

Nedosiran

Chemical Properties

CAS No. : 2266591-83-5

Formula:

Molecular Weight:

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

Nedosiran

Biological Description

Description	Nedosiran (DCR-PHXC) is a GalNAc-dsRNA conjugate designed for RNA interference-based silencing of lactate dehydrogenase (LDH), enabling selective hepatic targeting and reduction of oxalate production, Nedosiran is extensively studied as a potential therapeutic approach for patients with primary hyperoxaluria and end-stage renal disease to address metabolic dysregulation at the RNA level.
Targets(IC50)	Dehydrogenase
In vitro	Nedosiran (DCR-PHXC), an investigational therapy utilizing ribonucleic acid interference (RNAi) technology, is a synthetic, double-stranded RNA oligonucleotide, specifically a small interfering RNA (siRNA). It is engineered to selectively target and silence the mRNA responsible for encoding LDHA, offering a targeted approach to therapy.
In vivo	In animal models, Nedosiran is conjugated to an N-acetylgalactosamine (GalNAc3) ligand to ensure targeted delivery to the liver. This conjugation enables the compound to bind to the Asialoglycoprotein Receptor (ASGPR), which is highly expressed on the surface of hepatocytes. Following hepatic uptake, Nedosiran selectively suppresses the expression of hepatic LDHA. In these preclinical models, administration of the substance is associated with a reduction in plasma oxalate levels. This targeted approach is evaluated for its capacity to address the biochemical imbalances across PH1, PH2, and PH3 [1][3].

Reference

- Kevin Shee, et al. Nedosiran Dramatically Reduces Serum Oxalate in Dialysis-Dependent Primary Hyperoxaluria 1: A Compassionate Use Case Report. *Urology*. 2021 Oct;156:e147-e149.
- Gema Ariceta, et al. Hepatic Lactate Dehydrogenase A: An RNA Interference Target for the Treatment of All Known Types of Primary Hyperoxaluria. *Kidney Int Rep*. 2021 Feb 3;6(4):1088-1098.
- Thomas A Forbes, et al. Therapeutic RNA interference: A novel approach to the treatment of primary hyperoxaluria. *Br J Clin Pharmacol*. 2021 May 22.

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