To:

Head of Paediatric Medicines
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Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decision

Actives substances(s): Lumacaftor

Invented name: Lumacaftor

Latest Decision number(s): 1) P/0268/2013 2) P/ 3) P/ 4) P/

Corresponding PIP number(s): 1) EMEA-001173-PIP01-11-M01 2) EMEA- 3) EMEA- 4) EMEA-

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

☒ 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: Development of combined lumacaftor/ivacaftor (Orkambi))

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

EMEA-001173-PIP01-11-M01was the last modification for the PIP developed for lumacaftor monotherapy, for which EMA decision was issued on 19 December 2013.

On 12 December 2013, Vertex submitted the initial PIP for lumacaftor/ivacaftor fixed dose combination therapy (current PIP: EMEA-001582-PIP01-13-M08). This was intended to supersede activities with lumacaftor monotherapy, as early studies showed greater efficacy of lumacaftor/ivacaftor combination compared with ivacaftor monotherapy. Accordingly, no new studies with lumacaftor monotherapy have been initiated since 2014.

Lumacaftor/ivacaftor combination therapy is now approved as Orkambi.

Name and signature of the PIP contact point:	Signature on file
Date:	13 Feb 2019
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