Vertex Pharmaceuticals media statement in response to NICE Clarification
NICE, 20 December 2018

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On 13 December, we received a response from the National Institute for Health and Care Excellence (NICE) following our meeting on 30 November 2018. We are disappointed with the NICE response as it is inconsistent with the progress that we felt we had made in our recent meetings. In those meetings, we were encouraged that NICE would reassess how best to apply their methodologies to the unique set of characteristics of cystic fibrosis (CF) and our precision medicines.

In England, 9,000 people live with CF.[1] This is an exceptionally large number of patients to be affected by a disease that, globally, is rare.

While NICE's appraisal methodologies work for many medicines and chronic diseases, the response we received highlights how NICE is constrained by current processes and ways of working with the Department of Health and Social Care and NHS England. We continue to advocate for modernized NICE processes that are able to appropriately value innovative medicines that have the potential to extend life such as those that are used to treat patients with rare diseases, like CF.

It is our highest priority to find a path that provides eligible patients in England access to ORKAMBI® (lumacaftor/ivacaftor) and our future CF medicines – we are committed to working with NICE and NHS England on the best ways and methodologies to provide access.

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Notes to editors

About cystic fibrosis

- Over 10,000 people in the UK have CF[2] − the second highest number in the world.[3]
- CF is a debilitating, life-shortening inherited condition that causes progressive damage to organs across the body from birth.[4]
- Currently, there is no cure for CF and half of people in the UK with CF die before they are 31.[5]
- The daily impact of treatment is significant. It can take up to four or more hours involving, nebulisers, physiotherapy and up to 70 tablets a day.[6]
- CF accounts for 9,500 hospital admissions and over 100,000 hospital bed days a year. A third of these are used by children under 15.[7]

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases.

In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., USA, Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for nine years in a row.

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[1] CF Registry Scotland Report 2015 Available at: https://www.cysticfibrosis.org.uk/the-work-we-do/uk-cf-registry/reporting-and-resources [Last accessed: December 2018].
[2] CF Trust. FAQs. Available at: https://www.cysticfibrosis.org.uk/what-is-cystic-fibrosis/faqs. [Last accessed December 2018].
[3] Lopes-Pacheco M. CFTR Modulators: Shedding Light on Precision Medicine for Cystic Fibrosis. *Front Pharmacol.* 2016;7:275. Published 2016 Sep 5. doi:10.3389/fphar.2016.00275.
[4] NHS Choices. Cystic Fibrosis overview. https://www.nhs.uk/conditions/cystic-fibrosis/. [Last accessed December 2018].
[5] CF Trust. UK Cystic Fibrosis Registry 2016 Annual Data Report 2016 https://www.cysticfibrosis.org.uk/registryreports. [Last accessed December 2018].
[6] CF Trust. Transition and adherence. Available at: https://www.cysticfibrosis.org.uk/the-work-we-do/research/research-areas/transition-and-adherence [Last accessed December 2018].

[7] BLF CF Statistics. Available at: https://statistics.blf.org.uk/cystic-fibrosis. [Last accessed December 2018].