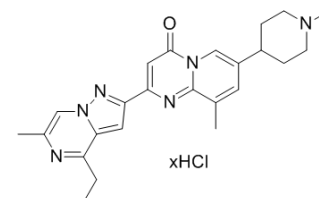


RG7800 hydrochloride

Cat. No.:	HY-101792A		
Molecular Formula:	C ₂₄ H ₂₈ N ₆ O.xHCl		
Target:	DNA/RNA Synthesis		
Pathway:	Cell Cycle/DNA Damage		
Storage:	Powder	-20°C	3 years
		4°C	2 years
	In solvent	-80°C	6 months
		-20°C	1 month



Solvent & Solubility

In Vitro	H ₂ O : ≥ 113.5 mg/mL
	* "≥" means soluble, but saturation unknown.

BIOLOGICAL ACTIVITY

Description	RG7800 hydrochloride is an orally active SMN2 splicing modulator, with EC _{1.5x5} of 23 nM and 87 nM for SMN2 splicing and SMN protein ; RG7800 hydrochloride has the potential to treat spinal muscular atrophy.
IC ₅₀ & Target	EC1.5x: 23 nM (SMN2 splicing), 87 nM (SMN protein) ^[1]
In Vitro	RG7800 hydrochloride is an orally active SMN2 splicing modulator, with EC _{1.5x5} of 23 nM and 87 nM for SMN2 splicing and SMN protein; and is the first small molecule SMN2 splicing modifier to enter human clinical trials ^[1] .
In Vivo	RG7800 is a favorable agent metabolism and pharmacokinetic (DMPK) profile in the rat and in cynomolgus monkey with good oral bioavailability. RG7800 (1, 3, 10 mg/kg, p.o.) dose-dependently elevates the SMN protein level in the brain and in peripheral tissue of Δ7 mice ^[1] .

PROTOCOL

Animal Administration ^[1]	<p>Mice^[1]</p> <p>Homozygous Δ7 mice are dosed ip with RG7800 or vehicle (100% DMSO, 2.5 mL/kg) once per day from PND3 through postnatal day 3 (PND23), and the dosing regimen is switched on PND24 to a 3-fold higher oral dose once daily in 0.5% HPMC and 0.1% Tween 80. Litters are randomized across groups. Body weight and survival are assessed daily. Survival analysis is done using GraphPad Prism (log-rank test), and a p <0.05 is considered as significant^[1].</p> <p>MCE has not independently confirmed the accuracy of these methods. They are for reference only.</p>
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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.

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

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