

Eplontersen

Chemical Properties

CAS No. :	1637600-16-8
Formula:	
Molecular Weight:	8606.50
Storage:	Keep away from moisture -20°C for 1 year <small>Actual storage temperature shall be subject to the COA.</small>

Biological Description

Description	Eplontersen is a triantennary N-acetylgalactosamine-conjugated antisense oligonucleotide that selectively inhibits both wild-type and mutant transthyretin mRNA, thereby reducing pathogenic TTR protein production and amyloid fibril formation associated with ATTR amyloidosis affecting cardiac and peripheral nerve tissues.
Targets(IC50)	Others
In vivo	In in vivo studies using transgenic C57BL/6 mice expressing human transthyretin (hTTR), Eplontersen exhibits dose-dependent gene silencing activity. Following subcutaneous (s.c.) administration at doses of 0.6, 2, and 6 mg/kg (once weekly for 3 weeks), the substance results in a reduction of TTR expression. In the 6 mg/kg dose group, hepatic TTR mRNA levels were observed to decrease to 15% of baseline, while plasma TTR protein levels were reduced to 21% by day 17 [3].

Solubility Information

Solubility	H2O: 80.00 mg/mL (9.30 mM), Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)
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Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	0.1162 mL	0.581 mL	1.1619 mL
5 mM	0.0232 mL	0.1162 mL	0.2324 mL
10 mM	0.0116 mL	0.0581 mL	0.1162 mL
50 mM	0.0023 mL	0.0116 mL	0.0232 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

Aimo A, et al. RNA-targeting and gene editing therapies for transthyretin amyloidosis. *Nat Rev Cardiol.* 2022 Mar 23.

Diep JK, et al. Population pharmacokinetic/pharmacodynamic modelling of eplontersen, an antisense oligonucleotide in development for transthyretin amyloidosis. *Br J Clin Pharmacol.* 2022 Jul 22.

Prakash Thazha P, et al. Antisense oligonucleotides to hepatitis B virus RNA or transthyretin mRNA conjugated with N-acetylgalactosamine targeting moieties: World Intellectual Property Organization, WO2014179627[P]. 2014-11-06.

Inhibitor · Natural Compounds · Compound Libraries · Recombinant Proteins

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