



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

Dear CF Support Group:

I am writing in response to the open letter from the CF Support Group, dated 28 January 2019. I take your views seriously, and I want to reply personally. Let me take this opportunity to reiterate Vertex's unwavering commitment to ensuring broad and timely access to the life-changing cystic fibrosis (CF) medicines that we have already discovered and developed in our research labs and the new ones that are on their way. You are right that we are at a pivotal moment for the global CF community. Within a few years, with continued investment and research, we hope to offer life-changing treatments for 90% of people with CF.

More than 20 years ago, Vertex embarked on a hugely ambitious program of investment and research, with no guarantee of success. In the last five years alone we have reinvested nearly 70% of our revenue back into this research. As a result, we have now achieved what was once thought impossible: we have discovered and brought to patients the first medicines to treat the underlying cause of this devastating disease. However, our work in CF is not done and we need to continue to invest at very high levels if we are to achieve our goal of developing such medicines for the 90% of CF patients we think we can help.

At Vertex, we believe the voices of patients and physicians should be heard, and we dedicate significant time and resources to maintaining an open and productive dialogue with the community. While we may not always agree on every point, please be assured that your views are an important part of the broader effort to ensure access to our new breakthrough medicines.

You are right, we have had some challenges in gaining public access in some countries outside of the U.S. However, we have also made real progress in many countries, with patients having access to our medicines in 36 countries.

Precision therapies like ours are delivering unprecedented results, but they also present new economic challenges. We recognise fully the constraints that health systems around the world are working under, which create unenviable choices for the health authorities. While I do not underestimate the difficulty of these pressures, health authorities also need to adapt their systems to the realities of modern medicine.

Vertex shares the responsibility with governments and regulators to do all that we can to agree to a fair price that reflects the value of these medicines in all countries where they are needed. This in turn will allow us to keep investing in the possibility of a world without CF and continuing our important work in other serious diseases, such as alpha-1 antitrypsin and sickle cell disease. And importantly, in the countries where access is still



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not agreed, our position is always the same – we are doing everything we can to work constructively with the government authorities to find workable solutions.

Yours sincerely,

Dr. Jeff Leiden
Chief Executive Officer, President