

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

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 60
Resultados Sin resultados	Bajo nivel intervención No	Enfermedad rara No
Cobertura geográfica Sin determinar	Ámbitos del ensayo tratamiento, seguridad, eficacia, farmacocinética, farmacodinámica	Terapia avanzada 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 SARS-CoV-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 14/02/2023	Inicio de Ensayo 06/07/2023	Inclusión Primer Paciente 06/07/2023	Interrumpido No aportado	Reiniciado No aportado
Fin de reclutamiento 18/11/2024	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

Paseo de la Castellana, 261 28046 Madrid

ceic.hulp@salud.madrid.org

917277413

Centros

Hospital G. Universitario J.M. Morales
Meseguer

Murcia

MURCIA

Activo (02/05/2024)

HOSPITAL UNIVERSITARI VALL
D'HEBRON

Barcelona

BARCELONA

Activo (06/07/2023)

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

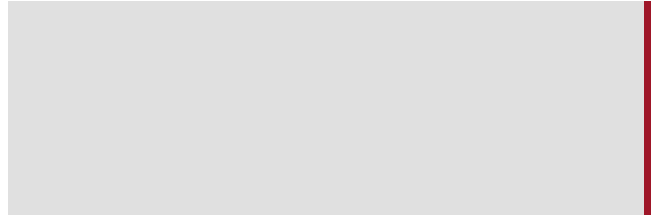
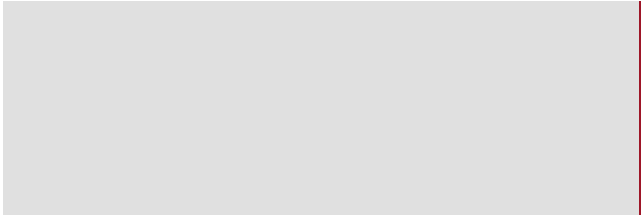
Experimental

-
-

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
End of recruitment

Type of participants
Not provided

Age Ranges
Older than 64 , Adults (18-64 years)

Gender
Both

Phases
Phase III

Expected Participants
60

Results
No results

Low level of intervention
No

Rare disease
No

Geographic coverage
Undetermined

Areas of the study
treatment, safety, effectiveness,
pharmacokinetics, pharmacodynamics

Advanced therapy
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

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)

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
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 SARS-CoV-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

Calendar

(Last Update: 11/03/2026)

Authorization 14/02/2023	Start of Trial 06/07/2023	First patient inclusion 06/07/2023	Halted Not aported	Restarted Not aported
End of recruitment 18/11/2024	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

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

ceic.hulp@salud.madrid.org

917277413

Sites

Active (02/05/2024)	Hospital G. Universitario J.M. Morales Meseguer
	Murcia
Active (06/07/2023)	HOSPITAL UNIVERSITARI VALL D'HEBRON
	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

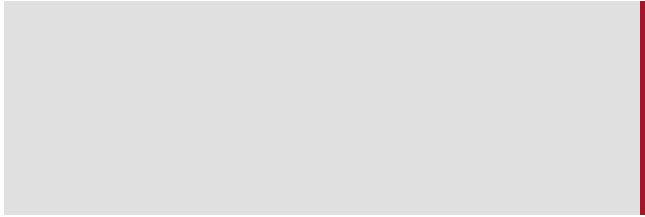
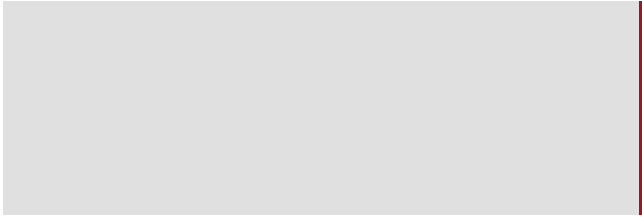
-

-

UNKNOWN USE

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

Experimental



-
-

ORAL USE

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

Experimental

No results