A multicenter, single arm, open-label trial to evaluate efficacy and safety of oral, twice daily iptacopan in adult PNH patients who have Hb10 g/dL in response to anti-C5 antibody and switch to iptacopan

Estado	Tipo de Participantes	Rangos de Edad
Reclutando	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	50
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	No
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Multicéntrico internacional	tratamiento, seguridad, eficacia, farmacocinética, farmacodinamica	NO

Información

Identificador
2022-502148-10-00 (CTIS)

Cod. Protocolo
CLNP023C12303

Área terapéutica
Enfermedades [C] - Patologías del sistema inmunitario [C20]|

Enfermedad investigada
Paroxysmal Nocturnal Hemoglobinuria (PNH)

Título Científico
A multicenter, single arm, open-label trial to evaluate efficacy and safety of oral, twice daily iptacopan in adult PNH patients who have Hb10 g/dL in response to anti-C5 antibody and switch to iptacopan

Justificación

No aportado

Objetivo Principal

The main question this trial is designed to answer: is iptacopan treatment equally effective to standard of care when switching treatment to iptacopan?

Variables de Evaluación Primaria

Measuring change in hemoglobin levels before start of iptacopan treatment and mean of hemoglobin levels at Day 126 and Day 168

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

Is iptacopan better in effectiveness than standard of care treatment

Variables de Evaluación Secundaria

Measuring change in hemoglobin levels before start of iptacopan treatment and mean of hemoglobin levels at Day 126 and Day 168

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Signed informed consent must be obtained prior to participation in the study Male and female participants 18 years of age, at the time of ICF signatures and with a diagnosis of PNH confirmed by PNH clone size WBCs 10% based on historical data. Stable regimen (dose and intervals) of anti-C5 antibody treatment (either eculizumab or i.v. ravulizumab) for at least 6 months prior to screening Mean hemoglobin level 10 g/dL, documented by the mean of all available Hb assessments (minimum 2 measurements) over a period of 6 months before screening visit, collected at any laboratory. In addition to fulfill the Hb eligibility criterion, participants must have two different samples collected during the screening period and tested by the central laboratory with the mean >10 g/dL, prior to starting iptacopan.

Vaccination against Neisseria meningitidis and S. pneumoniae infection are required prior to the start of iptacopan treatment. If the patient has not been previously vaccinated, or if a booster is required, vaccine are to be given according to local regulations, at least 2 weeks prior to first dosing. However, administration of these vaccines less than 2 weeks prior to start of iptacopan treatment is at the discretion of the investigator. If iptacopan treatment is started less than 2 weeks post-vaccination, participant must be given prophylactic antibiotic at the start of iptacopan and for at least 2 weeks after vaccination. If not received previously, vaccination against Haemophilus influenzae infections is recommended, if available and according to local regulations. The vaccines should be given at least 2 weeks prior to initiation of iptacopan treatment. However, administration of these vaccines less than 2 weeks prior to start of iptacopan treatment, is at the discretion of the investigator. If iptacopan treatment is started less than 2 weeks post-vaccination, participant must be given prophylactic antibiotic at the start of iptacopan and for at least 2 weeks after vaccination. Able to communicate well with the investigator, to understand and comply with the requirements of the study

Criterios de Exclusión

Participation in any other investigational drug trial or use of other investigational drugs at the time of enrollment
Ongoing drug or alcohol abuse that could interfere with patient's participation in the trial.
Any medical condition deemed likely to interfere with the patient's participation in the study
Female patients who are pregnant or breastfeeding, or intending to conceive during the course of the study
Patients requiring red blood cell transfusion in the 6 months prior to screening or during screening
History of stem cell transplantation or any solid organ transplantation
Active systemic bacterial, viral (incl. COVID-19) or fungal infection within 14 days prior to study drug administration
Presence of fever 38.0 °C (100.4 °F) within 7 days prior to study drug administration
Human immunodeficiency virus (HIV) infection (known history of HIV or test positive for HIV antibody at Screening)
A history of recurrent invasive infections caused by encapsulated organisms, e.g. meningococcus or pneumococcus
Unstable medical condition including, but not limited to, myocardial ischemia, active gastrointestinal bleeding, coexisting chronic anemia unrelated to PNH, or unstable thrombotic event not amenable to active treatment as judged by the investigator at Screening.
History of cancer of any part of the body within the past 5 years

