Efficacy and Safety of Switching From Anti-C5 Antibody Treatment to Iptacopan Treatment in Study Participants With Atypical Hemolytic Uremic Syndrome (aHUS)

Last Update: Mar 19, 2025
A Multicenter, Single Arm, Open-label Study to Evaluate Efficacy and Safety of Switching From Anti-C5 Antibody Treatment to Iptacopan Treatment in Study Participants With aHUS
ClinicalTrials.gov Identifier:
NCT05935215
Novartis Reference Number:CLNP023F12302
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All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

The purpose of this Phase 3 study is to evaluate the efficacy and safety of iptacopan upon switching from anti-C5 antibody to iptacopan treatment in study participants with aHUS. The study is designed as a multicenter, single-arm, open label study to evaluate the efficacy and safety of iptacopan upon switching from anti-C5 antibody to iptacopan treatment in participants with aHUS. It consists of a screening period of up to 8 weeks followed by a 12-Month Core Treatment period and 12-Month Extension Treatment period.

The study will assess the effects of iptacopan on a range of efficacy assessments relevant to aHUS.

Condition
Atypical Hemolytic Uremic Syndrome
Phase
Phase3
Overall Status
Recruiting
Number of Participants
50
Start Date
Feb 28, 2024
Completion Date
Jul 19, 2029
Gender
All
Age(s)
18 Years - 100 Years (Adult, Older Adult)

Interventions

Drug

Iptacopan

Open Label

Eligibility Criteria

Inclusion Criteria:

* Male and female adult participants ≥ 18 years of age with diagnosis of aHUS for whom etiologies of other types of TMA and non-aHUS kidney disease have been excluded.
* Currently on the recommended weight-based dosage regimen of anti-C5 antibody treatment for at least 3 months prior to the screening visit.
* Clinical evidence of response to anti-C5 antibody treatment (in absence of PE/PI) for at least 3 months prior to entering the screening period as defined by:

1. Hematological normalization in platelet count ≥150 x 109/L and LDH below upper limit of normal \[ULN\], and
2. Stable or improving kidney function as defined by ≤15% increase in serum creatinine.
* Vaccination against Neisseria meningitidis and Streptococcus pneumoniae infections is required prior to the start of treatment with iptacopan.
* If not received previously or if a booster is required, vaccination against Haemophilus influenzae infection, should be given, if available and according to local regulations.

Exclusion Criteria:

* History of aHUS disease relapse while on anti-C5 antibody treatment.
* eGFR \< 30 ml/min/1.73m\^2
* Active infection or history of recurrent invasive infections caused by encapsulated bacteria, i.e., meningococcus, pneumococcus (eg., N. meningitidis, S. pneumoniae) or H. influenzae.
* Participants with sepsis or active systemic bacterial, viral (including COVID-19) or fungal infection within 14 days prior to study treatment administration.
* Kidney, bone marrow transplant (BMT)/hematopoietic stem cell transplant (HSCT), heart, lung, small bowel, pancreas, liver transplantation or any other cell or solid organ transplantation
* Female patients who are pregnant or breastfeeding, or intending to conceive during the course of the study
* Any medical condition deemed likely to interfere with the patient's participation in the study

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