

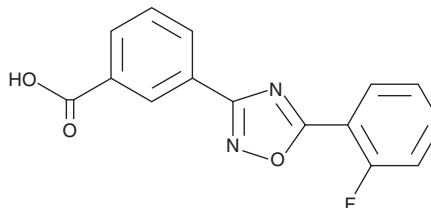
# PRODUCT INFORMATION



## 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<sub>15</sub>H<sub>9</sub>FN<sub>2</sub>O<sub>3</sub>  
**FW:** 284.3  
**Purity:** ≥98%  
**UV/Vis.:** λ<sub>max</sub>: 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.<sup>1</sup> 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.<sup>1</sup> 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.<sup>2</sup> The target activity of PTC-124 was initially evaluated by firefly luciferase reporter cell-based nonsense codon assay (IC<sub>50</sub> = 7 nM); however, subsequent assessments using a *Renilla reniformis* luciferase reporter have failed to produce nonsense codon suppression activity.<sup>3</sup> 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.<sup>1,3-5</sup>

### References

1. Finkel, R.S. *J. Child Neurol.* **25(9)**, 1158-1164 (2010).
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).

#### WARNING

THIS PRODUCT IS FOR RESEARCH ONLY - NOT FOR HUMAN OR VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

#### SAFETY DATA

This material should be considered hazardous until further information becomes available. Do not ingest, inhale, get in eyes, on skin, or on clothing. Wash thoroughly after handling. Before use, the user must review the complete Safety Data Sheet, which has been sent via email to your institution.

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