

Product Data Sheet

Product Name: SMN-C3

Cat. No.: GC30185

Chemical Properties

Cas. No. 1449597-34-5

SMILES O=C1C=C(C2=NN3C(C(C)=NC(C)=C3)=C2)N=C4N1C=C(C5CCN(CC)CC5)C=C4C

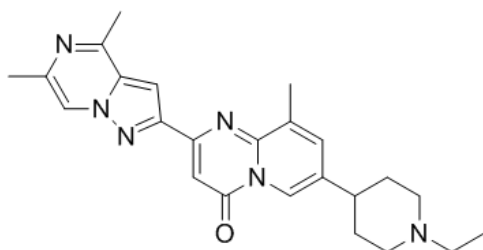
Formula C24H28N6O M.Wt 416.52

Solubility DMSO : 5 mg/mL (12.00 mM); Water : < 0.1 mg/mL (insoluble) Storage Store at -20°C

General tips For obtaining a higher solubility , please warm the tube at 37 °C and shake it in the ultrasonic bath for a while. Stock solution can be stored below -20°C for several months.

Shipping Condition Evaluation sample solution : ship with blue ice All other available size: ship with RT , or blue ice upon request.

Structure



Protocol

Caution: Product has not been fully validated for medical applications. For research use only.

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Animal experiment: Mice[1]The animals are treated with SMN-C3 at doses of 0.3, 1, and 3 mg/kg per day by intraperitoneal injections from P3 through P23 and thereafter at doses of 1, 3, and 10 mg/kg per day, respectively, by oral gavage[1].

References:

[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.

Background

SMN-C3 is an orally active SMN2 splicing modulator and has the potential to treat spinal muscular atrophy (SMA).

At P16, vehicle treated D7 mice are much smaller than heterozygous littermate controls and appear moribund. In contrast, D7 mice treated with the high dose of SMN-C3 show a phenotype similar to that of heterozygous controls. SMN-C3 treatment induces a dose-dependent bodyweight gain in the D7 mice, 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[1].

[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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