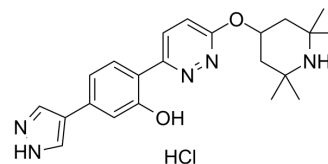


Branaplam hydrochloride

Cat. No.:	HY-19620A
CAS No.:	1562338-39-9
Molecular Formula:	C ₂₂ H ₂₈ ClN ₅ O ₂
Molecular Weight:	429.94
Target:	DNA/RNA Synthesis; Potassium Channel
Pathway:	Cell Cycle/DNA Damage; Membrane Transporter/Ion Channel
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

H₂O : 200 mg/mL (465.18 mM; Need ultrasonic)

Preparing Stock Solutions	Solvent		Mass		
	Concentration		1 mg	5 mg	10 mg
	1 mM		2.3259 mL	11.6295 mL	23.2591 mL
	5 mM		0.4652 mL	2.3259 mL	4.6518 mL
	10 mM		0.2326 mL	1.1630 mL	2.3259 mL

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

BIOLOGICAL ACTIVITY

Description

Branaplam (LMI070; NVS-SM1) hydrochloride is a highly potent, selective and orally active survival motor neuron-2 (SMN2) splicing modulator with an EC₅₀ of 20 nM for SMN. Branaplam hydrochloride inhibits human-ether-a-go-go-related gene (hERG) with an IC₅₀ of 6.3 μM. Branaplam hydrochloride elevates full-length SMN protein and extends survival in a severe spinal muscular atrophy (SMA) mouse model^{[1][2]}.

In Vitro

Branaplam (LMI070; NVS-SM1) hydrochloride treatment induces changes in the levels of 175 genes in human fibroblasts^[1]. MCE has not independently confirmed the accuracy of these methods. They are for reference only.

In Vivo

Branaplam (LMI070; NVS-SM1; 3, 10, 30 mg/kg; oral) hydrochloride produces dose-dependent elevations of SMN2-FL transcript and SMN protein in brain and spinal cord^[1].
 Branaplam (1 mg/kg of IV; 3 mg/kg of PO) hydrochloride has a CL of 25 mL/min/kg and an AUC of 3.03 μM·h^[2].
 A single Branaplam (oral; 30 mg/kg) hydrochloride results in significant and durable SMN protein elevation in brain for up to 160 hours in C/+ mice^[1].
 Branaplam (oral; 0.03, 0.1, 0.3, 1, 3 mg/kg) hydrochloride improves body weight and extends lifespan in n SMNΔ7 mice^[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.
- Nucleic Acids Res. 2021 Sep 7;49(15):8462-8470.
- bioRxiv. 2020 Jun.
- bioRxiv. 2020 Feb.

See more customer validations on www.MedChemExpress.com

REFERENCES

- [1]. 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.
- [2]. 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.
-

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

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