

Vutrisiran

Cat. No.:	HY-132589
CAS No.:	1867157-35-4
Target:	Others
Pathway:	Others
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.

Vutrisiran

BIOLOGICAL ACTIVITY

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] . 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] . 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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