优睿科
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Data Sheet

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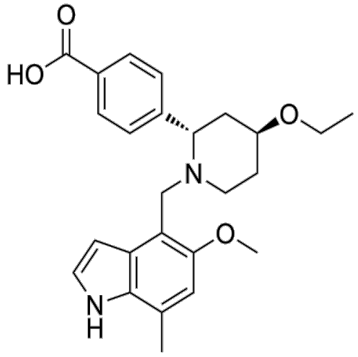
Global Supplier of Chemical Probes, Inhibitors & Agonists

Product Name :Iptacopan (Synonyms: LNP023)
Cat.No. :URK-V2532
CAS No. :1644670-37-0
Molecular Weight :422.52
Molecular Formula :$C_{25}H_{30}N_2O_4$
Target :
Solubility :



Biological Activity

Iptacopan, also known as Vebicorstat, is a novel oral inhibitor of factor B, a key component of the alternative complement pathway. It is indicated for the treatment of hematopoietic stem cell transplantation-associated thrombotic microangiopathy (HSCT-TMA) and is currently in late-stage clinical development.

Iptacopan works by blocking the activation of factor B and subsequent formation of the C3 convertase complex, which leads to the downstream effects of the alternative complement pathway. By inhibiting this pathway, Iptacopan has been shown to decrease the risk of thrombotic microangiopathy, a potentially life-threatening condition often see in patients undergoing hematopoietic stem cell transplantation.

Studies have shown promising results for the use of Iptacopan in the treatment of HSCT-TMA, with no significant adverse effects reported. In a phase 2 trial, Iptacopan treatment led to a complete response in 43% of patients with HSCT-TMA, compared to 0% in the placebo group. Additionally, Iptacopan has also been studied in other complement-mediated diseases such as paroxysmal nocturnal hemoglobinuria and C3 glomerulopathy.

References

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2. Hillmen P. Understanding complement mediated hemolysis and complement inhibition. Blood Rev. 2013;27(4):213-219.
3. Jodele S, Dandoy CE, Lane A, et al. Complement blockade for HSCT-associated TMA: lessons learned from a large cohort treated with eculizumab. Blood. 2020;135(12):1049-1057.
4. Cho BS, Yahng SA, Lee SE, et al. Efficacy of eculizumab and comparison of prognostic factors in patients with hematopoietic stem cell transplantation-associated thrombotic microangiopathy. Biol Blood Marrow Transplant. 2013;19(9):1268-1272.

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