ACCESS-1 (NCT05133531) **PHASE 3 ENROLLING**

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

Primary Objective: evaluate the safety and efficacy of pozelimab and cemdisiran in a C5 inhibitor-controlled study in adult patients with PNH who are complement treatment-naive 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) + cemdisiran (SC)**
- **Ravulizumab (IV)**

Cohort B

R 1:1

- **Pozelimab (IV and SC) + cemdisiran (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 + cemdisiran described herein is investigational and has not been evaluated by any regulatory authority.

[a]Defined as no red blood cell transfusion per protocol. [b]LDH ≤1.5 × ULN. [c]Based 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.

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ACCESS-1 (NCT05133531) **PHASE 3 ENROLLING**

POZELIMAB

An Investigational C5 Monoclonal Antibody
Designed to bind C5 protein to inhibit complement-mediated hemolytic activity[1]

+

CEMDISIRAN

An Investigational RNAi Therapeutic
Designed to reduce liver production of C5 to suppress circulating C5 levels[2]

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 ≥2 × ULN at the screening visit

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

For more information, visit www.clinicaltrials.gov or please call 844 REGN-MID.
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[a]Inclusion/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, riboneucleic 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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