

Tofersen

Cat. No.:	HY-132580
CAS No.:	2088232-70-4
Molecular Weight:	7128
Sequence:	DNA, d([2'-O-(2-methoxyethyl)]m5rC-sp-[2'-O-(2-methoxyethyl)]rA-[2'-O-(2-methoxyethyl)]rG-sp-[2'-O-(2-methoxyethyl)]rG-[2'-O-(2-methoxyethyl)]rA-sp-Tsp-A-sp-m5C-sp-A-sp-T-sp-T-sp-T-sp-m5C-sp-T-sp-A-sp-[2'-O-(2-methoxyethyl)]m5rC-[2'-O-(2-methoxyethyl)]rA-sp-[2'-O-(2-methoxyethyl)]rG-[2'-O-(2-methoxyethyl)]m5rC-sp-[2'-O-(2-methoxyethyl)]m5rU)
Target:	DNA/RNA Synthesis
Pathway:	Cell Cycle/DNA Damage
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)

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SOLVENT & SOLUBILITY

In Vitro	H ₂ O : 100 mg/mL (14.03 mM); Need ultrasonic)				
		Solvent Concentration	Mass		
	Preparing Stock Solutions	1 mM	1 mg	5 mg	10 mg
		5 mM	0.1403 mL	0.7015 mL	1.4029 mL
		10 mM	0.0281 mL	0.1403 mL	0.2806 mL
	10 mM	0.0140 mL	0.0701 mL	0.1403 mL	
Please refer to the solubility information to select the appropriate solvent.					
In Vivo	1. Add each solvent one by one: PBS Solubility: 100 mg/mL (14.03 mM); Clear solution; Need ultrasonic				

BIOLOGICAL ACTIVITY

Description	Tofersen (BIIB067) is an antisense oligonucleotide that mediates RNase H-dependent degradation of superoxide dismutase 1 (SOD1) mRNA to reduce the synthesis of SOD1 protein. Tofersen can be used for the research of amyotrophic lateral sclerosis (ALS) ^[1] .
In Vitro	Tofersen targets the SOD1 mRNA using antisense oligonucleotides (ASOs) that bind to the SOD1 mRNA by Watson-Crick base pairing. Tofersen activates RNase H1 that destroys the targeted RNA ^[2] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

[1]. Miller T, et, al. Phase 1-2 Trial of Antisense Oligonucleotide Tofersen for SOD1 ALS. N Engl J Med. 2020 Jul 9;383(2):109-119.

[2]. McCampbell A, et, al. Antisense oligonucleotides extend survival and reverse decrement in muscle response in ALS models. J Clin Invest. 2018 Aug 1;128(8):3558-3567.

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

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