An Roinn Sláinte
DEPARTMENT OF HEALTH

Oifig an Aire
Office of the Minister

25 April 2017

Mr Colm Murphy
Meeting Administrator
Corporate Performance and Change Management
South Dublin County Council
County Hall
Tallaght
Dublin 24

Dear Mr Murphy

Thank you for your recent letter concerning (Ref: M27/1216) Orkambi.

I am pleased to inform you, that the HSE and Vertex have reached agreement in principle for the supply to Irish patients of Orkambi (for patients aged 12 years and older) and for Kalydeco (for patients aged 2-5) from next month and for other treatments and age cohorts following market authorisation in Europe. Details on the specific treatments and patient groups are set out in Appendix A.

I wish to reassure you that the HSE and Vertex expect the medicines to be available from the 1st of May. I also wish to inform you that both parties are now working to finalise the contractual arrangements and complete approval processes in advance of the 1st of May.

I wish to apologise for the late reply to your letter. However, given the scale of the investment, the potential benefits for Irish patients and the impact of this decision on the health service overall, it is appropriate to allow the statutory process we have in this State to be appropriately concluded, so as to bring certainty to matters.

I want to also especially acknowledge that this has been an extraordinarily difficult time for CF patients, their families and friends as they have been waiting for this process to conclude.

I am pleased that I am now in a position to provide your with this positive update.

I hope this is of assistance to you.

Yours sincerely

Simon Harris T.D.
Minister for Health

Tús Áite do Shábháilteacht Othar
Patient Safety First

hi Healthy Ireland

Cuirfear fáilte roimh chomhfhreagras i nGaeilge

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Appendix A

What new medicines will be available following the process of approval:

1. Orkambi for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who are homozygous for the *F508del* mutation in the *CFTR* gene. The agreement is expected to enable at least 500 patients to commence treatment in 2017.

2. Kalydeco film-coated tablets for the treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an *R117H* mutation in the *CFTR* gene, The agreement is expected to enable at least 55 patients to commence treatment in 2017.

3. Kalydeco granules for the treatment of children with cystic fibrosis (CF) aged 2 years and older and weighing less than 25 kg who have one of the following gating (class III) mutations in the *CFTR* gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* or *S549R.* This is expected to enable 18 patients to commence treatment in 2017.

Kalydeco tablets are already reimbursed for the treatment of patients with cystic fibrosis (CF) aged 6 years and older and weighing 25 kg or more who have one of the following gating (class III) mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R.*

Will Orkambi for F508del homozygous patients aged under 12 be available on this basis?

Orkambi for the treatment of patients aged under 12 years does not have a market authorisation in Europe at present.

The HSE understands that Vertex is developing the clinical information necessary to underpin market authorisation applications for Orkambi for patients younger than 12 years of age to the European Medicines Agency in due course.

The HSE understands that extensions of the European market authorisation for Orkambi may be received for patients aged 6-11 in 2018 and for patients aged 2-5 from 2019 at the earliest. However, it is expected that Orkambi will be made available to those patient cohorts in Ireland within 28 days of Vertex receiving a market authorisation from the European Medicines Agency.