06 March 2017

Dr. Tomas Salmonson
CHMP Chair
European Medicines Agency
30 Churchill Place
Canary Wharf
London, E14 5EU
United Kingdom

Subject: **Translarna (ataluren), 125 mg, 250 mg, 1,000 mg**
Granules for oral suspension
Withdrawal letter for the Variation EMEA/H/C/002720/II/012

Dear Dr. Salmonson,

Reference is made to the Type II variation application dossier EMEA/H/C/002720/II/0012 that PTC Therapeutics International Limited (PTC) submitted to the Agency on 01 September 2015 to add a new therapeutic indication for Translarna (ataluren) 125 mg, 250 mg, 1,000 mg, granules for oral suspension (treatment of cystic fibrosis resulting from a nonsense mutation in the *cftr* gene).

PTC is hereby notifying the EMA of its intent to withdraw the application for the above-mentioned Type II variation. This follows the release on 02 March 2017 of the results of the ataluren Confirmatory Trial (ACT CF) in nonsense mutation cystic fibrosis (nmCF); the study did not achieve its primary or secondary endpoints. As a consequence PTC is discontinuing the current clinical development of ataluren in nmCF and is withdrawing the variation application.

An eCTD closing sequence will be submitted as soon as possible in order to remove any updated CTD sections submitted within the frame of this application from the Translarna dossier.

A separate notification will be sent for informing the EMA of the discontinuation of the ataluren Paediatric Development Plan (PIP) in nmCF.