

Efficacy and Safety of Iptacopan (LNP023) in Adult Patients With Atypical Hemolytic Uremic Syndrome Naive to Complement Inhibitor Therapy

Last Update: Feb 03, 2025

A Multicenter, Single-arm, Open Label Trial to Evaluate Efficacy and Safety of Oral, Twice Daily LNP023 in Adult aHUS Patients Who Are Naive to Complement Inhibitor Therapy

ClinicalTrials.gov Identifier:

[NCT04889430](#)

Novartis Reference Number:CLNP023F12301

[See if you Pre-qualify](#)

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 determine whether iptacopan (LNP023) is efficacious and safe for the treatment of aHUS in adult patients who are treatment naive to complement inhibitor therapy. The study is designed as a multicenter, single-arm, open label study to demonstrate the efficacy and safety of LNP023 (iptacopan) at a dose of 200 mg b.i.d. in adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody). The study will enroll approximately 50 participants and assess the effects of iptacopan on a range of efficacy assessments relevant to aHUS including hematological and kidney parameters, dialysis requirement, changes in chronic kidney disease (CKD) stage, as well as patient reported outcomes (PRO) for fatigue and quality of life.

Condition

Atypical Hemolytic Uremic Syndrome

Phase

Phase3

Overall Status

Recruiting

Number of Participants

50

Start Date

Jan 17, 2022

Completion Date

Mar 29, 2029

Gender

All

Age(s)

18 Years - 100 Years (Adult, Older Adult)

Interventions

Drug

Iptacopan

Iptacopan 200mg twice daily oral

Eligibility Criteria

Main Inclusion Criteria:

- * Adult patients with evidence of active thrombotic microangiopathy (TMA), including thrombocytopenia, evidence of hemolysis, and acute kidney injury
- * Vaccinations against *Neisseria meningitidis*, *Streptococcus pneumoniae* and *Haemophilus influenzae* infections are required prior to the start of study treatment. If the patient has not been previously vaccinated, or if a booster is required, vaccine should be given according to local regulations, at least 2 weeks prior to first study drug administration. If study treatment has to start earlier than 2 weeks post vaccination or before vaccination is given, prophylactic antibiotic treatment must be administered at the start of study treatment and for at least 2 weeks after vaccination

Main Exclusion Criteria:

- * Treatment with complement inhibitors, including anti-C5 antibody
- * ADAMTS13 deficiency ($<10\%$ activity or $<0.1\text{U/ml}$), and/or Shiga toxin-related hemolytic uremic syndrome (STx-HUS), and/or Positive direct Coombs test
- * Identified drug exposure-related HUS or HUS related to known genetic defects of cobalamin C metabolism or known diacylglycerol kinase ϵ (DGKE) mediated aHUS
- * Receiving PE/PI, for 14 days or longer, prior to the start of screening for the current TMA
- * Bone marrow transplantation (BMT)/hematopoietic stem cell transplantation (HSCT), heart, lung, small bowel, pancreas, or liver transplantation
- * Patients with sepsis or active severe systemic bacterial, viral (including COVID-19) or fungal infection, systemic infection which confounds an accurate diagnosis of aHUS or impedes the ability to manage the aHUS disease, active infection (or history of recurrent invasive infections) caused by encapsulated bacteria
- * Kidney disease suggestive of other disease than aHUS or of chronic kidney failure or family history of non-complement mediated genetic kidney disease
- * Liver disease or liver injury at screening
- * Systemic sclerosis (scleroderma), systemic lupus erythematosus (SLE), or antiphospholipid antibody positivity or syndrome
- * Chronic hemo- or peritoneal dialysis

Other protocol-defined inclusion/exclusion criteria may apply

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