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.

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: 28/09/2026)

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

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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

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Hematology

Cerrado (17/06/2026)

HOSPITAL UNIVERSITARIO VIRGEN DEL ROCIO

Sevilla

SEVILLA

Hematology

Cerrado (20/03/2026)

Institut Catala D'oncologia

Badalona

BARCELONA

Hematology

Cerrado (17/06/2026)

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
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Change in FACIT Fatigue scores over time
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Change in safety laboratory parameters over time
TEAEs leading to discontinuation

Temporary moments of secondary assessment

Not provided

Inclusion criteria

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Calendar

(Last Update: 28/09/2026)

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

Alexion Pharmaceuticals Inc. United States

121 Seaport Boulevard

Contact Person

European Clinical Trial Information

+33147100615

clinicaltrials.eu@alexion.com

Monetary support: N/A

Sponsor type: Commercial

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Sites

Closed (26/03/2026)

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SEVILLA

Haematology Department

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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