

Viltolarsen

Cat. No.:	HY-132586
CAS No.:	2055732-84-6
Molecular Weight:	6924.8
Target:	Others
Pathway:	Others
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)

Viltolarsen

SOLVENT & SOLUBILITY

In Vitro	H ₂ O : 100 mg/mL (14.44 mM); Need ultrasonic					
	Preparing Stock Solutions	Solvent Concentration	Mass	1 mg	5 mg	10 mg
			1 mM	0.1444 mL	0.7220 mL	1.4441 mL
			5 mM	0.0289 mL	0.1444 mL	0.2888 mL
			10 mM	0.0144 mL	0.0722 mL	0.1444 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 (7.22 mM); Clear solution; Need ultrasonic					

BIOLOGICAL ACTIVITY

Description	Viltolarsen (NS-065/NCNP-01), a phosphorodiamidate morpholino antisense oligonucleotide, targets the splicing of exon 53 in the dystrophin gene. Viltolarsen can be used for the research of the Duchenne muscular dystrophy (DMD) ^[1] .
In Vitro	Duchenne muscular dystrophy (DMD) is one of the most common lethal muscle-wasting disorders affecting young boys caused by mutations in the DMD gene. Exon skipping has emerged as a promising therapy for DMD. Antisense oligonucleotides (AONs) are designed to induce the skipping of exon(s), in order to restore the reading frame, and therefore, allow for dystrophin expression ^[1] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

[1]. Dzierlega K, et al. Optimization of antisense-mediated exon skipping for Duchenne muscular dystrophy. Gene Ther. 2020;27(9):407-416.

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

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