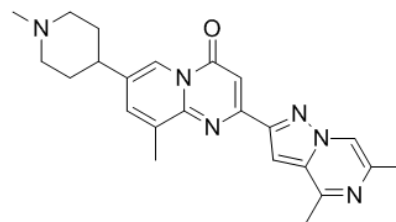


Product Name : RG7800
Cat. No. : PC-42423
CAS No. : 1449598-06-4
Molecular Formula : C₂₄H₂₈N₆O
Molecular Weight : 416.51872
Target : SMN2 Modulator
Solubility : 10 mM in DMSO



Biological Activity

RG7800 (RG-7800, RO6885247) is a potent, orally available, small molecule **SMN2 splicing modifier** with EC_{1.5X} of 23 nM and 87 nM for SMN2 splicing and SMN2 protein, respectively.

RG7800 specifically modifies the alternative splicing of SMN2 exon 7 in SMA patient-derived cells and in two SMA mouse models.

RG7800 exhibits excellent pharmacokinetic and in vivo efficacy and has a favorable safety profile.

References

Ratni H, et al. *J Med Chem.* 2016 Jul 14;59(13):6086-100.

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

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