A phase 3, randomized, double- blind, study of ianalumab (VAY736) versus placebo in addition to eltrombopag in primary immune thrombocytopenia (ITP) patients who failed steroids.

Estado	**Tipo de Participantes**	**Rangos de Edad**
Fin Reclutamiento	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	99
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2024-512890-28-00 (CTIS)

Cod. Protocolo
CVAY736Q12301

Transicionado 2022-001627-32

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

Enfermedad investigada
Primary immune thrombocytopenia (ITP)

Título Científico
A phase 3 randomized, double-blind study of ianalumab (VAY736) versus placebo in addition to eltrombopag in patients with primary immune thrombocytopenia (ITP) who had insufficient response or relapsed after first line steroid treatment (VAYHIT2)

Justificación

No aportado

Objetivo Principal

To demonstrate that the addition of ianalumab (either dose) to standard
of care, eltrombopag, prolongs Time to Treatment Failure (TTF) compared to eltrombopag alone in participants with primary ITP who have had an insufficient response to or relapsed after first-line treatment with corticosteroids

Variables de Evaluación Primaria

Time to treatment failure (TTF) defined as the time from randomization until: - Platelet counts <30 G/L or need for rescue treatment later than 8 weeks from randomization, - Start of new ITP treatment - Ineligibility to taper or inability to discontinue eltrombopag or - Death. TTF will be assessed in each treatment group and each of the 2 doses of ianalumab (ianalumab+ eltrombopag) will be compared to the control arm (placebo+ eltrombopag).

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To assess quality, time to and duration of response in each treatment arm
To assess the rate of participants who successfully taper and discontinue eltrombopag in each treatment arm
To assess the safety profile of ianalumab.
To assess the incidence and severity of bleeding in each treatment arm
To assess the need for rescue treatment in each treatment group.
To evaluate treatment effects on ITP related symptoms, functioning, and health-related quality of life (HFRQoL)
To assess B-Cell levels and immunoglobulin (Ig) levels
To assess ianalumab pharmacokinetics (PK)
To assess the immunogenicity of ianalumab

Variables de Evaluación Secundaria

At each time point: -Complete Response (CR) rate (proportion of participants with any platelet count 100 G/L without rescue or new ITP treatment).Response rate (R) (proportion of participants with any platelet count 50 G/L without rescue or new ITP treatment). Best response rate over all time points(proportion of participants with a best response of either R or CR). Time from randomization to R & CR. Duration of R & CR. Stable response at 6 months & at 1 year.
Probability to be treatment failure-free (as defined for the primary efficacy endpoint) at the end of the planned treatment period (end of Week 24)
Frequency of adverse events and other safety parameters. Number of severe infections and proportion of participants with severe infection.

Proportion of participants with bleeding events according to World Health Organization (WHO)Bleeding Scale.
Number and proportion of participants receiving rescue treatment.
Change from baseline on total score of the Patient- Reported Outcomes Measurement Information System (PROMIS)Short Form (SF) v1.0 Fatigue 13a. Change from baseline in ITP Patient Assessment Questionnaire (PAQ) domain scores of Symptoms, Fatigue, Bother, Activity.
B-cell levels: - Change from baseline in the frequency (% within the CD45) and absolute number of CD19+ B-cell counts - Time to first occurrence of B-cell recovery, defined as 80% of baseline or 50 cells/µL Immunoglobulins: - Change from baseline in immunoglobulin level
Ianalumab concentration in serum and PK parameters after the first and last dose in a subset of participants
Incidence and titer of anti-ianalumab antibodies in serum (anti-drug antibodies (ADA) assay) over time

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male or female patients aged 18 years and older on the day of signing the informed consent. A signed informed consent must be obtained prior to participation in the study A diagnosis of primary ITP, with insufficient response to, or relapse after a first-line corticosteroid therapy ± IVIG. Patients with platelet count <30 G/L for whom eltrombopag is clinically indicated as per physicians discretion and with no contraindication to receive eltrombopag.

