

Study of investigational pozelimab + cemdísiran in patients with paroxysmal nocturnal hemoglobinuria (PNH)



Primary Objective: evaluate the safety and efficacy of pozelimab and cemdísiran in a C5 inhibitor-controlled study in adult patients with PNH who are complement treatment-naïve or have not recently received complement inhibitors



PATIENTS WITH PNH (N≈190)

Who are complement treatment naïve or have not recently received complement inhibitors

OPEN LABEL INTERVENTION

Cohort A

R
1:1

**Pozelimab (IV and SC)
+ cemdísiran (SC)**

Ravulizumab (IV)

Cohort B

R
1:1

**Pozelimab (IV and SC)
+ cemdísiran (SC)**

Eculizumab (IV)

Primary Endpoints

Cohort A only: Percent change in LDH

Cohort B only: Transfusion avoidance^a and maintenance of adequate control of hemolysis^b

Key Secondary Endpoints

Cohort A only:

Maintenance of adequate control of hemolysis^b
Transfusion avoidance^a

Cohort B only:

Percent change in LDH

Cohort A and B:

Adequate control of hemolysis^b
Breakthrough hemolysis
Hemoglobin stabilization
Normalization of LDH
PF and GHS/QoL scores^c

Fatigue measured by FACIT-Fatigue Scale
Rate and units of RBC transfusion
Safety
Immunogenicity
Pharmacokinetics



FIND OUT MORE

Scan here to find out more about this study at <https://clinicaltrials.gov/ct2/show/NCT05133531>

This information is intended for investigators interested in open clinical trials.

The use of pozelimab + cemdísiran described herein is investigational and has not been evaluated by any regulatory authority.

^aDefined as no red blood cell transfusion per protocol. ^bLDH $\leq 1.5 \times$ ULN. ^cBased on the European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire (EORTC-QLQ-C30).

C5, complement protein 5; FACIT, Functional Assessment of Chronic Illness Therapy; GHS, global health status; IV, intravenous; LDH, lactate dehydrogenase; N, number of patients; PF, physical functioning; PNH, paroxysmal nocturnal hemoglobinuria; QoL, quality of life; R, randomized; RBC, red blood cell; SC, subcutaneous; ULN, upper limit of normal.

POZELIMAB

An Investigational C5 Monoclonal Antibody

Designed to bind C5 protein to inhibit complement-mediated hemolytic activity¹



CEMDISIRAN

An Investigational RNAi Therapeutic

Designed to reduce liver production of C5 to suppress circulating C5 levels²

SELECTED INCLUSION CRITERIA^a



Diagnosis of PNH confirmed by high-sensitivity flow cytometry testing with PNH granulocytes or monocytes



Active disease, as defined by the presence of ≥ 1 PNH-related signs or symptoms



LDH level $\geq 2 \times$ ULN at the screening visit



For more information, visit www.clinicaltrials.gov or please call +353 (0)61 533 400.
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^aInclusion/exclusion criteria include a summary of selected criteria. Please review the complete study design on clinicaltrials.gov for complete details.

C5, complement protein 5; LDH lactate dehydrogenase; PNH, paroxysmal nocturnal hemoglobinuria; RNAi, ribonucleic acid interference; ULN, upper limit of normal.

1. Latuszek A et al. *PLoS One*. 2020;15(5):e0231892. 2. Badri P et al. *Clin Pharmacokinet*. 2021;60(3):365–78.

Current per clinicaltrials.gov as of September 18, 2023.

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SELECTED EXCLUSION CRITERIA^a



Prior treatment with eculizumab within 3 months, ravulizumab within 6 months, or other complement inhibitors within 5 half-lives of the respective agent prior to screening



Planned use of any complement inhibitor therapy other than study drugs during the treatment period



Receipt of an organ transplant, history of bone marrow transplantation, or other hematologic transplant



Unable to take antibiotics for meningococcal prophylaxis if locally required or if necessary when vaccination is < 2 weeks from study treatment initiation



Not meeting meningococcal vaccination requirements at a minimum documentation of meningococcal vaccination within 5 years prior to screening visit



Any contraindication for receiving Neisseria meningitidis vaccination



Body weight < 40 kg at screening visit



Any active, ongoing infection or a recent infection requiring ongoing systemic treatment with antibiotics, antivirals, or antifungals within 2 weeks of screening



Documented history of active, uncontrolled, ongoing systemic autoimmune diseases