SMN-C3

Cat. No.: HY-112633

CAS No.: 1449597-34-5

Molecular Formula: $C_{24}H_{28}N_6O$

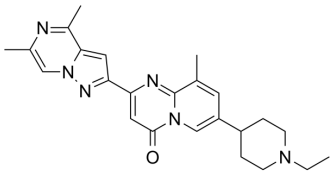
Molecular Weight: 416.52

Target: DNA/RNA Synthesis

Pathway: Cell Cycle/DNA Damage

Storage:

Powder	-20°C	3 years
	4°C	2 years
In solvent	-80°C	6 months
	-20°C	1 month



SOLVENT & SOLUBILITY

In Vitro

DMSO : 2.22 mg/mL (5.33 mM; ultrasonic and warming and heat to 60°C)

Preparing Stock Solutions	Solvent / Mass / Concentration	1 mg	5 mg	10 mg
	1 mM	2.4008 mL	12.0042 mL	24.0085 mL
	5 mM	0.4802 mL	2.4008 mL	4.8017 mL
	10 mM	---	---	---

Please refer to the solubility information to select the appropriate solvent.

BIOLOGICAL ACTIVITY

Description

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

IC_{50} & Target

SMN[1].

In Vivo

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].

MCE has not independently confirmed the accuracy of these methods. They are for reference only.

PROTOCOL

Animal Administration [1]	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]. MCE has not independently confirmed the accuracy of these methods. They are for reference only.

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.

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