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Prof Bruno Sepodes
European Medicines Agency (EMA)
Domenico Scarlattilaan 6
1083 HS Amsterdam
The Netherlands

31 August 2026

Subject: Withdrawal of Line Extension Application grouped with a Type II Variation for Kaftrio® (ivacaftor/tezacaftor/elexacaftor), 45mg (ivacaftor)/ 30mg (tezacaftor)/ 60mg (elexacaftor) - EMA/X/0000343848; Withdrawal of Type II Variation Application for Kalydeco® (ivacaftor) granules - EMA/VR/0000343903

Dear Prof. Sepodes,

At this point in time, Vertex Pharmaceuticals (Ireland) Limited (Vertex), has taken the decision to withdraw the Extension Application grouped with a Type II Variation to register a new strength of Kaftrio granules (45mg ivacaftor, 30mg tezacaftor, 60mg elexacaftor) and to extend the indication of Kaftrio granules for the treatment of cystic fibrosis (CF) in paediatric patients aged 1 to less than 2 years (EMA/X/0000343848). Vertex has also taken the decision to withdraw the related Type II Variation to extend the indication of Kalydeco® (ivacaftor) granules for use in combination with Kaftrio for the treatment of CF in paediatric patients aged 1 to less than 2 years (EMA/VR/0000343903).

The decision to withdraw the Kaftrio and Kalydeco applications is based on a company decision that is unrelated to these products' quality or clinical efficacy and safety and follows preliminary feedback from the Rapporteurs indicating that additional information would be required to support this extension of indication.

This withdrawal does not have any impact on the approved indications for Kaftrio and Kalydeco or on ongoing clinical trials with Kaftrio.

Vertex remains fully committed to further advancing therapies for people with CF and reserves the right to make further extensions of marketing authorisation applications and to vary the terms of these marketing authorisations at a future date, including for paediatric patients 1 to less than 2 years of age.

Vertex would like to sincerely thank the Rapporteurs, the EMA, the CHMP and PRAC members for the time dedicated to reviewing these applications and the support provided during the procedures.

Vertex agrees for this letter to be published on the EMA website.

Yours Sincerely,

Vice President, Regulatory Strategy International

On behalf of Vertex Pharmaceuticals (Ireland) Limited