

Vutrisiran

Chemical Properties

CAS No. : 1867157-35-4

Formula:

Molecular Weight:

Appearance: no data available

Storage: Powder: -20°C for 3 years | In solvent: -80°C for 1 year

Vutrisiran

Biological Description

Description	Vutrisiran (ALN-TTRsc02) is an investigational, liver-directed small interfering ribonucleic acid (siRNA) compound designed for research on transthyretin (TTR)-mediated amyloidosis.
In vitro	Vutrisiran (formerly ALN-TTRSC02) is being developed as a second-generation RNAi therapeutic for treating ATTR amyloidosis. It features an siRNA designed to target a common sequence in the TTR mRNA, relevant to both wild-type and all known TTR variants. Incorporating enhanced stabilization chemistry (ESC), and attached to a triantennary GalNAc ligand, Vutrisiran aims to facilitate less frequent, subcutaneous (SC) administration[1].
In vivo	In non-human primates, single subcutaneous (SC) doses of Vutrisiran at 0.3 and 1 mg/kg resulted in mean maximum transthyretin (TTR) reductions (nadirs) of 55% and 96% respectively, with the latter dose maintaining serum TTR reductions for over four months. Monthly administrations of 1 and 3 mg/kg consistently upheld TTR level reductions at 96% compared to baseline[1].

Reference

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

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