

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

Estado
EC Finalizado

Tipo de Participantes
No aportado

Rangos de Edad
Mayores de 64 , Adultos (18-64 años)

Género
Ambos

Fases
Fase III

Participantes esperados
55

Resultados
Sin resultados

Bajo nivel intervención
No

Enfermedad rara
Si

Cobertura geográfica
Sin determinar

Ámbitos del ensayo
seguridad, eficacia, farmacocinética

Terapia avanzada
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 $1.5 \times$ 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: 22/06/2026)

Autorización 04/07/2022	Inicio de Ensayo 04/05/2023	Inclusión Primer Paciente 15/05/2023	Interrumpido No aportado	Reiniciado No aportado
Fin de reclutamiento 17/11/2023	Fin prematuro (España) No aportado	Fin prematuro (Global) No aportado	Fin del ensayo en España 29/09/2025	Fin del ensayo global 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

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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 MADRID Hematology
Cerrado (17/06/2026)	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

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State
Finished CT

Type of participants
Not provided

Age Ranges
Older than 64 , Adults (18-64 years)

Gender
Both

Phases
Phase III

Expected Participants
55

Results
No results

Low level of intervention
No

Rare disease
Yes

Geographic coverage
Undetermined

Areas of the study
safety, effectiveness, pharmacokinetics

Advanced therapy
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
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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

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Exclusion criteria

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Female participants who are pregnant, breastfeeding, or intending to conceive during the course of the study.

Calendar

(Last Update: 22/06/2026)

Authorization 04/07/2022	Start of Trial 04/05/2023	First patient inclusion 15/05/2023	Halted Not aported	Restarted Not aported
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End of recruitment 17/11/2023	Premature end (Spain) Not aported	Premature End (Global) Not aported	Trial end (Spain) 29/09/2025	Trial end (Global) Not aported
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Sponsor

Alexion Pharmaceuticals Inc. United States

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Contact Person

European Clinical Trial Information

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clinicaltrials.eu@alexion.com

Monetary support: N/A

Sponsor type: Commercial

Ceim

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Sites

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Active (17/04/2023)	University Hospital Virgen Del Rocio S.L. Sevilla SEVILLA Haematology Department

Medication

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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