

To:

Head of Paediatric Medicines  
European Medicines Agency  
30 Churchill Place  
London E14 5EU  
United Kingdom  
paediatrics@ema.europa.eu

**Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decision**

Actives substances(s): ataluren

Invented name: Translarna

Latest Decision number(s): 1) P/0284/2016 2) P/0133/2015 3) P/0138/2013  
4) P/106/2011

Corresponding PIP number(s): 1) EMEA-000115-PIP02-09-M03 2) EMEA-000115-PIP02-09-M02  
3) EMEA-000115-PIP02-09-M01 4) EMEA-000115-PIP02-09

Please note that development of the medicinal product above in the [condition(s)/indication(s)]:

Treatment of cystic fibrosis

- has been discontinued  
 has been suspended/put on long-term hold (with possible re-start at a later time)

for the following reason(s): (tick all that apply)

- (possible) lack of efficacy in adults  
 (possible) lack of efficacy in children  
 (possible) unsatisfactory safety profile in adults  
 (possible) unsatisfactory safety profile in children  
 commercial reasons (please specify: )  
 manufacturing / quality problems  
 other regulatory action (please specify: ) (e.g. suspension, revocation of M.A.)  
 other reason (please specify: )

Please add a brief description (max 2000 characters) of the reason(s) for the discontinuation / suspension:

Following the release on 02 March 2017 of the inconclusive results of the ataluren Confirmatory Trial (ACT CF) in nonsense mutation cystic fibrosis (nmCF), PTC Therapeutics International Limited is discontinuing the current clinical development of ataluren in nmCF. As a consequence, the ataluren PIP in CF is discontinued. In particular, the following clinical studies are stopped:

- CF PIP Clinical Study #8: PTC124-GD-017-CF "Open label, multiple dose trial to evaluate the pharmacokinetics (PK), safety and pharmacodynamic (PD) effect of ataluren in paediatric patients with nmCF aged from 2 to less than 6 years."

- CF PIP Clinical Study #9: PTC124-GD-018-CF "Open label, multiple dose trial to evaluate the pharmacokinetics (PK) and safety of ataluren in paediatric patients with nmCF aged from 28 days to less than 23 months".

Name and signature of the PIP contact point: Signature on file

Date: 17 March 2017

Contact for inquiries from interested parties: [vcparis@voisinconsulting.com](mailto:vcparis@voisinconsulting.com)

Telephone: +33 1 41 31 83 00

Email: +33 1 41 31 83 09