

Givosiran

Chemical Properties

CAS No. : 1639325-43-1

Formula:

Molecular Weight: 16300.60

Keep away from direct sunlight

Storage: Powder: -20°C for 3 years | In solvent: -80°C for 1 year

Actual storage temperature shall be subject to the COA.

Givosiran

Biological Description

Description	Givosiran (ALN-AS1) is a small interfering RNA compound that selectively targets hepatic aminolevulinate synthase 1 (ALAS1) mRNA, leading to efficient downregulation of ALAS1 expression and consequent inhibition of neurotoxic δ -aminolevulinic acid and porphobilinogen accumulation. Givosiran provides a mechanism-driven research tool with significant relevance for studying metabolic dysregulation and therapeutic intervention strategies in acute intermittent porphyria.
Targets(IC50)	Others
In vitro	Givosiran is conjugated to a trivalent N-acetylgalactosamine ligand for specific binding to the asialoglycoprotein receptor, facilitating targeted hepatocyte delivery[2].
In vivo	In rat models, a single subcutaneous dose of Givosiran (10 mg/kg) reduced ALAS1 mRNA levels in both liver tissue and serum. In a phenobarbital-induced Acute Intermittent Porphyria (AIP) rat model, a regimen of 3 mg/kg (twice weekly for 3 weeks) prevented the elevation of ALAS1 and the accumulation of toxic metabolites. In T1/T2 AIP mice, a single dose of Givosiran (20 mg/kg) inhibited ALAS1 more rapidly than Hemin treatment. Studies in cynomolgus monkeys (1-10 mg/kg) showed a dose-dependent reduction of ALAS1 in serum and urine, with effects being reversible after an 8-week dosing period [2].

Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	0.0613 mL	0.3067 mL	0.6135 mL
5 mM	0.0123 mL	0.0613 mL	0.1227 mL
10 mM	0.0061 mL	0.0307 mL	0.0613 mL
50 mM	0.0012 mL	0.0061 mL	0.0123 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

Scott LJ. Givosiran: First Approval. *Drugs*. 2020 Feb;80(3):335-339.

Balwani M, et, al. Phase 3 Trial of RNAi Therapeutic Givosiran for Acute Intermittent Porphyria. *N Engl J Med*. 2020 Jun 11;382(24):2289-2301.

Chan A, et al. Preclinical Development of a Subcutaneous ALAS1 RNAi Therapeutic for Treatment of Hepatic Porphyrias Using Circulating RNA Quantification. *Mol Ther Nucleic Acids*. 2015 Nov 3;4(11):e263.

Inhibitor · Natural Compounds · Compound Libraries · Recombinant Proteins

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