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Philip Watt
Chief Executive
Cystic Fibrosis Ireland
24 Lower Rathmines Road
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30 November 2016
Open letter

Dear Mr Watt,

Access to Orkambi® for the treatment of Cystic Fibrosis (CF)

Thank you for your recent open letter to Vertex regarding access to Orkambi®.

Vertex was also disappointed to learn via the media over the weekend that the Health Service Executive (HSE) is considering not recommending Orkambi® for the treatment of CF in the Republic of Ireland.

Having continually engaged in good faith with the HSE since Orkambi® was licensed over one year ago, Vertex had hoped that an agreement was within reach and is surprised by the latest developments.

Vertex initially made a full submission to the National Centre for Pharmacoeconomics (NCPE) as requested by the HSE. This was despite internationally acknowledged limitations of HTA processes for the assessment of breakthrough precision medicines like Orkambi®, due to the use of cost-effectiveness thresholds not appropriate to CF and other rare diseases. In addition, the process gave no consideration to the wider holistic and societal benefits to patients, caregivers and families.

Vertex subsequently met with HSE representatives six times in five months, presenting a range of specific workable proposals that could expedite access for all eligible patients. At no time during the process did we receive a counter-offer or guidance from the HSE on decision making criteria. Proposals from Vertex included, for example, guarantees of budget certainty for the HSE, novel access schemes and ways to reduce the timelines on future reimbursement decisions.

Vertex therefore refutes allegations by the HSE of failing to meaningfully engage and, the publically quoted budget impact figure of €392m is incorrect, unrealistic and does not reflect discussions to date with the HSE. The current impasse now has a number of significant implications:

- Seriously ill people living with CF have now been waiting for treatment for over a year since Orkambi® was licenced in the EU (November 2015).
- Kalydeco®, another precision medicine that treats the underlying cause of CF, is currently available to most eligible patients in Ireland. There is therefore an inequity of access to precision medicines for the treatment of CF patients in Ireland based on genotype.

- Future investment in the research and development (R&D) of innovative treatments for CF could be compromised, to the potential detriment of future generations of CF patients and their families. In 2015 Vertex invested 83% of revenues into R&D, substantially higher than the industry average of 18%.

Vertex has spent over 15 years and hundreds of millions of dollars to date developing medicines for CF. Our mission is to cure the disease and we have a pipeline of medicines we are developing in pursuit of this mission.

Orkambi® remains an important step forward for the treatment of CF, helping patients stay out of hospital, breathe more easily, gain weight and live longer lives. Recent clinical data[i] has shown that Orkambi® reduces annual lung deterioration by 42%.

Vertex understand that the present situation and manner in which information, or misinformation, is being communicated is causing undue fear and confusion for the patient community.

We are however committed to finding a solution and have today called on Simon Harris TD, the responsible Minister of Health, to intervene directly ahead of a decision by the HSE in the mutual interests of patients, clinicians and industry.

Vertex stands ready to meet the Minister at short notice in order to resolve this vitally important issue for the CF community of Ireland as soon as possible.

I will keep you updated as soon as I receive a reply from the Minister. Should you like to meet me to discuss the matter further or require any other information at this time please do not hesitate to ask.

Yours sincerely,

Simon Lem
Vice President, Regional General Manager, Europe North

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[i] Konstan MW, et al. *Pediatr Pulmonol.* 2016;51:260. Abstract 180. Presented at the Annual North American Cystic Fibrosis Conference. Orlando, Florida. October 27 - 29, 2016.