ForPatients
by Roche

Paroxysmal Nocturnal Hemoglobinuria

A Study Evaluating the Efficacy, Safety, Pharmacokinetics and Pharmacodynamics of Crovalimab in Participants With Paroxysmal Nocturnal Hemoglobinuria (PNH) Not Previously Treated With Complement Inhibition

Trial Status	Trial Runs In	Trial Identifier
Active, not recruiting	1 Country	NCT04654468 YO42311

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase III, Multicenter, Single Arm Study Evaluating the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Crovalimab in Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH) Not Previously Treated With Complement Inhibition

Trial Summary:

This study will enrol participants aged 12 years or older with a body weight >= 40 kilograms (kg) diagnosed with PNH who have not been previously treated with complement inhibitor therapy. Approximately 50 participants will be treated with Crovalimab for at least 24 weeks.

Hoffmann-La Roche
Sponsor

Phase 3
Phase

NCT04654468 YO42311
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#12 Years	No

Inclusion Criteria:

- Body weight >= 40 kg at screening.
- Willingness and ability to comply with all study visits and procedures.

- Documented diagnosis of PNH, confirmed by high sensitivity flow cytometry.
- LDH Levels >= 2x the ULN at screening.
- Participants who have at least four transfusions during 12 months prior to screening (documented in the medical record).
- Presence of one or more of the following PNH-related signs or symptoms within 3 months of screening.
- Vaccination against Neisseria meningitidis serotypes A, C, W, and Y < 3 years prior to initiation of study treatment (Day 1)
- Vaccination against Haemophilius influenzae type B and Streptococcus pneumonia according to national vaccination recommendations.
- For participants receiving other therapies (e.g., immunosuppressants, corticosteroids): stable dose for >= 28 days prior to screening and up to the first drug administration.
- Adequate hepatic and renal function.
- Women of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use contraception during the treatment period and for 46 weeks (approximately 10.5 months) after the final dose of crovalimab.
- Platelet count >=30,000 cubic millimeter (mm^3) at screening
- ANC > 500/µl at screening

Exclusion Criteria:

- Current or previous treatment with a complement inhibitor.
- History of allogeneic bone marrow transplantation.
- History of Neisseria meningitidis infection within 6 months prior to screening and up to first drug administration.
- Known or suspected immune or hereditary complement deficiency.
- Known HIV infection with CD4 count < 200 cells per microlitre (cells/µl) within 24 weeks prior to screening.
- Infection requiring hospitalization or treatment with intravenous (IV) antibiotics within 28 days prior to screening and up to the first drug administration, or oral antibiotics within 14 days prior to screening and up to the first drug administration.
- Active systemic bacterial, viral, or fungal infection within 14 days before first drug administration.
- Presence of fever (>= 38#C) within 7 days before the first drug administration.
- Splenectomy < 6 months before screening.
- History of malignancy within 5 years prior to screening and up to the first drug administration.
- Pregnant or intending to become pregnant during the study or within 46 weeks (10.5 months) after the final dose of study treatment.
- Participation in another interventional treatment study with an investigational agent or use of any experimental therapy within 28 days of screening or within 5 half-lives of that investigational product, whichever is greater.