Calendario

(Última actualización: 28/05/2025)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
19/09/2023	27/10/2023	27/10/2023	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
No aportado	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

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Centros

No iniciado (--/--/----)

Complexo Hospitalario Universitario De Santiago

Santiago De Compostela

CORUÑA

#2102: Hematologia

Cerrado (03/04/2025)

Hospital Clinic De Barcelona

Barcelona

BARCELONA

#2101: Hematologia

Medicamentos

IPTACOPAN
HARD GELATIN CAPSULES
ORAL

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

A multicenter, single arm, open-label trial to evaluate efficacy and safety of oral, twice daily iptacopan in adult PNH patients who have Hb10 g/dL in response to anti-C5 antibody and switch to iptacopan

State	Type of participants	Age Ranges
Recruiting	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	50
Results	**Low level of intervention**	**Rare disease**
No results	No	No
Geographic coverage	**Areas of the study**	**Advanced therapy**
International multicenter	treatment, safety, effectiveness, pharmacokinetics, pharmacodynamics	NO

Information

Identifier
2022-502148-10-00 (CTIS)

Protocol Code
CLNP023C12303

Therapeutic area
Diseases [C] - Immune System Diseases [C20]|

Investigated Disease
Paroxysmal Nocturnal Hemoglobinuria (PNH)

Scientific Title
A multicenter, single arm, open-label trial to evaluate efficacy and safety of oral, twice daily iptacopan in adult PNH patients who have Hb10 g/dL in response to anti-C5 antibody and switch to iptacopan

Rationale

Not provided

Main Objective

The main question this trial is designed to answer: is iptacopan treatment equally effective to standard of care when switching treatment to iptacopan?

Primary Endpoints

Measuring change in hemoglobin levels before start of iptacopan treatment and mean of hemoglobin levels at Day 126 and Day 168

Temporary moments of secondary assessment

Not provided

Secondary Objective

Is iptacopan better in effectiveness than standard of care treatment

Secondary Endpoints

Measuring change in hemoglobin levels before start of iptacopan treatment and mean of hemoglobin levels at Day 126 and Day 168

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Signed informed consent must be obtained prior to participation in the study Male and female participants 18 years of age, at the time of ICF signatures and with a diagnosis of PNH confirmed by PNH clone size WBCs 10% based on historical data. Stable regimen (dose and intervals) of anti-C5 antibody treatment (either eculizumab or i.v. ravulizumab) for at least 6 months prior to screening Mean hemoglobin level 10 g/dL, documented by the mean of all available Hb assessments (minimum 2 measurements) over a period of 6 months before screening visit, collected at any laboratory. In addition to fulfill the Hb eligibility criterion, participants must have two different samples collected during the screening period and tested by the central laboratory with the mean >10 g/dL, prior to starting iptacopan.

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Exclusion criteria

Participation in any other investigational drug trial or use of other investigational drugs at the time of enrollment
Ongoing drug or alcohol abuse that could interfere with patient's participation in the trial.
Any medical condition deemed likely to interfere with the patient's participation in the study
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Unstable medical condition including, but not limited to, myocardial ischemia, active gastrointestinal bleeding, coexisting chronic anemia unrelated to PNH, or unstable thrombotic event not amenable to active treatment as judged by the investigator at Screening.
History of cancer of any part of the body within the past 5 years

Calendar

(Last Update: 28/05/2025)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
19/09/2023	27/10/2023	27/10/2023	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
Not aported	Not aported	Not aported	Not aported	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

not initialized (--/--/----)

Complexo Hospitalario Universitario De Santiago

Santiago De Compostela

CORUÑA

#2102: Hematologia

Closed (03/04/2025)

Hospital Clinic De Barcelona

Barcelona

BARCELONA

#2101: Hematologia

Medication

IPTACOPAN
HARD GELATIN CAPSULES
ORAL

-

Active Principles: N/A

Orphan **Experimental**

No results