## Tofersen

Cat. No.: HY-132580	
<b>CAS No.:</b> 2088232-70-4	
Molecular Weight: 7128	
Target:         DNA/RNA Synthesis	Tofersen
Pathway: Cell Cycle/DNA Damage	101612611
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)	

## SOLVENT & SOLUBILITY

	Preparing Stock Solutions	Solvent Concentration	1 mg	5 mg	10 mg
		1 mM	0.1403 mL	0.7015 mL	1.4029 mL
		5 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.				

BIOLOGICAL ACTIV	
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) <sup>[1]</sup> .
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 <sup>[2]</sup> . 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.

Inhibitors

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**Screening Libraries** 

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Proteins



[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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