

Golodirsen

Cat. No.:	HY-132611
CAS No.:	1422959-91-8
Molecular Weight:	8647
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)

Golodirsen

SOLVENT & SOLUBILITY

In Vitro	H ₂ O : 100 mg/mL (11.56 mM; Need ultrasonic)					
	Preparing Stock Solutions	Solvent Concentration	Mass	1 mg	5 mg	10 mg
			1 mM	0.1156 mL	0.5782 mL	1.1565 mL
			5 mM	0.0231 mL	0.1156 mL	0.2313 mL
			10 mM	0.0116 mL	0.0578 mL	0.1156 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 (5.78 mM); Clear solution; Need ultrasonic					

BIOLOGICAL ACTIVITY

Description	Golodirsen (SRP-4053) is a phosphorodiamidate morpholino oligomer (PMO) that specifically targets exon 53 of dystrophin pre-mRNA. Golodirsen can be used for the research of Duchenne muscular dystrophy (DMD) ^{[1][2]} .
In Vitro	Golodirsen specifically targets exon 53 of dystrophin pre-mRNA, resulting in its exclusion from the final mRNA product. Skipping of exon 53 results in restoration of the mRNA reading frame and leads to the production of a partially internally deleted dystrophin protein with intact C and N-terminal regions ^[2] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

[1]. Heo YA. Golodirsen: First Approval. *Drugs*. 2020 Feb;80(3):329-333.

[2]. Scaglioni D, et, al. The administration of antisense oligonucleotide golodirsen reduces pathological regeneration in patients with Duchenne muscular dystrophy. Acta Neuropathol Commun. 2021 Jan 6;9(1):7.

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

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