

RG7800

Chemical Properties

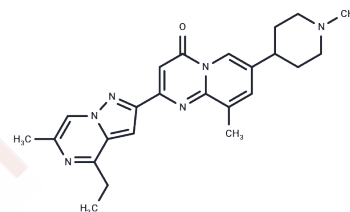
CAS No. : 1449598-06-4

Formula: C₂₄H₂₈N₆O

Molecular Weight: 416.52

Storage: Powder: -20°C for 3 years | In solvent: -80°C for 1 year

Actual storage temperature shall be subject to the COA.



Biological Description

Description	RG7800 (RO6885247) has the potential for spinal muscular atrophy treatment. RG7800 is an SMN2 splicing modifier.
Targets(IC50)	DNA/RNA Synthesis
In vitro	RG7800 has been demonstrated to facilitate the inclusion of exon 7 in SMN2 mRNA, thereby producing full-length mRNA in vitro from fibroblasts sourced from a patient with SMA type I. This compound enhances the level of SMN protein through the induction of alternative splicing in SMN2 mRNA.
In vivo	RG7800 displays favorable drug metabolism and pharmacokinetic profile in the rat and in cynomolgus monkey with good oral bioavailability. RG7800 dose-dependently corrects SMN2 splicing by including exon 7 to create FL mRNA, showing 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. The treatment of RG7800 displays a clear dose-dependent increase in SMN protein levels in the SMA mouse model. Mice treated with RG7800 demonstrate a dose-dependent increase in survival beginning at the low dose (0.3/1 mg/kg). Approximately 80-90% survive beyond PND50/PND60 with profound body weight gain when the study is terminated, in the middle and high dose groups (1/3 and 3/10 mg/kg, respectively) .

Solubility Information

Solubility	DMSO: 1.4 mg/mL (3.36 mM), Sonication and heating are recommended. H ₂ O: 1.25 mg/mL (3 mM), when pH is adjusted to 3 with HCl. Sonication is recommended. Ethanol: 2.5 mg/mL (6 mM), Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)
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Preparing Stock Solutions

	1mg	5mg	10mg
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
50 mM	0.048 mL	0.2401 mL	0.4802 mL

Please select the appropriate solvent to prepare the stock solution, according to the solubility of the product in different solvents. Please use it as soon as possible.

Note: The dilution table applies only to solid products. For liquid products, please calculate the stock solution based on the stated concentration and/or density.

Reference

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.

Inhibitor · Natural Compounds · Compound Libraries · Recombinant Proteins

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