Long-term safety and tolerability of iptacopan in patients with Paroxysmal Nocturnal Hemoglobinuria

Estado	**Tipo de Participantes**	**Rangos de Edad**
EC Finalizado	No aportado	Mayores de 64 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	107
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	tratamiento, seguridad, eficacia	NO

Información

Identificador
2023-509843-28-00 (CTIS)

Cod. Protocolo
CLNP023C12001B

Transicionado 2020-004385-19

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

Enfermedad investigada
Paroxysmal Nocturnal Hemoglobinuria (PNH)

Título Científico
An open label, multicenter roll-over extension program (REP) to characterize the long-term safety and tolerability of iptacopan (LNP023) in patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) who have completed PNH Phase 2 and Phase 3 studies with iptacopan.

Justificación

No aportado

Objetivo Principal

To evaluate the long-term safety and tolerability of iptacopan monotherapy in participants who have completed the treatment extension period (without tapering down) of the previous Phase II and any Phase III clinical studies with iptacopan

Variables de Evaluación Primaria

Safety evaluations including but not limited to adverse events/serious adverse events, safety laboratory parameters, vital signs, etc., through End of Study visit

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the clinical benefit of iptacopan in maintaining sustained hemoglobin levels 12 g/dL, in the absence of red blood cell transfusion
To evaluate the clinical benefit of iptacopan in maintaining transfusion avoidance (TA) defined as the proportion of participants who remain free from transfusions
To evaluate the clinical benefit of iptacopan by assessing the rates of breakthrough hemolysis (BTH) and of Major Adverse Vascular Events (MAVE)

Variables de Evaluación Secundaria

Response defined as maintaining sustained hemoglobin levels 12 g/dL in the absence of transfusions evaluated over yearly follow up intervals
Absence of administration of packed-red blood cell transfusions evaluated over yearly follow up intervals
Occurrences of breakthrough hemolysis and of Major Adverse Vascular Events
MAVE occurring evaluated over yearly follow up intervals

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

Male and female participants 18 years of age with a diagnosis of PNH who have completed the treatment extension period (without tapering down) of Phase II iptacopan studies (CLNP023X2204, CLNP023X2201), Period 4 of CLFGX2201 or completed any Phase III (eg. CLNP023C12302, CLNP023C12301, CLNP023C12303) clinical study at the time point of enrollment visit in this roll over extension study
Prior vaccinations against Neisseria meningitidis, Streptococcus pneumoniae and Haemophilus influenzae infections should be up to date (i.e., any boosters required administered according to local regulations).
Per investigators clinical judgement, the patient may benefit from continued treatment with iptacopan and has been clinically stable on iptacopan monotherapy for at least 3 months

Criterios de Exclusión

Any comorbidity or medical condition (including but not limited to any active systemic bacterial, viral or fungal infection or malignancy) that, in the opinion of the investigator, could put the subject at increased risk or potentially confound study data
History of recurrent invasive infections caused by encapsulated organisms, such as Neisseria meningitidis, Streptococcus pneumoniae or Haemophilus influenzae
Female participants who are pregnant or breastfeeding, or intending to conceive during the course of the study
Women of childbearing potential, defined as all women physiologically capable of becoming pregnant from menarche until becoming post-menopausal, unless they are using effective methods of contraception during dosing of investigational drug and for 1 week after stopping investigational drug. Women are considered post-menopausal if they have had 12 months of natural (spontaneous) amenorrhea with an appropriate clinical profile (e.g., hormonal profile confirming menopause and/or age-appropriate history of vasomotor symptoms).

Calendario

(Última actualización: 11/08/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
30/07/2021	18/05/2022	18/05/2022	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
17/10/2024	No aportado	No aportado	14/11/2025	No aportado

Promotor

Novartis Pharma AG Switzerland

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Contact Person

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Monetary support: N/A

Tipo de promotor: Comercial

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Centros

Cerrado (11/06/2026)

COMPLEXO HOSPITALARIO UNIVERSITARIO DE SANTIAGO

Santiago de Compostela

CORUÑA

Activo (18/05/2022)

HOSPITAL CLINIC DE BARCELONA

Barcelona

BARCELONA

Cerrado (30/06/2026)

