

Cat. No.: HY-124648 CAS No.: 1446311-56-3 Molecular Formula: $C_{24}H_{27}N_5O_2$ Molecular Weight: 417.5

Target: DNA/RNA Synthesis Pathway: Cell Cycle/DNA Damage

Powder -20°C Storage: 3 years

> 2 years In solvent -80°C 6 months

-20°C 1 month

Product Data Sheet

SOLVENT & SOLUBILITY

In Vitro

DMSO: 7.14 mg/mL (17.10 mM; ultrasonic and warming and heat to 80°C)

Preparing Stock Solutions	Solvent Mass Concentration	1 mg	5 mg	10 mg
	1 mM	2.3952 mL	11.9760 mL	23.9521 mL
	5 mM	0.4790 mL	2.3952 mL	4.7904 mL
	10 mM	0.2395 mL	1.1976 mL	2.3952 mL

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

BIOLOGICAL ACTIVITY

Description SMN-C2, an analog of RG-7916, is a selective modulator of SMN2 gene splicing that acts by binding SMN2 pre-mRNA, thereby increasing far upstream element binding protein 1 (FUBP1) and KH-spliced RNA binding Protein affinity regulator protein

(KHSRP) to the SMN2 pre-mRNA complex. SMN-C2 can be used in spinal muscular atrophy (SMA) research^[1].

In Vivo SMN-C2 (20 mg/kg, daily) can replace SMN2 splicing and divert FL mRNA, resulting in increased SMN protein levels in the

brain and spinal cord of mice^[2].

MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

[1]. Jingxin Wang, et al. Mechanistic studies of a small-molecule modulator of SMN2 splicing. Proc Natl Acad Sci U S A. 2018 May 15;115(20):E4604-E4612.

[2]. Nikolai A Naryshkin, et al. Motor neuron disease. SMN2 splicing modifiers improve motor function and longevity in mice with spinal muscular atrophy. Science. 2014

Aug 8;345(6197):688-93.

 $\label{lem:caution:Product} \textbf{Caution: Product has not been fully validated for medical applications. For research use only.}$

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