Web: www.taiclone.com
Tel: +886-2-2735-9682
Email: order@taiclone.com

Ataluren

Catalog No: tcsc0503

Available Sizes

Size: 10mg

Size: 50mg

Size: 100mg

Size: 200mg

Specifications

CAS No:
775304-57-9

Formula:
$C_{15}H_9FN_2O_3$

Pathway:
Membrane Transporter/Ion Channel

Target:
CFTR

Purity / Grade:
>98%

Solubility:
DMSO : ≥ 52 mg/mL (182.94 mM)

Alternative Names:
PTC124

Observed Molecular Weight:
284.24

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Product Description

Ataluren (PTC124) is an orally available **CFTR-G542X** nonsense allele inhibitor.

IC50 & Target: CFTR[1]

In Vitro: This premature “stop” signal (a class I mutation) prevents the cell from producing a full-length CFTR protein[1]. Ataluren (PTC124)-a new chemical entity that selectively induces ribosomal readthrough of premature but not normal termination codons[2].

In Vivo: Ataluren (PTC124) activity, optimized using nonsense-containing reporters, promotes dystrophin production in primary muscle cells from humans and *mdx* mice expressing dystrophin nonsense alleles, and rescues striated muscle function in *mdx* mice within 2-8 weeks of drug exposure. Ataluren (PTC124) is well tolerated in animals at plasma exposures substantially in excess of those required for nonsense suppression[2]. To induce nonsense suppression and increase PPT1 enzyme activity, the read-through drug Ataluren (PTC124) is given via intraperitoneal (i.p.) injection to male $Cln1^{R151X}$ mice at 2 months of age. These treatments are performed four times daily for 2 consecutive days in a proof-of-principle study. Used at 10 mg/kg, Ataluren (PTC124) increased PPT1 enzyme activity (P=0.0001 by unpaired t-test) and protein level (P=0.0014 by unpaired t-test) in the liver, but did not increase PPT1 enzyme activity or protein level in the cortex. This tissue-specific effect is likely due to the inability of Ataluren (PTC124) to breach the blood brain barrier (BBB), which decreased the bioavailability of Ataluren (PTC124) within the brain, and prevented Ataluren (PTC124) from reaching an efficacious concentration within the therapeutic window[3].