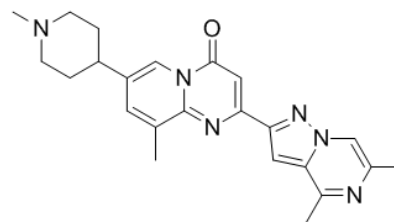


**Product Name** : RG7800  
**Cat. No.** : PC-42423  
**CAS No.** : 1449598-06-4  
**Molecular Formula** : C<sub>24</sub>H<sub>28</sub>N<sub>6</sub>O  
**Molecular Weight** : 416.51872  
**Target** : SMN2 Modulator  
**Solubility** : 10 mM in DMSO



## Biological Activity

RG7800 (RG-7800, RO6885247) is a potent, orally available, small molecule **SMN2 splicing modifier** with EC<sub>1.5X</sub> 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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