Criterios de Exclusión

ITP patients who received second-line ITP treatments (other than corticosteroid therapy ± IVIG) including splenectomy. However, patients exposed to thrombopoietin receptor agonist (TPO-RAs) for a limited time (max. one week) before screening are eligible. Patients with key lab abnormalities and patients with Evans syndrome or any other cytopenia (patients with low grade anemia related to bleeding or iron deficiency are eligible). Patients with history of clinically significant hematological disorders, or with marked altered hematologic parameters. Patients with current or history of life-treating bleeding. Patients that are Human Immunodeficiency Virus (HIV), Hepatitis C Virus (HCV), Hepatitis B surface Antigen (HBsAg)/ Hepatitis B core antibody (HBcAb)- positive. Patients with known active or uncontrolled infection requiring systemic treatment during screening period. Patients with hepatic impairment with Child-Pugh score >5 Patients with concurrent coagulation disorder and/or receiving anti-platelet or anticoagulant medication with an exemption of low dose of acetylsalicylic acid (150 mg daily). Nursing (breast feeding) or pregnant women.

Calendario

(Última actualización: 20/03/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
23/01/2023	01/07/2024	01/07/2024	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
27/02/2025	No aportado	No aportado	No aportado	No aportado

Promotor

Novartis Pharma AG Switzerland

Lichtstrasse 35

Contact Person

Novartis Pharma Arzneimittel GmbH

+499112730

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Monetary support: N/A

Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Centros

Activo (30/07/2024)

COMPLEXO HOSPITALARIO UNIVERSITARIO DE SANTIAGO

Santiago de Compostela

CORUÑA

Activo (01/07/2024)

HOSPITAL CLINICO UNIVERSITARIO DE SALAMANCA (COMPLEJO ASIST. UNIVERSIT.SA)

Salamanca

SALAMANCA

Cerrado (28/03/2025)

HOSPITAL DEL MAR.

Barcelona

BARCELONA

Cerrado (02/04/2025)

HOSPITAL G. UNIVERSITARIO J.M. MORALES MESEGUER

Murcia

MURCIA

Cerrado (12/03/2025)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Cerrado (07/04/2025)

HOSPITAL UNIVERSITARIO 12 DE OCTUBRE

Madrid

MADRID

Medicamentos

-
-
UNKNOWN USE

Código ATC: N02BE - ANILIDAS
Principios Activos: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL USE

-
Principios Activos: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-
Principios Activos: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

Código ATC: J06BA - INMUNOGLOBULINAS HUMANAS NORMALES
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

Código ATC: J05AF10 - ENTECAVIR
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

Código ATC: A02BA - ANTAGONISTAS DEL RECEPTOR H2
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

Código ATC: H02AB - GLUCOCORTICOIDES
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

Código ATC: R06A - ANTIHISTAMINICOS PARA USO SISTEMICO
Principios Activos: N/A

Experimental

VAY736
CONCENTRATE FOR SOLUTION FOR INFUSION
INTRAVENOUS

-

Principios Activos: N/A

Huérfano **Experimental**

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-

Principios Activos: N/A

Experimental

Sin resultados

A phase 3, randomized, double- blind, study of ianalumab (VAY736) versus placebo in addition to eltrombopag in primary immune thrombocytopenia (ITP) patients who failed steroids.

State	Type of participants	Age Ranges
End of recruitment	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	99
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2024-512890-28-00 (CTIS)

Protocol Code
CVAY736Q12301

Transitioned 2022-001627-32

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

Investigated Disease
Primary immune thrombocytopenia (ITP)

Scientific Title
A phase 3 randomized, double-blind study of ianalumab (VAY736) versus placebo in addition to eltrombopag in patients with primary immune thrombocytopenia (ITP) who had insufficient response or relapsed after first line steroid treatment (VAYHIT2)

Rationale

Not provided

Main Objective

To demonstrate that the addition of ianalumab (either dose) to standard
of care, eltrombopag, prolongs Time to Treatment Failure (TTF) compared to eltrombopag alone in participants with primary ITP who have had an insufficient response to or relapsed after first-line treatment with corticosteroids

Primary Endpoints

Time to treatment failure (TTF) defined as the time from randomization until: - Platelet counts <30 G/L or need for rescue treatment later than 8 weeks from randomization, - Start of new ITP treatment - Ineligibility to taper or inability to discontinue eltrombopag or - Death. TTF will be assessed in each treatment group and each of the 2 doses of ianalumab (ianalumab+ eltrombopag) will be compared to the control arm (placebo+ eltrombopag).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To assess quality, time to and duration of response in each treatment arm
To assess the rate of participants who successfully taper and discontinue eltrombopag in each treatment arm
To assess the safety profile of ianalumab.
To assess the incidence and severity of bleeding in each treatment arm
To assess the need for rescue treatment in each treatment group.
To evaluate treatment effects on ITP related symptoms, functioning, and health-related quality of life (HFRQoL)
To assess B-Cell levels and immunoglobulin (Ig) levels
To assess ianalumab pharmacokinetics (PK)
To assess the immunogenicity of ianalumab

