To the UK Cystic Fibrosis Community,

**Update on access to Vertex cystic fibrosis medicines across the UK**

Vertex is working hard to secure access to our CF medicines for all eligible people across the UK. This is taking significantly longer than we would hope, and we are acutely aware of the anxiety this delay is causing. We are ready to negotiate anytime, anywhere and we call on the National Health Service (NHS) to demonstrate a similar commitment to finding a solution. Our aim with this letter is to help inform you about the steps we've taken and, above all, provide reassurance that we remain fully committed to finding a solution.

Our commitment

Since 1998, Vertex has been committed to change the way CF is treated and, ultimately, to try to cure the disease. We have already made remarkable progress: the significant investment we are making into the development of new medicines (86% of our revenue over the last five years) has resulted in Kalydeco (ivacaftor) and Orkambi (ivacaftor/lumacaftor). Our work isn't done yet - we are working hard on developing new medicines for the two out of three people with CF who still do not have a medicine to treat the underlying cause of their disease.

This is an exciting prospect, yet also presents challenges for access to all of these new medicines. If the Government and NHS continue to use current processes, lengthy delays and restrictions are inevitable – so we are calling for a more innovative approach.

Since we started the NICE assessment process in the Autumn of 2015, we have repeatedly raised our concern that the current processes are not appropriate for assessing medicines for rare diseases like CF. We have urged NICE and NHS England to work with us toward an innovative solution so as not to unduly delay or preclude access to this important new medicine. If medicines for rare diseases are to be made available in the UK country, changes to the processes are needed. Thousands of patients around the world have access to Orkambi, with reimbursement in Ireland, Austria, Luxembourg, Denmark and Germany, and patients in the UK deserve to join them.

Innovative solutions that can work for the UK

Cystic Fibrosis is a special case in the UK: around 12 percent of all people with the disease worldwide live here (although just 1% of the world's population). We are therefore calling for CF to be looked at differently in the UK.

We have proposals on an innovative way to make our transformative CF medicines available on the NHS to give the government budget certainty and to allow us to continue our significant reinvestment in R&D. One way to do this could be a 'portfolio approach' that would provide accelerated access to our current CF medicines and those we develop in the future. We have proposed this approach to senior government officials in England, Scotland, Northern Ireland and Wales. We are anxiously awaiting feedback from each as to how we can take discussions forward.

Innovative approaches like this can work. In June 2017, we reached an agreement on a similar 'portfolio approach' with the Republic of Ireland, which covers both our existing medicines, and new medicines that we bring forward for these patients. We are ready with proposals on how we can make a similar approach work in the UK.

We recognise that for people with CF, each day matters and there is an urgent need for transformative medicines. We call on the NHS in each country in the UK to negotiate with us on this as soon as possible.

Vertex will be looking to update the CF community with further details in the near future.

**Simon Lem**
**Vice President, Regional General Manager, Europe North**