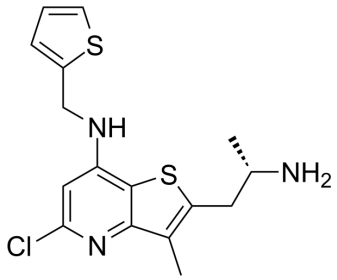
PTC258

Cat. No.:	HY-148772
CAS No.:	2476724-74-8
Molecular Formula:	$C_{16}H_{18}ClN_3S_2$
Molecular Weight:	351.92
Target:	Others
Pathway:	Others
Storage:	-20°C, protect from light, stored under nitrogen * In solvent : -80°C, 6 months; -20°C, 1 month (protect from light, stored under nitrogen)



SOLVENT & SOLUBILITY

In Vitro

DMSO : 100 mg/mL (284.16 mM; Need ultrasonic)

Preparing Stock Solutions	Mass / Solvent / Concentration	1 mg	5 mg	10 mg
	1 mM	2.8416 mL	14.2078 mL	28.4155 mL
	5 mM	0.5683 mL	2.8416 mL	5.6831 mL
	10 mM	0.2842 mL	1.4208 mL	2.8416 mL

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

BIOLOGICAL ACTIVITY

Description

PTC258 is a specific and orally active splicing modulator of Elongator complex protein 1 gene (ELP1). PTC258 increases the expression of ELP1 in vitro and in vivo. PTC258 is well tolerated in mouse model[1].

In Vitro

PTC258 (0.01 nM-0.01 μM; 48 h) efficiently increases full-length ELP1 mRNA and protein in Familial Dysautonomia (FD) patient fibroblasts[1].

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

In Vivo

PTC258 (3-24 mg/kg; p.o.; once daily for 3 months) is well tolerated. It corrects the splicing of the ELP1 transcript and significantly increases the amount of functional protein in vivo in all tissues tested, including the brain in mouse model[1].

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Animal Model:	Familial Dysautonomia (FD) Mouse model[1]
Dosage:	3 mg/kg, 6 mg/kg, 12 mg/kg, 24 mg/kg

Administration:	Oral gavage; once daily for 3 month
Result:	Increased full-length ELP1 transcript in a dose-dependent manner. And also, it increased in functional ELP1 protein in the brain, trigeminal, liver, and quadricep.

REFERENCES

[1]. Morini E, et al. Development of a novel oral treatment that rescues gait ataxia and retinal degeneration in a phenotypic mouse model of familial dysautonomia[J]. bioRxiv, 2022: 2022.11. 04.515198.

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