

## **Data Sheet**

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 Product Name
 :
 RG3039

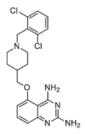
 Cat. No.
 :
 PC-47085

 CAS No.
 :
 1005504-62-0

 Molecular Formula
 :
 C<sub>21</sub>H<sub>23</sub>Cl<sub>2</sub>N<sub>5</sub>O

 Molecular Weight
 :
 432.35

Target : Other Targets
Solubility : 10 mM in DMSO



## **Biological Activity**

RG3039 (PF-06687859) is a potent, orally bioavailable and brain-penetrant inhibitor of the ribonucleic acid scavenger enzyme **DcpS** with IC50 of 3.4 nM.

RG3039 crosses the blood brain barrier and inhibits the RNA decapping enzyme DcpS in the CNS.

RG3039 showed a dose-dependent increase in survival, weight and motor function in SMA mice, with improved motor neuron somal and neuromuscular junction synaptic innervation and function and increased muscle size.

RG3039 also enhanced survival of conditional SMA mice in which SMN had been genetically restored to motor neurons.

## References

Van Meerbeke JP, et al. Hum Mol Genet. 2013 Oct 15;22(20):4074-83.

Gogliotti RG, et al. *Hum Mol Genet.* 2013 Oct 15;22(20):4084-101.

Calder AN, et al. *J Med Chem.* 2016 Nov 23;59(22):10067-10083.

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

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