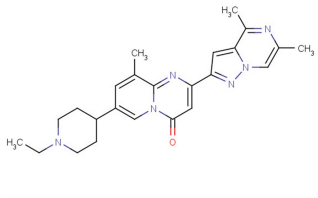
Data Sheet (Cat.No.T12935)

TargetMol
A DRUG SCREENING EXPERT

SMN-C3

Chemical Properties



CAS No.:	1449597-34-5
Formula:	C24H28N6O
Molecular Weight:	416.52
Appearance:	N/A
Storage:	0-4℃ for short term (days to weeks), or -20℃ for long term (months).

Biological Description

Description	SMN-C3 is an orally active modulator of SMN2 splicing, and has the potential to treat spinal muscular atrophy (SMA).
Targets(IC_{50})	SMN: None
In vivo	At P16, the vehicle-treated D7 mice were much smaller than the control group of heterozygous litter pups and were dying. In contrast, D7 mice treated with high doses of SMN-C3 showed a phenotype similar to the heterozygous control.In the D7 mice, SMN-C3 treatment induces a dose-dependent bodyweight gain , with some animals showing a body weight that is ~80% that of heterozygous controls. SMN-C3 normalizes the motor behavior of D7 mice, illustrated by the ability of the mice to right themselves as quickly as heterozygous controls and by their level of locomotor activity. Most importantly, whereas vehicle-treated mice die within 3 weeks after birth with a median survival of 18 days, SMN-C3 treatment increases survival in a dose-dependent manner to a median survival time of 28 days in the low-dose (0.3 mg/kg per day) group. In the two higher-dose groups (1 and 3 mg/kg per day), ~90% of animals survive beyond P65 when the study is completed.

Solubility Information

Solubility	DMSO: 5 mg/mL (12.00 mM) (< 1 mg/ml refers to the product slightly soluble or insoluble)

Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	2.401 mL	12.004 mL	24.008 mL
5 mM	0.48 mL	2.401 mL	4.802 mL
10 mM	0.24 mL	1.2 mL	2.401 mL
50 mM	0.048 mL	0.24 mL	0.48 mL

Please select the appropriate solvent to prepare the stock solution, according to the solubility of the product in different solvents. The storage conditions and period of the stock solution: - 80 ℃ for 6 months; - 20 ℃ for 1 month. Please use it as soon as possible.

Reference

1. Naryshkin NA, et al. Motor neuron disease. SMN2 splicing modifiers improve motor function and longevity in mice with spinal muscular atrophy. Science. 2014 Aug 8;345(6197):688-93.

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