

Drisapersen

Cat. No.:	HY-145724
CAS No.:	1251830-50-8
Molecular Weight:	6978
Sequence:	RNA, (P-thio)(Um-Cm-Am-Am-Gm-Gm-Am-Am-Gm-Am-Um-Gm-Gm-Cm-Am-Um-Um-Um-Cm-Um)
Target:	Others
Pathway:	Others
Storage:	-20°C, sealed storage, away from moisture * In solvent : -80°C, 6 months; -20°C, 1 month (sealed storage, away from moisture)

Drisapersen

SOLVENT & SOLUBILITY

In Vitro

H₂O : 100 mg/mL (14.33 mM; Need ultrasonic)

Concentration	Solvent	Mass		
		1 mg	5 mg	10 mg
Preparing Stock Solutions	1 mM	0.1433 mL	0.7165 mL	1.4331 mL
	5 mM	0.0287 mL	0.1433 mL	0.2866 mL
	10 mM	0.0143 mL	0.0717 mL	0.1433 mL

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

BIOLOGICAL ACTIVITY

Description

Drisapersen, a antisense oligonucleotide, induces exon 51 skipping during dystrophin pre-mRNA splicing and allows synthesis of partially functional dystrophin in Duchenne muscular dystrophy (DMD) patients with amenable mutations.

REFERENCES

- [1]. Flanigan KM, Voit T, Rosales XQ, et al. Pharmacokinetics and safety of single doses of drisapersen in non-ambulant subjects with Duchenne muscular dystrophy: results of a double-blind randomized clinical trial. *Neuromuscul Disord.* 2014;24(1):16-24.
- [2]. Voit T, Topaloglu H, Straub V, et al. Safety and efficacy of drisapersen for the treatment of Duchenne muscular dystrophy (DEMAND II): an exploratory, randomised, placebo-controlled phase 2 study. *Lancet Neurol.* 2014;13(10):987-996.

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

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