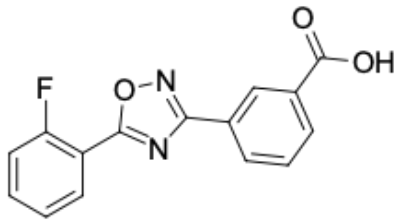


## Ataluren

<http://hk.lumiprobe.com/p/ataluren-775304-57-9>

Ataluren is a drug designed for the treatment of diseases caused by nonsense mutations (premature stop codons). The substance enables the ribosome to ignore a premature stop codon in the messenger RNA and continue the synthesis of a full-length, functionally active protein. It is believed that ataluren interacts with the cellular translational machinery, specifically with the ribosome and the termination factors eRF1/eRF3, promoting the insertion of near-cognate amino acids instead of premature synthesis arrest. Ataluren is used for the treatment of Duchenne muscular dystrophy (DMD) caused by a nonsense mutation in the dystrophin gene in ambulatory patients aged two years and older. The therapy is aimed at restoring dystrophin protein synthesis, which slows disease progression and helps preserve motor function in patients for longer. In preclinical research, ataluren is employed to study the mechanisms of nonsense suppression and to evaluate efficacy in cellular and animal models of various genetic disorders caused by premature stop codons. In addition to Duchenne muscular dystrophy, research is ongoing into its potential for the treatment of cystic fibrosis (CFTR), Shwachman-Diamond syndrome (SBDS), and other hereditary pathologies.

This product is intended for research use only.



外观:

分子量: 284.25

CAS 编号: 775304-57-9

分子式:  $C_{15}H_9FN_2O_3$

溶解度:

质量控制: NMR  $^1H$  和 HPLC-MS (98+%)

储存条件:

法律声明: 僅供研究用途, 我們不向患者銷售, 不可用於醫療用途。