

## Nusinersen

<b>Cat. No.:</b>	HY-112980
<b>CAS No.:</b>	1258984-36-9
<b>Molecular Weight:</b>	7127.3
<b>Sequence:</b>	RNA, [2'-O-(2-methoxyethyl)](P-thio)(m5U-m5C-A-m5C-m5U-m5U-m5U-m5C-A-m5U-A-A-m5U-G-m5C-m5U-G-G)
<b>Target:</b>	DNA/RNA Synthesis
<b>Pathway:</b>	Cell Cycle/DNA Damage
<b>Storage:</b>	-20°C, stored under nitrogen, away from moisture * In solvent : -80°C, 6 months; -20°C, 1 month (stored under nitrogen, away from moisture)

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### SOLVENT & SOLUBILITY

<b>In Vivo</b>	1. Add each solvent one by one: PBS Solubility: 50 mg/mL (7.02 mM); Clear solution; Need ultrasonic
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### BIOLOGICAL ACTIVITY

<b>Description</b>	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> .
<b>In Vitro</b>	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.
- [2]. 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.

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

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