# 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: 14/10/2026)

| 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

**Lichtstrasse 35**

**Contact Person**

Novartis Pharma Arzneimittel GmbH

**+499112730**

**legal.representative@novartis.com**

**Monetary support: N/A**

**Tipo de promotor: Comercial**

# Ceim

### CEIm Hospital Clínico San Carlos

**C/ Doctor Martín Lagos, s/n - Ciudad Universitaria 28040 Madrid**

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# Centros

Cerrado (11/06/2026)

**COMPLEXO HOSPITALARIO UNIVERSITARIO DE SANTIAGO**

Santiago de Compostela

CORUÑA

Cerrado (05/08/2026)

**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

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

| 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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## 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
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# Calendar

(Last Update: 14/10/2026)

| 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

**Lichtstrasse 35**

**Contact Person**

Novartis Pharma Arzneimittel GmbH

**+499112730**

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**Monetary support: N/A**

**Sponsor type: Commercial**

# Ceim

### CEIm Hospital Clínico San Carlos

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# Sites

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Santiago de Compostela

CORUÑA

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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