Jeff Leiden, M.D., Ph. D.
President, CEO, and Chairman

Dr Sarah Wollaston MP
Chair, Health and Social Care Committee
House of Commons
London
SW1A 0AA

By Email

24 October 2019

Dear Dr Wollaston,

Availability of Orkambi on the NHS

I wanted to provide you with an update regarding your inquiry, 'Availability of Orkambi on the NHS'.

I am pleased to inform you that Vertex and NHS England have reached an agreement on access to our currently licensed cystic fibrosis (CF) medicines to all eligible patients in England, specifically:

- CF patients in England ages 2 years and older who have two copies of the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene will have access to Orkambi (lumacaftor/ivacaftor);
- CF patients ages 12 years and older who either have two copies of the F508del mutation or one copy of the F508del mutation and a copy of one of the other 14 licensed mutations will have access to Symkevi (tezacaftor/ivacaftor) in combination with ivacaftor;
- Expanded access to Kalydeco (ivacaftor) to include people ages 18 years and older who have the R117H mutation and those patients ages 12 months and older who have one of the nine licensed gating mutations.
- Access to any future indications of these medicines.

This important agreement, reached in collaboration and partnership with NHS England and the National Institute for Health and Care Excellence (NICE), means that more than 5,000 eligible CF patients in England can be prescribed these CFTR modulators within 30 days. The agreement includes real world data collection that will be used for a future submission of our medicines to NICE.

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The announcement in England follows the agreement we reached in Scotland in September on access to Orkambi and Symkevi. We will now work urgently to extend access to Wales and Northern Ireland, so that every eligible cystic fibrosis patient can access precision medicines that treat the underlying cause of their disease.

Looking ahead, we welcome the current review of evaluation methods used by NICE, which we hope will be an opportunity to address the gaps in these methods for future assessment of medicines for rare, genetic, life-long conditions. Some of these have been highlighted in evidence you have taken as part of your inquiry, and I am sure your Committee will continue to play a role in ensuring they are reviewed and debated further.

We appreciate the interest and continued engagement that you and the Committee have taken in this important matter.

Yours sincerely,

Jeffrey Leiden, M.D., Ph.D.

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