Secondary Endpoints

At each time point: -Complete Response (CR) rate (proportion of participants with any platelet count 100 G/L without rescue or new ITP treatment).Response rate (R) (proportion of participants with any platelet count 50 G/L without rescue or new ITP treatment). Best response rate over all time points(proportion of participants with a best response of either R or CR). Time from randomization to R & CR. Duration of R & CR. Stable response at 6 months & at 1 year.
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Ianalumab concentration in serum and PK parameters after the first and last dose in a subset of participants
Incidence and titer of anti-ianalumab antibodies in serum (anti-drug antibodies (ADA) assay) over time

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male or female patients aged 18 years and older on the day of signing the informed consent. A signed informed consent must be obtained prior to participation in the study A diagnosis of primary ITP, with insufficient response to, or relapse after a first-line corticosteroid therapy ± IVIG. Patients with platelet count <30 G/L for whom eltrombopag is clinically indicated as per physicians discretion and with no contraindication to receive eltrombopag.

Exclusion criteria

ITP patients who received second-line ITP treatments (other than corticosteroid therapy ± IVIG) including splenectomy. However, patients exposed to thrombopoietin receptor agonist (TPO-RAs) for a limited time (max. one week) before screening are eligible. Patients with key lab abnormalities and patients with Evans syndrome or any other cytopenia (patients with low grade anemia related to bleeding or iron deficiency are eligible). Patients with history of clinically significant hematological disorders, or with marked altered hematologic parameters. Patients with current or history of life-treating bleeding. Patients that are Human Immunodeficiency Virus (HIV), Hepatitis C Virus (HCV), Hepatitis B surface Antigen (HBsAg)/ Hepatitis B core antibody (HBcAb)- positive. Patients with known active or uncontrolled infection requiring systemic treatment during screening period. Patients with hepatic impairment with Child-Pugh score >5 Patients with concurrent coagulation disorder and/or receiving anti-platelet or anticoagulant medication with an exemption of low dose of acetylsalicylic acid (150 mg daily). Nursing (breast feeding) or pregnant women.

Calendar

(Last Update: 20/03/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
23/01/2023	01/07/2024	01/07/2024	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
27/02/2025	Not aported	Not aported	Not aported	Not aported

Sponsor

Novartis Pharma AG Switzerland

Lichtstrasse 35

Contact Person

Novartis Pharma Arzneimittel GmbH

+499112730

legal.representative@novartis.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

CEIm Hospital Universitario La Paz

Paseo de la Castellana, 261 28046 Madrid

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917277413

Sites

Active (30/07/2024)

COMPLEXO HOSPITALARIO UNIVERSITARIO DE SANTIAGO

Santiago de Compostela

CORUÑA

Active (01/07/2024)

HOSPITAL CLINICO UNIVERSITARIO DE SALAMANCA (COMPLEJO ASIST. UNIVERSIT.SA)

Salamanca

SALAMANCA

Closed (28/03/2025)

HOSPITAL DEL MAR.

Barcelona

BARCELONA

Closed (02/04/2025)

HOSPITAL G. UNIVERSITARIO J.M. MORALES MESEGUER

Murcia

MURCIA

Closed (12/03/2025)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Closed (07/04/2025)

HOSPITAL UNIVERSITARIO 12 DE OCTUBRE

Madrid

MADRID

Medication

-
-
UNKNOWN USE

ATC code: N02BE - ANILIDAS
Active Principles: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL USE

-
Active Principles: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-
Active Principles: N/A

Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

ATC code: J06BA - INMUNOGLOBULINAS HUMANAS NORMALES
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

ATC code: J05AF10 - ENTECAVIR
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

ATC code: A02BA - ANTAGONISTAS DEL RECEPTOR H2
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

ATC code: H02AB - GLUCOCORTICOIDES
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

ATC code: R06A - ANTIHISTAMINICOS PARA USO SISTEMICO
Active Principles: N/A

Experimental

VAY736
CONCENTRATE FOR SOLUTION FOR INFUSION
INTRAVENOUS

-
Active Principles: N/A

Orphan Experimental

ELTROMBOPAG
FILM-COATED TABLET
ORAL

-
Active Principles: N/A

Experimental

No results