Vertex Pharmaceuticals Incorporated
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May 3, 2018

BY ELECTRONIC DELIVERY

Steven D. Pearson
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

Dear Mr. Pearson:

This letter serves as our response to ICER's May 3rd revised evidence report on our cystic fibrosis (CF) medicines, Kalydeco® (ivacaftor), Orkambi® (lumacaftor/ivacaftor) and Symdeko® (tezacaftor/ivacaftor and ivacaftor). We are disappointed by your review for three reasons – (i) it relied on a flawed scientific methodology, (ii) it employed a constantly shifting process with a pre-ordained outcome, and (iii) most importantly, it is being used by you and others to attempt to restrict the currently broad access of CF patients to transformative and life-saving medicines.

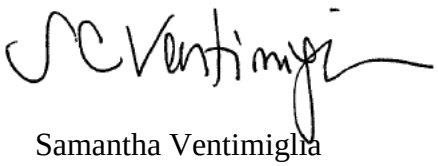
From a scientific standpoint, your analyses utilized a series of arbitrary modeling choices that fail to capture the full benefits of our medicines, thereby intentionally leading to worse outcomes for these transformative medicines. Omission of such evidence for medicines that are the first to treat the underlying cause of CF and fundamentally change the course of disease progression for patients is particularly egregious. Moreover, the process used in the ICER evaluation was a sham, as best evidenced by the fact that you presented the final cost-effectiveness recommendations last week, three weeks before the public hearing at which patients, physicians and Vertex were scheduled to present their evidence. In addition, over the last seven months you have ignored numerous concerns from patients, physicians, the CF Foundation and other advocates concerning your methodology and its inability to evaluate breakthrough orphan drugs for CF. By intentionally ignoring this evidence, it is clear that your conclusions were agenda-driven and pre-ordained.

Today, CF patients in the United States have broad access to Kalydeco, Orkambi, and Symdeko, which address the underlying cause of their devastating disease. Both public and private payers have universally recognized their clinical value. We object strongly to your intentions to use your flawed results to attempt to remove this broad access for patients with this

serious disease. We believe the doctors who care for CF patients - not panels of academics and health economists - should be making treatment decisions for these patients.

Given these issues, our further participation in this process is pointless. Instead, our focus and efforts will remain on honoring our commitment to the CF community – to develop more new breakthrough medicines and ultimately a cure for this devastating disease, and to ensure that all eligible patients will have equal access to these treatments. We will continue to vigorously oppose attempts by ICER and others who seek to derail these efforts.

Sincerely,



Samantha Ventimiglia

Vice President
Government Affairs & Public Policy