**Proteins** 

# **Product** Data Sheet

Nusinersen

## **Nusinersen**

Cat. No.: HY-112980 1258984-36-9 CAS No.:

Molecular Weight: 7127.3

Sequence:

A-A-m5U-G-m5C-m5U-G-G)

Target: DNA/RNA Synthesis Pathway: Cell Cycle/DNA Damage

Storage: -20°C, stored under nitrogen, away from moisture

\* In solvent: -80°C, 6 months; -20°C, 1 month (stored under nitrogen, away from

moisture)

#### **SOLVENT & SOLUBILITY**

In		

H<sub>2</sub>O: 100 mg/mL (14.03 mM; ultrasonic and warming and heat to 60°C)

Preparing Stock Solutions	Solvent Mass Concentration	1 mg	5 mg	10 mg
	1 mM	0.1403 mL	0.7015 mL	1.4031 mL
	5 mM	0.0281 mL	0.1403 mL	0.2806 mL
	10 mM	0.0140 mL	0.0702 mL	0.1403 mL

Please refer to the solubility information to select the appropriate solvent.

In Vivo

1. Add each solvent one by one: PBS

Solubility: 50 mg/mL (7.02 mM); Clear solution; Need ultrasonic

### **BIOLOGICAL ACTIVITY**

Description	Nusinersen is an antisense oligonucleotide agent that modifies pre-messenger RNA splicing of the SMN2 gene and thus promotes increased production of full-length SMN protein <sup>[1]</sup> .
In Vitro	Nusinersen is an SMN-ASO that increases SMN levels by blocking an intronic splice silencer in SMN2, thereby facilitating the exon 7 inclusion and generation of FL-SMN2 transcripts <sup>[2]</sup> .  MCE has not independently confirmed the accuracy of these methods. They are for reference only.

#### **REFERENCES**

[1]. 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.

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2]. Laura Torres-Benito, et al. 2019 Jul 3;105(1):221-230.	NCALD Antisense Oligonucle	otide Therapy in Addition to Nus	inersen further Ameliorates Spinal Muscular Atro	ophy in Mice. Am J Hum Genet.
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