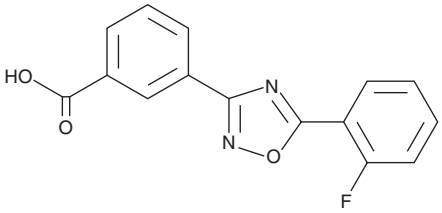
PRODUCT INFORMATION

PTC-124

Item No. 16758

CAS Registry No.:	775304-57-9
Formal Name:	3-[5-(2-fluorophenyl)-1,2,4-oxadiazol-3-yl]-benzoic acid
Synonym:	Ataluren
MF:	$C_{15}H_9FN_2O_3$
FW:	284.3
Purity:	≥98%
UV/Vis.:	λ_{max}: 242 nm
Supplied as:	A crystalline solid
Storage:	-20°C
Stability:	≥4 years



Information represents the product specifications. Batch specific analytical results are provided on each certificate of analysis.

Laboratory Procedures

PTC-124 is supplied as a crystalline solid. A stock solution may be made by dissolving the PTC-124 in the solvent of choice, which should be purged with an inert gas. PTC-124 is soluble in organic solvents such as DMSO and dimethyl formamide. The solubility of PTC-124 in these solvents is approximately 30 mg/ml.

PTC-124 is sparingly soluble in aqueous buffers. For maximum solubility in aqueous buffers, PTC-124 should first be dissolved in DMSO and then diluted with the aqueous buffer of choice. PTC-124 has a solubility of approximately 0.5 mg/ml in a 1:1 solution of DMSO:PBS (pH 7.2) using this method. We do not recommend storing the aqueous solution for more than one day.

Description

Nonsense mutations create a premature termination of mRNA translation and have been implicated in various genetic disorders, including muscular dystrophy and cystic fibrosis.[1] PTC-124 is a nonaminoglycoside that has been reported to selectively induce ribosomes to read through premature nonsense stop signals on mRNA, thus allowing the production of full length, functional proteins.[1] In a mouse model of cystic fibrosis caused by nonsense mutations, PTC-124 treatment (60 mg/kg s.c. injection or 0.3-0.9 mg/ml orally) has been shown to restore cystic fibrosis transmembrane conductance regulator (CFTR) protein expression and function.[2] The target activity of PTC-124 was initially evaluated by firefly luciferase reporter cell-based nonsense codon assay (IC_{50} = 7 nM); however, subsequent assessments using a *Renilla reniformis* luciferase reporter have failed to produce nonsense codon suppression activity.[3] Thus, while PTC-124 is in clinical testing in patients with nonsense mutations within the *CFTR* or dystrophin genes, controversy surrounds its exact mechanism of action.[1,3-5]

References

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2. Du, M., Liu, X., Welch, E.M., *et al. Proc. Natl. Acad. Sci. USA* **105(6)**, 2064-2069 (2008).
3. Auld, D.S., Thorne, N., Maguire, W.F., *et al. Proc. Natl. Acad. Sci. USA* **106(9)**, 3585-3590 (2009).
4. Wilschanski, M., Miller, L.L., Shoseyov, D., *et al. Eur. Respir. J.* **38(1)**, 59-69 (2011).
5. McElroy, S.P., Nomura, T., Torrie, L.S., *et al. PLoS Biol.* **11(6)**, 1-8 (2013).

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