

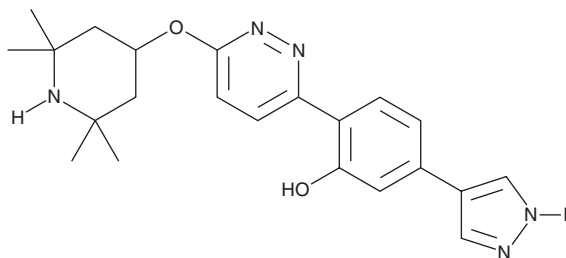
PRODUCT INFORMATION



LMI070

Item No. 26757

CAS Registry No.: 1562338-42-4
Formal Name: 5-(1H-pyrazol-4-yl)-2-[6-[(2,2,6,6-tetramethyl-4-piperidinyloxy]-3-pyridazinyl]-phenol
Synonyms: Branaplam, NVS-SM1
MF: C₂₂H₂₇N₅O₂
FW: 393.5
Purity: ≥98%
UV/Vis.: λ_{max}: 216, 282 nm
Supplied as: A crystalline solid
Storage: -20°C
Stability: ≥4 years



Information represents the product specifications. Batch specific analytical results are provided on each certificate of analysis.

Laboratory Procedures

LMI070 is supplied as a crystalline solid. A stock solution may be made by dissolving the LMI070 in the solvent of choice, which should be purged with an inert gas. LMI070 is soluble in organic solvents such as ethanol, DMSO, and dimethyl formamide (DMF). The solubility of LMI070 in ethanol is approximately 0.5 mg/ml and approximately 16 mg/ml in DMSO and DMF.

Further dilutions of the stock solution into aqueous buffers or isotonic saline should be made prior to performing biological experiments. Ensure that the residual amount of organic solvent is insignificant, since organic solvents may have physiological effects at low concentrations. Organic solvent-free aqueous solutions of LMI070 can be prepared by directly dissolving the crystalline solid in aqueous buffers. LMI070 is slightly soluble in PBS, pH 7.2. We do not recommend storing the aqueous solution for more than one day.

Description

LMI070 is an SMN2 splice modulator.¹ It selectively enhances SMN2 splicing by stabilizing the complex formed by the U1 small nuclear ribonucleic protein (snRNP) and SMN2 pre-mRNA. LMI070 (3-30 mg/kg) increases the expression of the full-length SMN2 transcript and, at doses ranging from 0.3 to 30 mg/kg, increases SMN protein levels in the brain and spinal cord in the C/+ mouse model of spinal muscular atrophy (SMA).^{1,2} It also increases survival of SMNΔ7 mice, a model of severe SMA, when administered at doses of 1 and 3 mg/kg.¹

References

1. Palacino, J., Swalley, S.E., Song, C., *et al.* SMN2 splice modulators enhance U1-pre-mRNA association and rescue SMA mice. *Nat. Chem. Biol.* **11**(7), 511-517 (2015).
2. Cheung, A.K., Hurley, B., Kerrigan, R., *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.* **61**(24), 11021-11036 (2018).

WARNING

THIS PRODUCT IS FOR RESEARCH ONLY - NOT FOR HUMAN OR VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

SAFETY DATA

This material should be considered hazardous until further information becomes available. Do not ingest, inhale, get in eyes, on skin, or on clothing. Wash thoroughly after handling. Before use, the user must review the complete Safety Data Sheet, which has been sent via email to your institution.

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