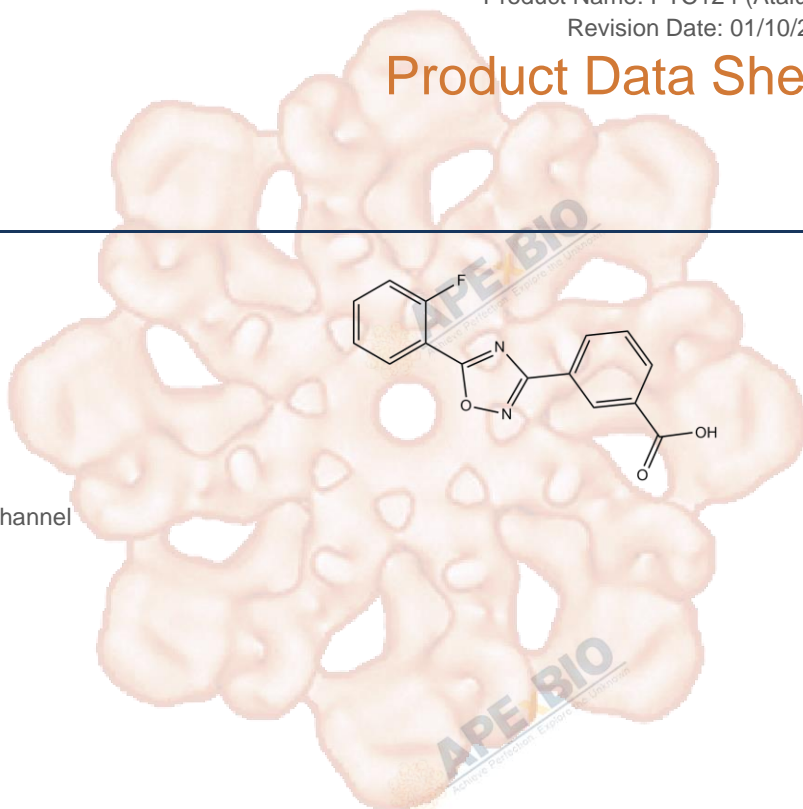


Product Data Sheet

PTC124 (Ataluren)

| | |
|------------------|---------------------------------------------------------------|
| Cat. No.: | A8553 |
| CAS No.: | 775304-57-9 |
| Formula: | C ₁₅ H ₉ FN ₂ O ₃ |
| M.Wt: | 284.24 |
| Synonyms: | PTC 124;PTC-124 |
| Target: | Membrane Transporter/Ion Channel |
| Pathway: | CFTR |
| Storage: | Store at -20°C |



Solvent & Solubility

insoluble in H₂O; ≥10.6 mg/mL in DMSO; ≥2.37 mg/mL in EtOH with gentle warming

In Vitro

| Preparing Stock Solutions | Solvent | Mass | | |
|---------------------------|----------------------|-----------|------------|------------|
| | | 1mg | 5mg | 10mg |
| | Concentration | | | |
| | 1 mM | 3.5182 mL | 17.5908 mL | 35.1815 mL |
| | 5 mM | 0.7036 mL | 3.5182 mL | 7.0363 mL |
| | 10 mM | 0.3518 mL | 1.7591 mL | 3.5182 mL |

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

Biological Activity

Shortsummary

CFTR-G542X nonsense allele inhibitor

IC₅₀ & Target

In Vitro

Cell Viability Assay

| | |
|----------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Cell Line: | HEK293 cells |
| Preparation method: | The solubility of this compound in DMSO is >10 mM. General tips for obtaining a higher concentration: Please warm the tube at 37 °C for 10 minutes and/or shake it in the ultrasonic bath for a while. Stock solution can be stored below -20°C for several months. |
| Reacting conditions: | 3 μM, 16 hours |

| | | |
|--------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| | Applications: | Cultured HEK293 cells harbouring UAA, UAG or UGA LUC-190 nonsense alleles were treated with increasing concentrations of PTC124 for 16 h, and assayed for luciferase activity. PTC124 promoted dose-dependent readthrough of all three nonsense codons. Levels of suppression correlated inversely with established termination efficiencies, with the highest readthrough at UGA, followed by UAG and then UAA. The minimal concentration of PTC124 showing discernable readthrough was 0.01–0.1 μ M, whereas the concentration promoting maximal activity was approximately 3 μ M. |
| In Vivo | Animal experiment | |
| | Animal models: | Cftr-/- hCFTR-G542X Mice |
| | Dosage form: | Subcutaneous injection, 60, 30, or 15 mg/kg body weight for 14–21 days |
| | Applications: | After the treatment, the mice were killed and intestinal tissues were harvested for immunofluorescence staining to determine whether hCFTR protein could be detected. No hCFTR protein was detected in intestinal tissues from untreated mice with hCFTR-specific antiserum. However, strong hCFTR staining was observed at the apical surface of epithelial cells in submucosal glands from mice treated with 60 mg/kg PTC124. Much weaker staining was detected in submucosal glands from mice treated with 30 mg/kg PTC124, whereas no signal could be detected in mice treated with 15 mg/kg PTC124. These results indicate that PTC124 can suppress the G542X mutation and partially restore hCFTR protein expression in Cftr-/- hCFTR-G542X mice. |
| Other notes: | Please test the solubility of all compounds indoor, and the actual solubility may slightly differ with the theoretical value. This is caused by an experimental system error and it is normal. | |

Product Citations

See more customer validations on www.apexbt.com.

References

- [1] Welch E M, Barton E R, Zhuo J, et al. PTC124 targets genetic disorders caused by nonsense mutations. *Nature*, 2007, 447(7140): 87-91.
- [2] Du M, Liu X, Welch E M, et al. PTC124 is an orally bioavailable compound that promotes suppression of the human CFTR-G542X nonsense allele in a CF mouse model. *Proceedings of the National Academy of Sciences*, 2008, 105(6): 2064-2069.

Caution

FOR RESEARCH PURPOSES ONLY.

NOT FOR HUMAN, VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

Specific storage and handling information for each product is indicated on the product datasheet. Most APExBIO products are stable under the recommended conditions. Products are sometimes shipped at a temperature that differs from the recommended storage temperature. Shortterm storage of many products are stable in the short-term at temperatures that differ from that required for long-term storage. We ensure that the product is shipped under conditions that will maintain the quality of the reagents. Upon receipt of the product, follow the storage recommendations on the product data sheet.

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