Data Sheet (Cat.No.T38656)

TargetMol

Nusinersen

Chemical Properties

CAS No. : 1258984-36-9

Formula:

Molecular Weight:

Nusinersen

Storage: Keep away from moisture
Powder: -20°C for 3 years | In solvent: -80°C for 1 year
Actual storage temperature shall be subject to the COA.

Biological Description

Description	Nusinersen (nusinersen) is an antisense oligonucleotide (ASO) for the treatment of pediatric and adult spinal muscular atrophy (SMA) that alters the way the SMN2 gene is processed and increases the production of SMN proteins, which are deficient in SMA patients.
Targets(IC50)	DNA/RNA Synthesis
In vivo	In the ENDEAR phase 3 clinical trial, intrathecal administration of Nusinersen in infants with spinal muscular atrophy significantly improved outcomes. A motor milestone response was observed in 51% of treated infants (vs. 0% in controls), with increased event-free survival (HR = 0.53) and overall survival (HR = 0.37)[1].In severe SMA mouse models, a single subcutaneous injection of Nusinersen (30μg) on postnatal day 1 significantly extended survival, improved motor neuron function, neuromuscular junction integrity, and motor behavior[2].

Reference

Richard S Finkel, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. N Engl J Med. 2017 Nov 2;377(18):1723-1732.

Laura Torres-Benito, et al. NCALD Antisense Oligonucleotide Therapy in Addition to Nusinersen further Ameliorates Spinal Muscular Atrophy in Mice. Am J Hum Genet. 2019 Jul 3;105(1):221-230.

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