during the DBTP with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline in participants with baseline platelet counts of <15×10^9/L Extent of disease control, defined as the percentage of weeks with platelet counts of at least 50×10^9/L In participants receiving placebo IV in the DBTP, the extent of disease control, defined as the number of cumulative weeks with platelet counts of at least 50×10^9/L, between weeks 13 and 24 of the OLTP1 Overall platelet count response, defined as the proportion of participants achieving a platelet count of at least 50×10^9/L on at least 4 occasions during the first 52 weeks of treatment with efgartigimod IV. Mean change from baseline in platelet count at each study visit The percentage of weeks with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline In participants with baseline platelet counts <15×10^9/L, the percentage of weeks with platelet counts of at least 30×10^9/L and at least 20×10^9/L above baseline Proportion of participants who achieve a sustained platelet count response, defined as achieving platelet counts of at least 50×10^9/L for at least 4 occasions during 6-week intervals. In participants receiving placebo IV in the DBTP, the proportion of participants achieving platelet counts of at least 50×10^9/L for at least 8 of the 12 study visits between weeks 13 and 24 of the OLTP1 In participants receiving placebo IV in the DBTP, the proportion of participants who achieve a sustained platelet count response, defined as achieving platelet counts of at least 50×10^9/L for at least 6 of the 8 study visits between weeks 17 and 24 of the OLTP1 In participants receiving placebo IV in the DBTP, the extent of disease control, defined as the number of cumulative weeks with platelet counts of at least 50×10^9/L during the first 24 weeks of treatment with efgartigimod IV Occurrence rate of rescue ITP therapy Proportion of participants with reduction in concurrent ITP therapy during the OLTP1 Incidence and severity of bleeding, assessed by the ITP Bleeding Scale (IBLS) Incidence and severity of adverse events (AEs) (including AEs of clinical interest) and serious AEs. Vital signs, laboratory safety measurements, physical examination Incidence and prevalence of antidrug antibodies (ADA) against efgartigimod in serum over time Incidence and prevalence of NAb against efgartigimod in serum over time Efgartigimod Cmax and Ctrough over time Percent change from baseline in total IgG levels in serum over time

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Is at least 18 years of age and the local legal age of consent for clinical studies when signing the informed consent form (ICF). Has documented baseline mean platelet count of <30 x 10^9/L before randomization Has a documented duration of primary immune thrombocytopenia (ITP) of more than 12 months on the date of informed consent form (ICF) signature Has documented prior ITP treatment with at least 1 of the following treatments: corticosteroids, intravenous immunoglobulin (IVIg), anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive), thrombopoietin receptor agonist (TPO-RAs), or rituximab Has documented insufficient response to a prior ITP treatment with corticosteroids, IVIg, anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive), TPO-RAs, rituximab (the specific criteria can be found in the protocol). Has documented prior response defined as 1 platelet count of 50×10^9/L to at least 1 of the following ITP treatments in the 3 years before the date of ICF signature: prednisone, dexamethasone, other or nonspecified corticosteroids, IVIg, or anti-D immunoglobulin (for participants who are nonsplenectomized and Rho(D)-positive).

Exclusion criteria

Other than the indication under study, known autoimmune disease or any medical condition that would interfere with an accurate assessment of clinical symptoms of ITP, confound the results of the study or put the participant at undue risk.
Secondary ITP
Nonimmune thrombocytopenia

Autoimmune hemolytic anemia
ITP-associated critical or severe bleeding

Calendar

(Last Update: 13/08/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
24/01/2025	27/02/2025	03/04/2025	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

Argenx Belgium

Industriepark-Zwijnaarde 7

Contact Person

Vice President Clinical Development

+3293103400

ClinicalTrials@argenx.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Parc de Salut Mar

Doctor Aiguader, 88 Edificio PRBB 08003 Barcelona

ceic-psmar@researchmar.net

933160679

Sites

Active (27/03/2025)

Complexo Hospitalario Universitario A Coruna

A Coruna

CORUÑA

Hematology

Active (28/02/2025)

Hospital Clinico Universitario De Valencia

Valencia

VALENCIA

Hematology

Active (27/02/2025)

Hospital General Universitario Morales Meseguer

Murcia

MURCIA

Hematology

not initialized (--/--/----)

Hospital Universitari Vall D Hebron

Barcelona

BARCELONA

Hematology

Active (20/03/2025)

Hospital Universitario Quironsalud Madrid

Pozuelo De Alarcon

MADRID

Hematology

Active (19/03/2025)

Hospital Universitario Ramon Y Cajal

Madrid

MADRID

Hematology

not initialized (--/--/----)

Institut Catala D´oncologia

L'hospitalet De Llobregat

BARCELONA

Hematology

Medication

Vyvgart 20 mg/mL concentrate for solution for infusion

SOLUTION FOR INFUSION

INTRAVENOUS USE

ATC code: L04AA58 - EFGARTIGIMOD ALFA

Active Principles: N/A

Orphan Experimental

ARGX-113

SOLUTION FOR INFUSION

INTRAVENOUS USE

-

Active Principles: N/A

Orphan Experimental

No results