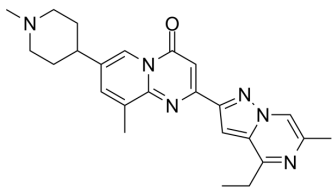
RG7800

Cat. No.:	HY-101792
CAS No.:	1449598-06-4
Molecular Formula:	$C_{24}H_{28}N_6O$
Molecular Weight:	416.52
Target:	DNA/RNA Synthesis
Pathway:	Cell Cycle/DNA Damage
Storage:	Powder -20°C 3 years 4°C 2 years In solvent -80°C 2 years -20°C 1 year



SOLVENT & SOLUBILITY

In Vitro

1M HCl : 100 mg/mL (240.08 mM; Need ultrasonic)
Ethanol : 2.5 mg/mL (6.00 mM; Need ultrasonic)
H_2O : 1.25 mg/mL (3.00 mM; ultrasonic and adjust pH to 3 with HCl)
H_2O : < 0.1 mg/mL (insoluble)

Preparing Stock Solutions

Solvent / Mass / Concentration	1 mg	5 mg	10 mg
1 mM	2.4008 mL	12.0042 mL	24.0085 mL
5 mM	0.4802 mL	2.4008 mL	4.8017 mL
10 mM	0.2401 mL	1.2004 mL	2.4008 mL

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

BIOLOGICAL ACTIVITY

Description

RG7800 is a SMN2 splicing modifier. RG7800 has the potential for spinal muscular atrophy treatment.

In Vitro

RG7800 increases the SMN protein level via induction of alternative splicing of the SMN2 mRNA. RG7800 is shown to promote the inclusion of exon 7 in SMN2 mRNA, generating full-length mRNA in vitro using fibroblasts from an SMA type I patient[1].
MCE has not independently confirmed the accuracy of these methods. They are for reference only.

In Vivo

RG7800 shows favorable drug metabolism and pharmacokinetic profile in the rat and in cynomolgus monkey with good oral bioavailability. In SMA mouse model, treatment of RG7800 shows a clear dose dependent increase in SMN protein levels. Mice treated with RG7800 demonstrate a dose dependent increase in survival beginning at the low dose (0.3/1 mg/kg). In the middle and high dose groups (1/3 and 3/10 mg/kg, respectively), approximately 80–90% survive beyond PND50/PND60 with profound body weight gain when the study is terminated. RG7800 dose-dependently corrects SMN2 splicing by including exon 7 to create FL mRNA, suggesting that RG7800 corrects alternative splicing of the human SMN2 gene in the brain of

transgenic SMA model mice, leading to an increase of the SMN protein in the brain[1].

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PROTOCOL

Animal Administration [1]

Mice: Compounds (RG7800) are administered orally once daily (qd) for 10 days at three different doses (1, 3, and 10 mg/kg). One hour after the final dose, tissues are collected from the mice, and the level of the SMN protein is determined in the brain and quadriceps muscle[1].

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

CUSTOMER VALIDATION

- Nature. 2021 Aug;596(7871):291-295.
- J Mol Med (Berl). 2019 Aug;97(8):1183-1193.
- Life Sci Alliance. 2019 Mar 25;2(2):e201800268.
- Patent. US20230340498A1.
- bioRxiv. 2020 Feb.

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REFERENCES

[1]. Ratni H, et al. Specific Correction of Alternative Survival Motor Neuron 2 Splicing by Small Molecules: Discovery of a Potential Novel Medicine To Treat Spinal Muscular Atrophy. J Med Chem. 2016 Jul 14;59(13):6086-100.

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