HOSPITAL UNIVERSITARIO DONOSTIA

Donostia/San Sebastián

GUIPÚZCOA

Medicamentos

LNP023

HARD GELATIN CAPSULES

ORAL

-

Principios Activos: N/A

Huérfano **Experimental**

IPTACOPAN

HARD GELATIN CAPSULES

ORAL

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

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State	Type of participants	Age Ranges
Finished CT	Not provided	Older than 64 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	107
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	treatment, safety, effectiveness	NO

Information

Identifier
2023-509843-28-00 (CTIS)

Protocol Code
CLNP023C12001B

Transitioned 2020-004385-19

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

Investigated Disease
Paroxysmal Nocturnal Hemoglobinuria (PNH)

Scientific Title
An open label, multicenter roll-over extension program (REP) to characterize the long-term safety and tolerability of iptacopan (LNP023) in patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) who have completed PNH Phase 2 and Phase 3 studies with iptacopan.

Rationale

Not provided

Main Objective

To evaluate the long-term safety and tolerability of iptacopan monotherapy in participants who have completed the treatment extension period (without tapering down) of the previous Phase II and any Phase III clinical studies with iptacopan

Primary Endpoints

Safety evaluations including but not limited to adverse events/serious adverse events, safety laboratory parameters, vital signs, etc., through End of Study visit

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the clinical benefit of iptacopan in maintaining sustained hemoglobin levels 12 g/dL, in the absence of red blood cell transfusion
To evaluate the clinical benefit of iptacopan in maintaining transfusion avoidance (TA) defined as the proportion of participants who remain free from transfusions
To evaluate the clinical benefit of iptacopan by assessing the rates of breakthrough hemolysis (BTH) and of Major Adverse Vascular Events (MAVE)

Secondary Endpoints

Response defined as maintaining sustained hemoglobin levels 12 g/dL in the absence of transfusions evaluated over yearly follow up intervals
Absence of administration of packed-red blood cell transfusions evaluated over yearly follow up intervals
Occurrences of breakthrough hemolysis and of Major Adverse Vascular Events
MAVE occurring evaluated over yearly follow up intervals

Temporary moments of secondary assessment

Not provided

Inclusion criteria

Male and female participants 18 years of age with a diagnosis of PNH who have completed the treatment extension period (without tapering down) of Phase II iptacopan studies (CLNP023X2204, CLNP023X2201), Period 4 of CLFGX2201 or completed any Phase III (eg. CLNP023C12302, CLNP023C12301, CLNP023C12303) clinical study at the time point of enrollment visit in this roll over extension study
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Per investigators clinical judgement, the patient may benefit from continued treatment with iptacopan and has been clinically stable on iptacopan monotherapy for at least 3 months

Exclusion criteria

Any comorbidity or medical condition (including but not limited to any active systemic bacterial, viral or fungal infection or malignancy) that, in the opinion of the investigator, could put the subject at increased risk or potentially confound study data
History of recurrent invasive infections caused by encapsulated organisms, such as Neisseria meningitidis, Streptococcus pneumoniae or Haemophilus influenzae
Female participants who are pregnant or breastfeeding, or intending to conceive during the course of the study
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Calendar

(Last Update: 11/08/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
30/07/2021	18/05/2022	18/05/2022	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
17/10/2024	Not aported	Not aported	14/11/2025	Not aported

Sponsor

Novartis Pharma AG Switzerland

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Contact Person

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Monetary support: N/A

Sponsor type: Commercial

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Sites

Closed (11/06/2026)

COMPLEXO HOSPITALARIO UNIVERSITARIO DE SANTIAGO

Santiago de Compostela

CORUÑA

Active (18/05/2022)

HOSPITAL CLINIC DE BARCELONA

Barcelona

BARCELONA

Closed (30/06/2026)

HOSPITAL UNIVERSITARIO DONOSTIA

Donostia/San Sebastián

GUIPÚZCOA

Medication

LNP023

HARD GELATIN CAPSULES

ORAL

-

Active Principles: N/A

Orphan **Experimental**

IPTACOPAN

HARD GELATIN CAPSULES

ORAL

-

Active Principles: N/A

Orphan **Experimental**

No results