

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

Status: RECRUITING

Eligibility Criteria

Age: 18 years to 100 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

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 ($\leq 10\%$ activity or ≤ 0.1 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

Conditions & Interventions

Interventions:

DRUG: Iptacopan

Conditions:

Atypical Hemolytic Uremic Syndrome

Keywords:

LNP023, iptacopan, aHUS, atypical hemolytic uremic syndrome, thrombotic microangiopathy

More Information

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Phase: PHASE3

IRB

Number:

System ID: NCT04889430

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