Estudio de ianalumab (VAY736) en combinación con corticosteroides en primera línea en pacientes con trombocitopenia inmune primaria

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	168
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica, dosis	NO

Información

Identificador
2024-511704-17-00 (CTIS)

Cod. Protocolo
CVAY736I12301

Transicionado 2022-001672-34

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

Enfermedad investigada
Trombocitopenia inmune primaria (PTI)

Título Científico
Estudio de fase III, aleatorizado y doble ciego de ianalumab (VAY736) frente a placebo en combinación con corticosteroides en primera línea en trombocitopenia inmune primaria (VAYHIT1).

Justificación

No aportado

Objetivo Principal

Demostrar que la adición de ianalumab (en cualquiera de las dosis) a los corticosteroides estándar de primera línea aumenta la tasa de respuesta estable sin tratamiento a los 12 meses (SROT-12) en comparación con el grupo de control (placebo + corticosteroides) en participantes con PTI primaria que respondieron a los corticosteroides (+/- IVIG) antes de la aleatorización.

Variables de Evaluación Primaria

Definición de variable binaria: un participante se considerará que tiene SROT-12 si al menos el 75% de los recuentos de plaquetas recogidos entre las visitas de D1 de S53 y de D1 de S65 (dias 352 y 463 del estudio) se clasifican como ¿respuesta¿ (cualquier recuento de plaquetas de al menos 50G/L sin tratamiento de rescate o nuevo tratamiento para PTI). Cada una de las dos dosis de ianalumab (ianalumab+corticosteroides) se comparará con la del grupo control (placebo+corticosteriodes)

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Demostrar que la adición de ianalumab (en cualquiera de las dosis) a los corticosteroides estándar de primera línea prolonga el tiempo hasta el fracaso del tratamiento (TTF) en comparación con el grupo de control (placebo + corticosteroides) en participantes con PTI primaria que respondieron a los corticosteroides (+/- IVIG) antes de la aleatorización.
Evaluar la calidad de la respuesta, el tiempo hasta y la duración de la respuesta completa en cada grupo de tratamiento.
Evaluar el perfil de seguridad de ianalumab.
Evaluar la incidencia y la gravedad de sangrado en cada grupo de tratamiento.
Evaluar la necesidad de utilizar tratamiento de rescate en cada grupo de tratamiento.
Evaluar los efectos del tratamiento en los síntomas, el funcionamiento y la calidad de vida relacionada con la salud (HRQoL) en relación con la PTI.
Evaluar los niveles de células B e inmunoglobulina en cada grupo de tratamiento.
Evaluar la farmacocinética (PK) de ianalumab.
Evaluar la inmunogenicidad de ianalumab

Variables de Evaluación Secundaria

Time from randomization to Treatment Failure (TTF) defined as platelet count below 30 G/L later than 8 weeks from randomization, need for a rescue treatment later than 8 weeks from randomization, start of a second-line therapy or

death (whatever the cause). TTF will be assessed in each treatment group and each of the two doses of ianalumab (ianalumab+corticosteroids) will be compared to the control arm (placebo+corticosteroids)
Complete Response (CR) rate at each timepoint defined as the proportion of participants with any platelet count of at least 100 G/L in the absence of rescue treatment or new ITP treatment.
Response (R) rate at each timepoint defined as the proportion of participants with any platelet count of at least 50 G/L in the absence of rescue treatment or new ITP treatment
Time from randomization to date of first complete response
Duration of complete response is defined as the time from achievement of CR to loss of CR.
Stable response at 6 months and at 1 year
Number of severe infections and proportion of participants with severe infection. Frequency of adverse events and other safety parameters
Proportion of participants with bleeding events overall and by WHO bleeding scale severity
Number and proportion of participants receiving rescue treatment. Cumulative dose/duration of corticosteroids exposure.
Change from baseline on T-Scores of the PROMIS SF v1.0 Fatigue 13a. Change from baseline in ITP-PAQ domain scores of Symptoms, Fatigue, Bother, Activity
B-cell levels: Change from baseline in 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/L. Immunoglobulins: Change from baseline in immunoglobulin levels.
Ianalumab concentration in serum and PK parameters.
Incidence and titer of anti-ianalumab antibodies in serum (ADA assay) over time.

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Consentimiento informado firmado antes de participar en el estudio.
Participantes de ambos sexos de 18 años de edad o más en el día de la firma del consentimiento informado
PTI primaria diagnosticada en los 3 meses anteriores al inicio del tratamiento en primera línea para la PTI (corticosteroides, IgIV).
Recuento de plaquetas por debajo de 30 G/L antes de comenzar cualquier tratamiento de primera línea para la PTI (corticosteroides, IgIV).
Respuesta (recuento de plaquetas ¿50 G/L) a los corticosteroides (+/- IgIV) en cualquier
momento antes de la aleatorización. Nota: El recuento de plaquetas medido en los 7 días
posteriores a la transfusión de plaquetas no se considerará como una respuesta

Criterios de Exclusión

Síndrome de Evans o cualquier otra citopenia (los pacientes con anemia de bajo grado relacionada con sangrado o deficiencia de hierro son elegibles).
Sangrado actual potencialmente mortal.
Tratamiento previo de la PTI, incluida la esplenectomía y exceptuando los corticosteroides
y/o la IgIV iniciados como tratamiento de primera línea, hasta 28 días antes de la aleatorización
y corticosteroides de rescate y/o IgIV administrados antes de la confirmación del diagnóstico
de PTI primaria
Uso previo de terapia de depleción de células B (p. ej., rituximab).
Recuento absoluto de neutrófilos por debajo de 1,0 G/L en la aleatorización.

