

## RG7800 tetrahydrochloride

## Chemical Properties

CAS No. :

Formula: C<sub>24</sub>H<sub>32</sub>Cl<sub>4</sub>N<sub>6</sub>O

Molecular Weight: 562.36

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 SMN2 splicing modulator with EC <sub>50</sub> values of 23 nM for splicing and 87 nM for SMN protein production, holding promise as a therapeutic agent for [spinal muscular atrophy].
Targets(IC <sub>50</sub> )	DNA/RNA Synthesis
In vitro	RG7800 hydrochloride is an orally active SMN2 splicing modulator with EC <sub>50</sub> s of 23 nM for SMN2 splicing and 87 nM for SMN protein production. It is the first small molecule SMN2 splicing modifier to advance into human clinical trials [1].
In vivo	RG7800 exhibits a favorable metabolism and pharmacokinetic (DMPK) profile in both rats and cynomolgus monkeys, paired with good oral bioavailability. Administered at doses of 1, 3, and 10 mg/kg orally, RG7800 dose-dependently increases SMN protein levels in both the central nervous system and peripheral tissues of Δ7 mice [1].

## Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	1.7782 mL	8.8911 mL	17.7822 mL
5 mM	0.3556 mL	1.7782 mL	3.5564 mL
10 mM	0.1778 mL	0.8891 mL	1.7782 mL
50 mM	0.0356 mL	0.1778 mL	0.3556 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.

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