# A Long term Safety and Efficacy Study of Danicopan as an Add-on Therapy to C5i in Patients with PNH.

| | | |
|---|---|---|
| **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 | 55 |
| **Resultados** | **Bajo nivel intervención** | **Enfermedad rara** |
| Sin resultados | No | Si |
| **Cobertura geográfica** | **Ámbitos del ensayo** | **Terapia avanzada** |
| Sin determinar | seguridad, eficacia, farmacocinética | NO |

## Información

### Identificador
2023-504867-18-00 (CTIS)

### Cod. Protocolo
ALXN2040-PNH-303

Transicionado 2021-004253-22

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

### Enfermedad investigada
Patients with Paroxysmal Nocturnal Hemoglobinuria

### Título Científico
A Long-term Extension (LTE) Study to Characterize the Safety and Efficacy of Danicopan as an Add-on Therapy to a Complement Component 5 Inhibitor (C5i) in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Previously Treated with Danicopan in an Alexion-sponsored Clinical Study.

## Justificación

No aportado

## Objetivo Principal

To characterize the long-term safety of treatment with danicopan as an add-on therapy to a complement component 5 inhibitor (C5i)

## Variables de Evaluación Primaria

Incidence of treatment-emergent adverse events (TEAEs) and serious TEAEs

## Momentos temporales de evaluación primaria

No aportado

## Objetivo Secundario

To characterize long-term efficacy of danicopan as an add-on therapy to a C5i
To characterize the long-term effect of treatment with danicopan as an add-on therapy to a C5i on Functional Assessment of Chronic Illness Therapy (FACIT) Fatigue scores and on European Organisation for Research and Treatment of Cancer Quality-of-life Questionnaire-Core 30 Scale (EORTC-QLQ-C30) scores
To further characterize the safety of danicopan as an add-on therapy to a C5i

## Variables de Evaluación Secundaria

Change in hemoglobin (Hgb) values over time
Proportion of patients with Hgb increase of 2g/dL in the absence of transfusion over time
Change in absolute reticulocyte count over time
Change in lactate dehydrogenase (LDH) over time
Proportion of patients with LDH 1.5 × upper limit of normal (ULN) over time
Proportion of patients with transfusion avoidance (TA), defined as patients who remain transfusion free and do not require transfusion as per protocol specified guidelines
Change in FACIT Fatigue scores over time
Change in EORTC-QLQ-C30 scores over time
Change in safety laboratory parameters over time
TEAEs leading to discontinuation

## Momentos temporales de evaluación secundaria

No aportado

## Criterios de Inclusión

All participants who completed their participation in an Alexion sponsored clinical study with danicopan as an add on to a C5i treatment
Documentation of vaccination for Neisseria meningitidis: All participants must be revaccinated as per national vaccination guidelines or local practice for vaccination use with complement inhibitors.

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## Criterios de Exclusión

Any medical condition (for example, cardiac, pulmonary, renal, oncologic, or psychiatric) that, in the opinion of the Investigator, might interfere with participation in the study, pose any added risk to the participant, or confound the assessment of the participant.
Female participants who are pregnant, breastfeeding, or intending to conceive during the course of the study.

# Calendario

(Última actualización: 27/03/2026)

| Autorización | Inicio de Ensayo | Inclusión Primer Paciente | Interrumpido | Reiniciado |
|---|---|---|---|---|
| 04/07/2022 | 04/05/2023 | 15/05/2023 | No aportado | No aportado |

| Fin de reclutamiento | Fin prematuro (España) | Fin prematuro (Global) | Fin del ensayo en España | Fin del ensayo global |
|---|---|---|---|---|
| 17/11/2023 | No aportado | No aportado | 29/09/2025 | No aportado |

# Promotor

### Alexion Pharmaceuticals Inc. United States

**121 Seaport Boulevard**

**Contact Person**

European Clinical Trial Information

**+33147100615**

**clinicaltrials.eu@alexion.com**

**Monetary support: N/A**

**Tipo de promotor: Comercial**

# Ceim

### CEIm Hospital Clinic de Barcelona

**Villarroel, 170 - Planta 0 - Puerta 6b 08036 Barcelona**

**ceic@clinic.cat**

**932275766**

# Centros

Cerrado (26/03/2026)

**HOSPITAL CLINIC DE BARCELONA**

Barcelona

BARCELONA

Hematology

Cerrado (26/03/2026)

**Hospital Clinic De Barcelona**

Barcelona

BARCELONA

Haematology Department

Cerrado (20/03/2026)

**Hospital Germans Trias I Pujol**

Badalona

BARCELONA

Haematology Department

Cerrado (11/03/2026)

**Hospital Universitario Puerta De Hierro De Majadahonda**

Majadahonda

MADRID

Haematology Department

Cerrado (11/03/2026)

**HOSPITAL UNIVERSITARIO PUERTA DE HIERRO MAJADAHONDA**

Majadahonda

MADRID

Hematology

Activo (17/04/2023)

**HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO**

Sevilla

SEVILLA

Hematology

Cerrado (20/03/2026)

**Institut Catala D'oncologia**

Badalona

BARCELONA

Hematology

Activo (17/04/2023)

**University Hospital Virgen Del Rocio S.L.**

Sevilla

SEVILLA

Haematology Department

# Medicamentos

### Soliris 300 mg concentrate for solution for infusion

SOLUTION FOR INFUSION

INTRAVENOUS USE

**Código ATC: L04AA25 - ECULIZUMAB**

**Principios Activos: N/A**

**Experimental**

### Ultomiris 300 mg/30 mL concentrate for solution for infusion

SOLUTION FOR INFUSION

INTRAVENOUS USE

**Código ATC: L04AA43 - RAVULIZUMAB**

**Principios Activos: N/A**

**Experimental**

### ALXN 2040

FILM-COATED TABLET

ORAL USE

-

**Principios Activos: N/A**

**Huérfano** **Experimental**

# Sin resultados

# A Long term Safety and Efficacy Study of Danicopan as an Add-on Therapy to C5i in Patients with PNH.

| | | |
|---|---|---|
| **State** | **Type of participants** | **Age Ranges** |
| Finished CT | Not provided | Older than 64 , Adults (18-64 years) |
| **Gender** | **Phases** | **Expected Participants** |
| Both | Phase III | 55 |
| **Results** | **Low level of intervention** | **Rare disease** |
| No results | No | Yes |
| **Geographic coverage** | **Areas of the study** | **Advanced therapy** |
| Undetermined | safety, effectiveness, pharmacokinetics | NO |

## Information

### Identifier
2023-504867-18-00 (CTIS)

### Protocol Code
ALXN2040-PNH-303

Transitioned 2021-004253-22

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

### Investigated Disease
Patients with Paroxysmal Nocturnal Hemoglobinuria

### Scientific Title
A Long-term Extension (LTE) Study to Characterize the Safety and Efficacy of Danicopan as an Add-on Therapy to a Complement Component 5 Inhibitor (C5i) in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Previously Treated with Danicopan in an Alexion-sponsored Clinical Study.

## Rationale

Not provided

## Main Objective

To characterize the long-term safety of treatment with danicopan as an add-on therapy to a complement component 5 inhibitor (C5i)

## Primary Endpoints

Incidence of treatment-emergent adverse events (TEAEs) and serious TEAEs

## Temporary moments of secondary assessment

Not provided

## Secondary Objective

To characterize long-term efficacy of danicopan as an add-on therapy to a C5i
To characterize the long-term effect of treatment with danicopan as an add-on therapy to a C5i on Functional Assessment of Chronic Illness Therapy (FACIT) Fatigue scores and on European Organisation for Research and Treatment of Cancer Quality-of-life Questionnaire-Core 30 Scale (EORTC-QLQ-C30) scores
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## Secondary Endpoints

Change in hemoglobin (Hgb) values over time
Proportion of patients with Hgb increase of 2g/dL in the absence of transfusion over time
Change in absolute reticulocyte count over time
Change in lactate dehydrogenase (LDH) over time
Proportion of patients with LDH 1.5 × upper limit of normal (ULN) over time
Proportion of patients with transfusion avoidance (TA), defined as patients who remain transfusion free and do not require transfusion as per protocol specified guidelines
Change in FACIT Fatigue scores over time
Change in EORTC-QLQ-C30 scores over time
Change in safety laboratory parameters over time
TEAEs leading to discontinuation

## Temporary moments of secondary assessment

Not provided

## Inclusion criteria

All participants who completed their participation in an Alexion sponsored clinical study with danicopan as an add on to a C5i treatment
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## Exclusion criteria

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# Calendar

(Last Update: 27/03/2026)

| Authorization | Start of Trial | First patient inclusion | Halted | Restarted |
|---|---|---|---|---|
| 04/07/2022 | 04/05/2023 | 15/05/2023 | Not aported | Not aported |

| End of recruitment | Premature end (Spain) | Premature End (Global) | Trial end (Spain) | Trial end (Global) |
|---|---|---|---|---|
| 17/11/2023 | Not aported | Not aported | 29/09/2025 | Not aported |

# Sponsor

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**Monetary support: N/A**

**Sponsor type: Commercial**

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# Sites

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Barcelona

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Hematology

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# Medication

### Soliris 300 mg concentrate for solution for infusion

SOLUTION FOR INFUSION

INTRAVENOUS USE

ATC code: L04AA25 - ECULIZUMAB

Active Principles: N/A

Experimental

### Ultomiris 300 mg/30 mL concentrate for solution for infusion

SOLUTION FOR INFUSION

INTRAVENOUS USE

ATC code: L04AA43 - RAVULIZUMAB

Active Principles: N/A

Experimental

### ALXN 2040

FILM-COATED TABLET

ORAL USE

-

Active Principles: N/A

Orphan

Experimental

# No results