

Nedosiran

Cat. No.:	HY-132606
CAS No.:	2266591-83-5
Molecular Weight:	20985
Target:	Small Interfering RNA (siRNA)
Pathway:	Epigenetics
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.

Nedosiran

BIOLOGICAL ACTIVITY

Description	Nedosiran (DCR-PHXC) is an RNA interference (RNAi) targeting lactate dehydrogenase (LDH). Nedosiran represents an impactful potential therapeutic for primary hyperoxaluria (PH) with end-stage renal disease (ESRD). Nedosiran is a GalNAc-dsRNA conjugate ^{[1][2][3]} .
In Vitro	Nedosiran (DCR-PHXC) is an investigational ribonucleic acid interference (RNAi) therapy ^[2] . Nedosiran is a synthetic, double-stranded RNA oligonucleotide (i.e., small interfering RNA [siRNA]) designed to target the mRNA encoding LDHA ^[2] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.
In Vivo	Nedosiran is a RNAi therapy for primary hyperoxaluria that selectively reduce hepatic expression of lactate dehydrogenase ^[3] . Nedosiran specially inhibits hepatic expression of lactate dehydrogenase (LDHA) ^[3] . Nedosiran is a double-strand siRNA molecule that is conjugated with GalNAc, which takes advantage of the aforementioned unique asialoglycoprotein receptor (ASGPR) delivery system in the liver. Nedosiran is administered by monthly subcutaneous injection. Nedosiran decreases plasma oxalate in animal models ^[3] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

- [1]. 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.
- [2]. 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.
- [3]. Thomas A Forbes, et al. Therapeutic RNA interference: A novel approach to the treatment of primary hyperoxaluria. *Br J Clin Pharmacol*. 2021 May 22.

Caution: Product has not been fully validated for medical applications. For research use only.

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