

---

HTA - - Health Technology Assessment Report

## **Ataluren (Translarna) for CFTR class I nonsense mutation mediated cystic fibrosis (Structured abstract)**

**Code:** HTA-32016000391 **Year:** 2015 **Date:** 2015

**Author:** NIHR HSRIC

### **Participants**

cystic fibrosis transmembrane conductance regulator (CFTR) class I nonsense mutation mediated

### **Interventions**

Ataluren (Translarna; PTC-124) is a 284.24 Da, achiral, 1,2,4-oxadiazole linked to fluorobenzene and benzoic acid rings. It is an orally delivered compound designed to selectively promote ribosomal read-through of premature stop codons, but not normal termination codons

<http://www.hsruc.nihr.ac.uk/topics/ataluren-translarna-for-cftr-class-i-nonsense-mutation-mediated-cystic-fibrosis/>

### **See also**

Health Technology Assessment Database

### **Keywords**

PTC124; Ataluren; CFTR Modulators; pharmacological\_intervention;