A study of efficacy and safety of ianalumab in previously treated patients with warm autoimmune haemolytic anaemia

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	60
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2024-510635-21-00 (CTIS)

Cod. Protocolo
CVAY736O12301

Transicionado 2022-001773-31

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

Enfermedad investigada
warm autoimmune haemolytic anaemia (wAIHA)

Título Científico
A phase 3, randomized, double-blind, study to assess efficacy and safety of ianalumab (VAY736) versus placebo in warm autoimmune hemolytic anemia (wAIHA) patients who failed at least one line of treatment (VAYHIA)

Justificación

No aportado

Objetivo Principal

To demonstrate that either dose of ianalumab induces durable hemoglobin (Hb) response compared to placebo in patients with wAIHA. The primary clinical question of interest is: What is the effect of either dose of ianalumab versus placebo (with or without supportive care), in inducing durable hemoglobin response, in wAIHA patients 18 years of age who failed at least one line of treatment, regardless of premature discontinuation from study treatment or temporary treatment interruption/delay, and in the absence of rescue or prohibited treatment?

Variables de Evaluación Primaria

Binary variable indicating whether a patient achieves a durable response (Hb 10 g/dL and 2 g/dL increase from baseline), for a period of at least 8 weeks, between W9 and W25, in the absence of rescue or prohibited treatment

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To demonstrate that either dose of ianalumab maintains durable hemoglobin response, that is sustained beyond the end of the treatment period, compared to placebo
To assess the time to durable response / response / complete response in each treatment group
To assess the quality of response in each treatment group.
To assess the need for rescue treatments in each treatment group
To assess the safety profile of ianalumab
To characterize the pharmacokinetics (PK) of ianalumab
To assess B-cell levels and immunoglobulin levels at each treatment group
To assess the immunogenicity against ianalumab
To assess the quality of life (QoL) in each treatment group

Variables de Evaluación Secundaria

Duration of response
Time from randomization to achievement of durable response, to first response, to first complete response
Response rate, complete response rate and hemoglobin level
Number and proportion of participants that receive rescue treatment overall and by type of rescue treatment
Time-standardized numbers of each type of rescue treatment
Change from baseline in time-standardized number of transfusions
Frequency of AEs and other safety parameters
Ianalumab concentration in serum and PK parameters after the first and last dose

B-cell levels: Change from baseline in the frequency 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
Incidence and titer of anti-drug antibodies (ADA) in serum over time
Change from baseline in dedicated Patient Reported Outcomes (PROs)

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Written informed consent form (ICF) must be obtained prior to any screening assessments Male or female participants aged 18 years and older on the day of signing the ICF Participants with primary or secondary wAIHA (previously documented by positive direct antiglobulin test (DAT) specific for anti-IgG or anti-IgA), who had an insufficient response to, or relapsed after at least one line of treatment, including patients with corticosteroid resistance, dependence, or intolerance Hemoglobin concentration 5 g/dL and <10 g/dL and presence of symptoms related to anemia at Screening and Week 1. The dose of supportive care must be stable for at least 4 weeks prior randomization.

Criterios de Exclusión

Patients with wAIHA secondary to hematologic disease involving bone marrow (e.g., chronic lymphocytic leukemia (CLL)) or another disease requiring prohibited medication. Of note, the patients with autoimmune diseases like lupus nephritis (LN), systemic lupus erythematosus (SLE), Primary Sjögrens Syndrome (pSS) or autoimmune hepatitis (AIH) after wash-out from the treatments are allowed
Prior use of B-cell depleting therapy: within 12 weeks prior randomization, or no hematologic response to the last course of B-cell depleting therapy, irrespective of time of administration
Active viral, bacterial, or other infections (including active or latent tuberculosis or SARSCoV- 2) requiring systemic treatment at the time of screening or history of recurrent clinically significant infection
Known history of primary or secondary immunodeficiency, or patients that are Human Immunodeficiency Virus (HIV), Hepatitis C Virus (HCV), Hepatitis B surface Antigen (HBsAg)/ Hepatitis B core antibody (HBcAb)-positive. Refer to Section 5.2 exclusion criterion #7b for exemptions applicable for patients who are HBsAg negative and HBcAb positive.
Live or live-attenuated vaccination within 4 weeks before randomization

Calendario

(Última actualización: 11/03/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
14/02/2023	06/07/2023	06/07/2023	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
18/11/2024	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

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ceic.hulp@salud.madrid.org

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Centros

Activo (02/05/2024)

Hospital G. Universitario J.M. Morales Meseguer

Murcia

MURCIA

Activo (06/07/2023)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Medicamentos

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

Código ATC: B03XA01 - ERITROPOYETINA
Principios Activos: N/A

Experimental

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

Código ATC: N02BE - ANILIDAS
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

