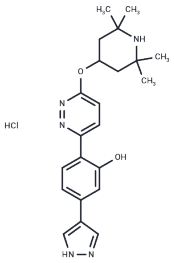
Data Sheet (Cat.No.T62381)

TargetMol

Branaplam hydrochloride

Chemical Properties



CAS No. : 1562338-39-9

Formula: C22H28ClN5O2

Molecular Weight: 429.95

Storage: Keep away from moisture,Keep away from direct sunlight
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	Branaplam hydrochloride (LMI070; NVS-SM1) is a selective and orally active SMN2 splicing modulator that acts directly on SMN with an EC50 value of 20 nM. Branaplam hydrochloride also inhibits hERG channels, demonstrating an IC50 of 6.3 μM. In a mouse model of severe spinal muscular atrophy (SMA), Branaplam hydrochloride significantly increased full-length SMN protein levels and markedly improved survival.
Targets(IC50)	Others,DNA/RNA Synthesis,Potassium Channel
In vitro	In Homo sapiens fibroblasts, Branaplam hydrochloride caused changes in the expression levels of 175 genes. [1]
In vivo	After oral administration of Branaplam hydrochloride (3, 10, 30 mg/kg), the SMN2-FL transcript and SMN protein levels in the brain and spinal cord of mice showed a dose-dependent increase [1].

Solubility Information

Solubility	H2O: < 1mg/mL (Insoluble) DMSO: 200 mg/mL (465.17 mM),Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)

Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	2.3259 mL	11.6293 mL	23.2585 mL
5 mM	0.4652 mL	2.3259 mL	4.6517 mL
10 mM	0.2326 mL	1.1629 mL	2.3259 mL
50 mM	0.0465 mL	0.2326 mL	0.4652 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

Palacino J, et al. SMN2 splice modulators enhance U1-pre-mRNA association and rescue SMA mice. Nat Chem Biol. 2015 Jul;11(7):511-517.

Cheung AK, et al. Discovery of Small Molecule Splicing Modulators of Survival Motor Neuron-2 (SMN2) for the Treatment of Spinal Muscular Atrophy (SMA). J Med Chem. 2018 Dec 27;61(24):11021-11036.