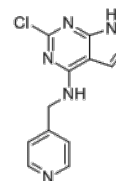


Product Name : BPN15477
Cat. No. : PC-49818
CAS No. : 1971086-99-3
Molecular Formula : C₁₂H₁₀ClN₅
Molecular Weight : 259.70
Target : Spliceosome
Solubility : 10 mM in DMSO



Biological Activity

BPN-15477 (BPN15477) is a potent splicing modulator compound that restores correct splicing of ELP1 exon 20, increases full-length ELP1 mRNA by increasing exon 20 inclusion with EC₅₀ of 1.9 μ M in luciferase splicing assays.

BPN-15477 is significantly more potent and efficacious than kinetin, and BPN-15477 modulates splicing selectively.

BPN-15477 corrects splicing of the ELP1 transcript, significantly increases the level of functional protein in vivo in all tissues, including brain.

BPN-15477 treatment increases LIPA and CFTR protein in disease-relevant cellular models.

BPN-15477 treatment (70 mg/Kg/day) improves exon 20 inclusion in the retina and brain of mice carrying the human FD transgene TgFD9.

References

Dadi Gao, et al. Nat Commun. 2021 Jun 7;12(1):3332.

Salani, M. et al. SLAS Discov.24, 57–67 (2018).

Morini E, et al. J Genet Genomics. 2022 Jul;49(7):654-665.

Anil Chekuri, et al. Hum Mol Genet. 2022 Jun 4;31(11):1776-1787.

Caution: Product has not been fully validated for medical applications. Lab Use Only!

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