# Vutrisiran

**Cat. No.:** HY-132589

**CAS No.:** 1867157-35-4

**Sequence:** RNA, (Um-sp-(2'-deoxy-2'-fluoro)C-sp-Um-Um-Gm-(2'-deoxy-2'-fluoro)G-UmUm-(2'-deoxy-2'-fluoro)A-Cm-Am-Um-Gm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy2'-fluoro)A-Um-Cm-Cm-Cm-Am-sp-Um-sp-Cm), complex with RNA (Um-sp-Gm-sp-Gm-Gm-Am-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)A-(2'-deoxy-2'-fluoro)U-Gm-Um-Am-Am-Cm-Cm-Am-Am-GmAm) 3'-[[(2S,4R)-1-[29-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-14,14-bis[[3-[[3-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxopentyl]amino]propyl]amino]-3-oxopropoxy]methyl]-1,12,19,25-tetraoxo-16-oxa-13,20,24-triazanonacos-1-yl]-4-hydroxy-2-pyrrolidinyl]methyl hydrogen phosphate] (1:1)


**Target:** Transthyretin (TTR); Small Interfering RNA (siRNA)

**Pathway:** Neuronal Signaling; Epigenetics

**Storage:** 4°C, sealed storage, away from moisture
* In solvent : -80°C, 6 months; -20°C, 1 month (sealed storage, away from moisture)

## SOLVENT & SOLUBILITY

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| **In Vitro** | $H_2O$ : ≥ 20 mg/mL<br>* "≥" means soluble, but saturation unknown. |

## BIOLOGICAL ACTIVITY

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| **Description** | Vutrisiran (ALN-TTRsc02) is a liver-directed, investigational, small interfering ribonucleic acid (siRNA) agent. Vutrisiran can be used for transthyretin (TTR)-mediated amyloidosis research[1]. |
| **In Vitro** | Vutrisiran (previously ALN-TTRSC02) is a second-generation investigational RNAi therapeutic under development for the study of ATTR amyloidosis. Vutrisiran contains an siRNA that targets a sequence within the TTR mRNA which is conserved across wt and all known TTR variants. However, the Vutrisiran siRNA utilizes enhanced stabilization chemistry (ESC) and is conjugated to a triantennary GalNAc ligand, with the aim of enabling infrequent, subcutaneous (SC) dosing[1].<br>MCE has not independently confirmed the accuracy of these methods. They are for reference only. |
| **In Vivo** | In non-human primates, single SC doses of Vutrisiran 0.3 and 1 mg/kg achieved mean maximum TTR reductions (nadirs) of 55% and 96%, respectively, with serum TTR reductions persisting beyond 4 months for the 1 mg/kg dose. In the same study, monthly doses of 1 and 3 mg/kg maintained a reduction of TTR levels at 96%, relative to baseline[1].<br>MCE has not independently confirmed the accuracy of these methods. They are for reference only. |

## REFERENCES

[1]. Bahru A Habtemariam, et al. Single-Dose Pharmacokinetics and Pharmacodynamics of Transthyretin Targeting N-acetylgalactosamine-Small Interfering Ribonucleic Acid Conjugate, Vutrisiran, in Healthy Subjects. Clin Pharmacol Ther. 2021 Feb;109(2):372-382.

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

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