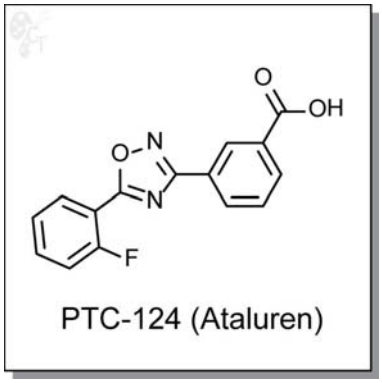
Cellagen Technology

Product Specification Sheet

Product Name: PTC-124 (Ataluren)

Catalog Number: C7124



PTC-124 (Ataluren)

Technical information:

Chemical Formula:	$C_{15}H_9FN_2O_3$
CAS #:	775304-57-9
Molecular Weight:	284.24
Purity:	> 98%
Appearance:	White solid
Solubility:	Soluble in DMSO up to 100 mM
Chemical Name:	3-(5-(2-fluorophenyl)-1,2,4-oxadiazol-3-yl)benzoic acid
Storage:	Store solid powder at 4°C desiccated; Store DMSO solution at -20°C.
Shelf Life:	In the unopened package, powder is stable for 1 year and DMSO solution is stable for 6 months under proper storage condition.

Handling:

- To make 10 mM stock solution, add 0.352mL of DMSO for each mg of PTC-124 (Ataluren).
- For DMSO solution, briefly spin the vial at 500 rpm in a 50 mL conical tube to ensure maximum sample recovery.

Biological Activity:

PTC-124 (Ataluren) is an oxadiazole-based, orally available agent deisgned to selectively promote ribosomal readhtrough of premature stop codons, but not normal termination codons. [1] The minimal concentration of PTC124 showing observable readthrough was 0.01 - 0.1 uM (2.8 - 28 ng/ml), while maximal activity was observed at 3 uM (852 ng/ml). [2]

PTC-124 has been studied extensively in the treatment of cystic fibrosis and has shown good tolerability and efficacy in preclinical and clinical settings. [3, 4]

Reference:

1. Sermet-Gaudelus et al., Ataluren (PTC124) induces cystic fibrosis transmembrane conductance regulator protein expression and activity in children with nonsense mutation cystic fibrosis. Am. J. Respir. Crit. Care Med. 2010, 182, 1262-1272. Pubmed ID: 20622033
2. Welch et al., PTC124 targets genetic disorders caused by nonsense mutations. Nature, 2007, 447, 87-91. Pubmed ID: 17450125
3. Du et al., PTC124 is an orally bioavailable compound that promotes suppression of the human CFTR-G542X nonsense allele in a CF mouse model. Proc. Natl. Acad. Sci. 2008, 105(6), 2064-2069 Pubmed ID: 18272502
4. Wilschanski et al., Chronic ataluren (PTC124) treatment of nonsense mutation cystic fibrosis. Eur. Respir. J. 2011, 38, 59-69. Pubmed ID: 21233271

To reorder: http://www.cellagentech.com/PTC-124-Ataluren/

For Technical Support: technical@cellagentech.com

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