-
-
UNKNOWN USE

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

Experimental

-
-
ORAL USE

Código ATC: G03XA01 - DANAZOL
Principios Activos: N/A

Experimental

Sin resultados

A study of efficacy and safety of ianalumab in previously treated patients with warm autoimmune haemolytic anaemia

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	60
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2024-510635-21-00 (CTIS)

Protocol Code
CVAY736O12301

Transitioned 2022-001773-31

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

Investigated Disease
warm autoimmune haemolytic anaemia (wAIHA)

Scientific Title
A phase 3, randomized, double-blind, study to assess efficacy and safety of ianalumab (VAY736) versus placebo in warm autoimmune hemolytic anemia (wAIHA) patients who failed at least one line of treatment (VAYHIA)

Rationale

Not provided

Main Objective

To demonstrate that either dose of ianalumab induces durable hemoglobin (Hb) response compared to placebo in patients with wAIHA. The primary clinical question of interest is: What is the effect of either dose of ianalumab versus placebo (with or without supportive care), in inducing durable hemoglobin response, in wAIHA patients 18 years of age who failed at least one line of treatment, regardless of premature discontinuation from study treatment or temporary treatment interruption/delay, and in the absence of rescue or prohibited treatment?

Primary Endpoints

Binary variable indicating whether a patient achieves a durable response (Hb 10 g/dL and 2 g/dL increase from baseline), for a period of at least 8 weeks, between W9 and W25, in the absence of rescue or prohibited treatment

Temporary moments of secondary assessment

Not provided

Secondary Objective

To demonstrate that either dose of ianalumab maintains durable hemoglobin response, that is sustained beyond the end of the treatment period, compared to placebo
To assess the time to durable response / response / complete response in each treatment group
To assess the quality of response in each treatment group.
To assess the need for rescue treatments in each treatment group
To assess the safety profile of ianalumab
To characterize the pharmacokinetics (PK) of ianalumab
To assess B-cell levels and immunoglobulin levels at each treatment group
To assess the immunogenicity against ianalumab
To assess the quality of life (QoL) in each treatment group

Secondary Endpoints

Duration of response
Time from randomization to achievement of durable response, to first response, to first complete response
Response rate, complete response rate and hemoglobin level
Number and proportion of participants that receive rescue treatment overall and by type of rescue treatment
Time-standardized numbers of each type of rescue treatment
Change from baseline in time-standardized number of transfusions
Frequency of AEs and other safety parameters
Ianalumab concentration in serum and PK parameters after the first and last dose

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Immunoglobulins: Change from baseline in immunoglobulin levels
Incidence and titer of anti-drug antibodies (ADA) in serum over time
Change from baseline in dedicated Patient Reported Outcomes (PROs)

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Written informed consent form (ICF) must be obtained prior to any screening assessments Male or female participants aged 18 years and older on the day of signing the ICF Participants with primary or secondary wAIHA (previously documented by positive direct antiglobulin test (DAT) specific for anti-IgG or anti-IgA), who had an insufficient response to, or relapsed after at least one line of treatment, including patients with corticosteroid resistance, dependence, or intolerance Hemoglobin concentration 5 g/dL and <10 g/dL and presence of symptoms related to anemia at Screening and Week 1. The dose of supportive care must be stable for at least 4 weeks prior randomization.

Exclusion criteria

Patients with wAIHA secondary to hematologic disease involving bone marrow (e.g., chronic lymphocytic leukemia (CLL)) or another disease requiring prohibited medication. Of note, the patients with autoimmune diseases like lupus nephritis (LN), systemic lupus erythematosus (SLE), Primary Sjögrens Syndrome (pSS) or autoimmune hepatitis (AIH) after wash-out from the treatments are allowed
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Live or live-attenuated vaccination within 4 weeks before randomization

Calendar

(Last Update: 11/03/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
14/02/2023	06/07/2023	06/07/2023	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
18/11/2024	Not aported	Not aported	Not aported	Not aported

Sponsor

Novartis Pharma AG Switzerland

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Contact Person

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Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

Active (02/05/2024)

Hospital G. Universitario J.M. Morales Meseguer

Murcia

MURCIA

Active (06/07/2023)

HOSPITAL UNIVERSITARI VALL D'HEBRON

Barcelona

BARCELONA

Medication

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

ATC code: B03XA01 - ERITROPOYETINA
Active Principles: N/A

Experimental

-
-
UNKNOWN USE

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

Experimental

-
-
UNKNOWN USE

ATC code: N02BE - ANILIDAS
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

-
-
UNKNOWN USE

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

Experimental

-
-
ORAL USE

ATC code: G03XA01 - DANAZOL
Active Principles: N/A

Experimental

No results