Viltolarsen sodium

Cat. No.: HY-132586A

Molecular Formula: $C_{244}H_{360}N_{113}Na_{21}O_{88}P_{20}$

Molecular Weight: 7386.42

Target: Nucleoside Antimetabolite/Analog

Pathway: Cell Cycle/DNA Damage

Storage: Please store the product under the recommended conditions in the Certificate of Analysis.

Viltolarsen (sodium)

BIOLOGICAL ACTIVITY

Description	Viltolarsen (NS-065/NCNP-01) sodium is a phosphorodiamidate morpholino antisense oligonucleotide. Viltolarsen sodium binds to exon 53 of the dystrophin mRNA precursor and restores the amino acid open-reading frame by skipping exon 53, resulting in the production of a shortened dystrophin protein that contains essential functional portions. Viltolarsen sodium has the potential for Duchenne muscular dystrophy (DMD) research[1][2].
In Vitro	Viltolarsen (NS-065/NCNP-01; 2 days) sodium induces exon 53 skipping in cells from a DMD model with a deletion of exons 45-52 with an EC_{50} value of 0.63 μM and in cells with a deletion of exons 48-52 with an EC_{50} value of 2.3 μM[2]. Viltolarsen (10 μM; 3 days) sodium causes the dystrophin protein expression in cells from a DMD model[2]. MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

[1]. Sohita Dhillon. Viltolarsen: First Approval. Drugs. 2020 Jul;80(10):1027-1031.

[2]. Naoki Watanabe, et al. NS-065/NCNP-01: An Antisense Oligonucleotide for Potential Treatment of Exon 53 Skipping in Duchenne Muscular Dystrophy. Mol Ther Nucleic Acids. 2018 Dec 7:13:442-449.

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