

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
Fin Reclutamiento

Tipo de Participantes
No aportado

Rangos de Edad
Mayores de 64 , Adultos (18-64 años)

Género
Ambos

Fases
Fase III

Participantes esperados
99

Resultados
Sin resultados

Bajo nivel intervención
No

Enfermedad rara
Si

Cobertura geográfica
Sin determinar

Ámbitos del ensayo
tratamiento, seguridad, eficacia,
farmacocinética, farmacodinamica

Terapia avanzada
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/ μ L
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 $\pm$ 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 $\pm$ 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-threatening 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 23/01/2023	Inicio de Ensayo 01/07/2024	Inclusión Primer Paciente 01/07/2024	Interrumpido No aportado	Reiniciado No aportado
Fin de reclutamiento 27/02/2025	Fin prematuro (España) No aportado	Fin prematuro (Global) No aportado	Fin del ensayo en España No aportado	Fin del ensayo global No aportado

Promotor

Novartis Pharma AG Switzerland Lichtstrasse 35
Contact Person Novartis Pharma Arzneimittel GmbH +499112730 legal.representative@novartis.com
Monetary support: N/A Tipo de promotor: Comercial

Ceim

CEIm Hospital Universitario La Paz

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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

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-
UNKNOWN USE

Código ATC: J06BA - INMUNOGLOBULINAS HUMANAS NORMALES

Principios Activos: N/A

Experimental

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UNKNOWN USE

Código ATC: J05AF10 - ENTECAVIR

Principios Activos: N/A

Experimental

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UNKNOWN USE

Código ATC: A02BA - ANTAGONISTAS DEL RECEPTOR H2

Principios Activos: N/A

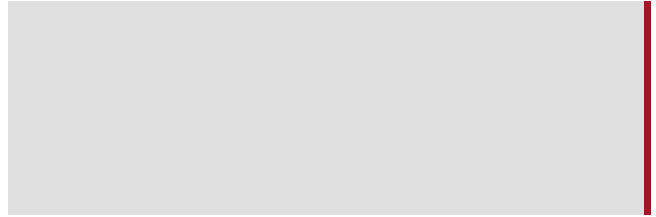
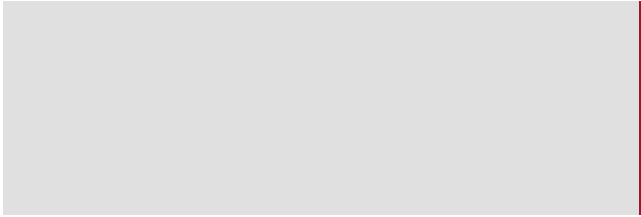
Experimental

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-
UNKNOWN USE

Código ATC: H02AB - GLUCOCORTICOIDES

Principios Activos: N/A

Experimental



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

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Exclusion criteria

ITP patients who received second-line ITP treatments (other than corticosteroid therapy $\pm$ 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-threatening 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 23/01/2023	Start of Trial 01/07/2024	First patient inclusion 01/07/2024	Halted Not aported	Restarted Not aported
End of recruitment 27/02/2025	Premature end (Spain) Not aported	Premature End (Global) Not aported	Trial end (Spain) Not aported	Trial end (Global) Not aported

Sponsor

Novartis Pharma AG Switzerland
Lichtstrasse 35

Contact Person

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Monetary support: N/A
Sponsor type: Commercial

Ceim

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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
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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

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UNKNOWN USE

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

Experimental

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-
UNKNOWN USE

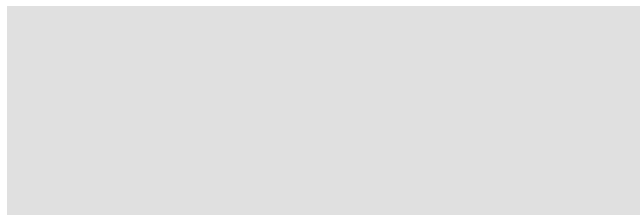
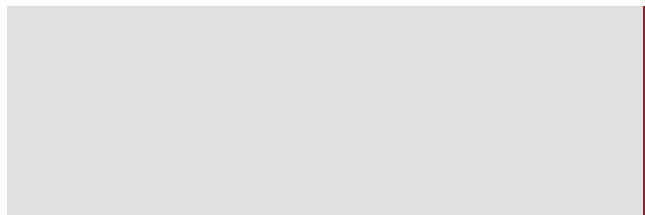
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Active Principles: N/A

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