RG7800 tetrahydrochloride

Cat. No.: HY-101792A

Molecular Formula: $C_{24}H_{32}Cl_4N_6O$

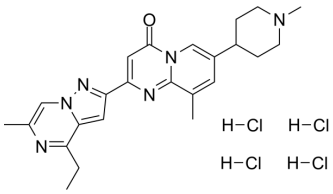
Molecular Weight: 562.36

Target: DNA/RNA Synthesis

Pathway: Cell Cycle/DNA Damage

Storage: 4°C, sealed storage, away from moisture

* In solvent : -80°C, 6 months; -20°C, 1 month (sealed storage, away from moisture)



SOLVENT & SOLUBILITY

In Vitro

H_2O : ≥ 113.5 mg/mL (201.83 mM)

* "≥" means soluble, but saturation unknown.

Preparing Stock Solutions

Solvent / Mass / Concentration	1 mg	5 mg	10 mg
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

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

BIOLOGICAL ACTIVITY

Description

RG7800 hydrochloride is an orally active SMN2 splicing modulator, with $EC_{1.5x}$s 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 $EC_{1.5x}$s 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].

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

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].

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

PROTOCOL

Animal Administration [1]	Mice[1] 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]. 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.

See more customer validations on www.MedChemExpress.com

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.

Tel: 609-228-6898 Fax: 609-228-5909 E-mail: tech@MedChemExpress.com
Address: 1 Deer Park Dr, Suite Q, Monmouth Junction, NJ 08852, USA