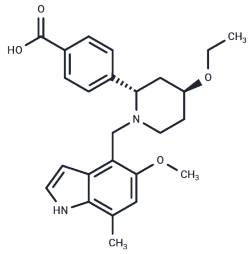
Data Sheet (Cat.No.T11864)

TargetMol

Iptacopan

Chemical Properties



CAS No. : 1644670-37-0

Formula: C25H30N2O4

Molecular Weight: 422.52

Storage: Powder: -20°C for 3 years | In solvent: -80°C for 1 year
Actual storage temperature shall be subject to the COA.

Biological Description

Description	Iptacopan (LNP023) is an inhibitor with high affinity to factor B, with a KD value of 7.9 nM and an IC50 value of 10 nM.
Targets(IC50)	Others,Complement System
In vitro	**METHODS**: Blood from three patients was analyzed in 2-14 replicates and Iptacopan was used at 1 μM. Hemolysis of PNH erythrocytes was determined by FACS analysis. **RESULTS** LNP023 can block C3 deposition on the surface of CD59-negative red blood cells. [2]
In vivo	**METHODS**: Iptacopan (LNP023) was tested in KRN-induced arthritis at doses of 20, 60 and 180 mg/kg by injection twice daily. **RESULTS** Comprehensive disease protection was observed at 60 and 180 mg/kg, and LNP023 significantly inhibited complement activation in joints at all doses, with significant reductions in Ba, C3d, and C5a levels. [1] **METHODS**: Sixty-eight patients with biopsy-proven C3G, decreased C3 (<77 mg/dl), proteinuria ≥1.0 g/g), and estimated glomerular filtration rate (eGFR) ≥30 ml/min per 1.73 m2 were recruited. Patients will be randomized 1:1 to receive iptacopan 200 mg twice daily or placebo for 6 months, followed by open-label treatment of all patients with iptacopan 200 mg twice daily for 6 months. **RESULTS** Iptacopan slows disease progression by inhibiting FB and preventing excessive activation of AP and may become one of the first targeted therapies available for C3G patients. [3]

Solubility Information

Solubility	DMSO: 255 mg/mL (603.52 mM),Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)
In vivo Formulation	10% DMSO+40% PEG300+5% Tween 80+45% Saline: 5 mg/mL (11.83 mM),Sonication is recommended. *Please add the solvents sequentially, clarifying the solution as much as possible before adding the next one. Dissolve by heating and/or sonication if necessary. Working solution is recommended to be prepared and used immediately. The formulation provided above is for reference purposes only. In vivo formulations may vary and should be modified based on specific experimental conditions.*

Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	2.3668 mL	11.8338 mL	23.6675 mL
5 mM	0.4734 mL	2.3668 mL	4.7335 mL
10 mM	0.2367 mL	1.1834 mL	2.3668 mL
50 mM	0.0473 mL	0.2367 mL	0.4734 mL

Please select the appropriate solvent to prepare the stock solution, according to the solubility of the product in different solvents. Please use it as soon as possible.

Note: The dilution table applies only to solid products. For liquid products, please calculate the stock solution based on the stated concentration and/or density.

Reference

Kavanagh D, et al. Design and Rationale of the APPELHUS Phase 3 Open-Label Study of Factor B Inhibitor Iptacopan for Atypical Hemolytic Uremic Syndrome. Kidney Int Rep. 2023 Apr 29;8(7):1332-1341.
Schubart A, et al. Small-molecule factor B inhibitor for the treatment of complement-mediated diseases. Proc Natl Acad Sci U S A. 2019 Apr 16;116(16):7926-7931.
Bomback AS,et al. Alternative Complement Pathway Inhibition With Iptacopan for the Treatment of C3 Glomerulopathy-Study Design of the APPEAR-C3G Trial. Kidney Int Rep. 2022 Aug 2;7(10):2150-2159.

Tel:781-999-4286 E_mail:info@targetmol.com Address:34 Washington Street,Wellesley Hills,MA 02481