HTA - - Health Technology Assessment Report

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

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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.hsric.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;