

RG7800 hydrochloride (1449598-06-4 free base)

Chemical Properties

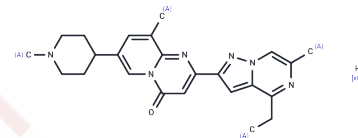
CAS No. :

Formula: C₂₄H₂₈N₆O.xHCl

Molecular Weight:

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 hydrochloride is an orally active modulator of SMN2 splicing.
Targets(IC50)	Others
In vitro	RG7800 hydrochloride is an orally active modulator of SMN2 splicing (EC1.5xs of 23 nM and 87 nM for SMN2 splicing and SMN protein). RG7800 hydrochloride is the first small molecule SMN2 splicing modifier to enter human clinical trials.
In vivo	RG7800 dose-dependently elevates the SMN protein level in the brain and in peripheral tissue of Δ7 mice. RG7800 is a favorable agent metabolism and pharmacokinetic (DMPK) profile in the rat and in cynomolgus monkey with good oral bioavailability.

Solubility Information

Solubility	H ₂ O: 113.5 mg/mL, Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)
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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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Tel: 781-999-4286 E_mail: info@targetmol.com Address: 34 Washington Street, Wellesley Hills, MA 02481