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Data Sheet

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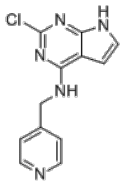
Product Name	:	BPN15477
Cat. No.	:	PC-49818
CAS No.	:	1971086-99-3
Molecular Formula	:	$C_{12}H_{10}ClN_5$
Molecular Weight	:	259.70
Target	:	Spliceosome
Solubility	:	10 mM in DMSO



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 uM 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

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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.

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