

Eplontersen sodium

Cat. No.:	HY-148089A
CAS No.:	2131025-75-5
Molecular Formula:	C ₂₉₆ H ₄₁₇ N ₇₇ Na ₂₀ O ₁₅₆ P ₂₀ S ₁₃
Molecular Weight:	9046.1
Target:	Transthyretin (TTR)
Pathway:	Neuronal Signaling
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.

Eplontersen (sodium)

BIOLOGICAL ACTIVITY

Description

Eplontersen sodium is a triantennary N-acetyl galactosamine (GalNAc₃-7a)-conjugated antisense oligonucleotide targeting transthyretin (TTR) mRNA to inhibit production of both variant and wild-type TTR protein. Misfolded TTR induces amyloid fibrils formation in the heart and peripheral nerves, leads to amyloid TTR (ATTR) amyloidosis diseases^{[1][2][3]}.

REFERENCES

- [1]. Aimo A, et al. RNA-targeting and gene editing therapies for transthyretin amyloidosis. *Nat Rev Cardiol.* 2022 Mar 23.
- [2]. 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.
- [3]. 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.

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

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