Participantes con trastornos de la coagulación concurrentes y/o que reciban medicación antiplaquetaria o anticoagulante exceptuando una dosis baja de ácido acetilsalicílic

Calendario

(Última actualización: 15/09/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
23/12/2022	18/11/2023	18/11/2023	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
14/08/2025	No aportado	No aportado	No aportado	No aportado

Promotor

Novartis Pharma AG Switzerland

Lichtstrasse 35

Contact Person

Novartis Pharma Arzneimittel GmbH

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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

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Centros

Cerrado (28/11/2025)

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

Salamanca

SALAMANCA

Activo (01/02/2024)

HOSPITAL G. UNIVERSITARIO J.M. MORALES MESEGUER

Murcia

MURCIA

Activo (14/12/2023)

HOSPITAL GENERAL UNIVERSITARIO GREGORIO MARAÑON

Madrid

MADRID

Activo (13/11/2023)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Cerrado (19/11/2025)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Activo (28/05/2025)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Medicamentos

VAY736
CONCENTRATE FOR SOLUTION FOR INFUSION
INTRAVENOUS

-
Principios Activos: N/A
Huérfano
Experimental

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

Sin resultados

A study of ianalumab (VAY736) in addition to first-line corticosteroids in patients with primary immune thrombocytopenia

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	168
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, dose	NO

Information

Identifier
2024-511704-17-00 (CTIS)

Protocol Code
CVAY736I12301

Transitioned 2022-001672-34

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

Investigated Disease
Primary Immune thrombocytopenia (ITP)

Scientific Title
A phase III, randomized, double-blind study of ianalumab (VAY736) versus placebo in addition to first-line corticosteroids in primary immune thrombocytopenia (VAYHIT1)

Rationale

Not provided

Main Objective

To demonstrate that the addition of ianalumab (either dose) to standard first-line corticosteroids increases the rate of SROT-12 compared to the control arm (placebo+corticosteroids) in participants with primary ITP who responded to corticosteroids (+/-IVIG) prior to randomization.

Primary Endpoints

The binary variable is defined as: A participant will be considered in SROT-12 if at least 75% of the platelet counts collected between visits Week 53 Day 1 and Week 65 Day 1 (Study Days 352 and 463) qualify as Response (any platelet counts of at least 50 G/L in the absence of rescue treatment or new ITP treatment). Each of the two doses of ianalumab (ianalumab+corticosteroids) will be compared to the control arm (placebo+corticosteroids).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To demonstrate that the addition of ianalumab (either dose) to standard first-line corticosteroids prolongs Time to Treatment Failure (TTF) compared to the control arm (placebo+corticosteroids) in participants with primary ITP who responded to corticosteroids (+/-IVIG) prior to randomization.
To assess quality of response, time to and duration of complete response in each treatment group
To assess the safety profile of ianalumab
To assess the incidence and severity of bleeding in each treatment arm
To assess the need of rescue treatment in each treatment group
To evaluate treatment effects on ITP related symptoms, functioning, and health-related quality of life (HRQoL)
To assess B-cell and immunoglobulin levels in each treatment group
To assess ianalumab pharmacokinetics (PK)
To assess the immunogenicity of ianalumab

Secondary Endpoints

Time from randomization to Treatment Failure (TTF) defined as platelet count below 30 G/L later than 8 weeks from randomization, need for a rescue treatment later than 8 weeks from randomization, start of a second-line therapy or death (whatever the cause). TTF will be assessed in each treatment group and each of the two doses of ianalumab (ianalumab+corticosteroids) will be compared to the control arm (placebo+corticosteroids)
Complete Response (CR) rate at each timepoint defined as the proportion of participants with any platelet count of at least 100 G/L in the absence of rescue treatment or new ITP treatment.
Response (R) rate at each timepoint defined as the proportion of participants with any platelet count of at least 50

G/L in the absence of rescue treatment or new ITP treatment
Time from randomization to date of first complete response
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Ianalumab concentration in serum and PK parameters.
Incidence and titer of anti-ianalumab antibodies in serum (ADA assay) over time.

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Signed informed consent prior to participation in the study.
Male or female participants aged 18 years and older on the day of signing informed consent
Primary ITP diagnosed within 3 months before initiating first-line ITP therapy (corticosteroids, IVIG)
Platelet count below 30 G/L before starting any first-line ITP therapy (corticosteroids, IVIG)
Response (platelet count 50 G/L) to corticosteroids (+/- IVIG) at any time prior to randomization. Note: Platelet count measured within 7 days of platelet transfusion will not be considered as response

Exclusion criteria

Evans syndrome or any other cytopenia (patients with low grade anemia related to bleeding or iron deficiency are eligible).
Current life-threatening bleeding
Previous ITP treatment, including splenectomy, except for corticosteroids and/or IVIG initiated as first-line therapy for up 28 days before randomization/or IVIG given prior to confirmed diagnosis of primary ITP.
Prior use of B-cell depleting therapy (e.g., rituximab)
Absolute neutrophil count below 1.0 G/L at randomization
Participants with concurrent coagulation disorders and/or receiving anti-platelet or anticoagulant medication with an exemption of low dose of acetylsalicylic acid

Calendar

(Last Update: 15/09/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
23/12/2022	18/11/2023	18/11/2023	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
14/08/2025	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 (28/11/2025)

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

Salamanca

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BARCELONA

Closed (19/11/2025)

HOSPITAL UNIVERSITARIO LA PAZ

Madrid

MADRID

Active (28/05/2025)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Medication

VAY736
CONCENTRATE FOR SOLUTION FOR INFUSION
INTRAVENOUS

-
Active Principles: N/A
Orphan Experimental

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

-
-
UNKNOWN USE

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

No results