TBCAB
GLOBAL TB COMMUNITY ADVISORY BOARD

Jeffrey Leiden, Chairman, President and CEO
Vertex Pharmaceuticals
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CC:
Dr. David Altshuler, Executive Vice President, Global Research and Chief Scientific Officer
Dr. Jeffrey A. Chodakewitz, Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer
Dr. Patricia Hurter, Senior Vice President, CMC and Preclinical Development
Mr. Amit K. Sachdev, Executive Vice President, Policy, Access and Value

September 15, 2015

Open letter re: Appeal to develop VXc-486 for tuberculosis

Dear Dr. Leiden and colleagues,

We congratulate you on the exciting findings presented at the 2014 Annual Interscience Conference on Antimicrobial Agents and Chemotherapy in regards to your compound VXc-486's potency against drug-sensitive, drug-resistant, and latent tuberculosis (TB).[1] As advocates for and representatives of communities affected by TB, we are concerned that nearly one year after these findings were presented, development of VXc-486 for TB has not progressed. As such, we appeal to Vertex to publicly commit to the development of VXc-486 for TB treatment, and to collaborate with partners in bringing this compound forward.

TB is a devastating disease, causing 9 million people to fall sick and 1.5 million to die each year. Nearly 1/3 of the world is estimated to have TB infection, meaning they could develop active TB disease at any time. Existing treatment for TB disease and TB infection is lengthy, difficult to tolerate, and—in the case of drug-resistant TB—not effective. We urgently need new treatment options for all forms of TB. As such, the demonstrated potency of the prodrug of VXc-486 in mouse models against drug-sensitive and drug-resistant TB as well as TB infection, and its additive activity with other existing treatment options, warrant urgent further study.

[1] Locher CP, Jones SM, Hanzelka BL, et al. VXc-486, a novel dual targeting GyrB/ParE inhibitor for the treatment of bacterial infections: VXc-486 prodrug sterilizes Mycobacterium tuberculosis infection in combination with anti-mycobacterial Drugs In Vivo. Poster F-270. Presented at the LIVth Annual Interscience Conference on Antimicrobial Agents and Chemotherapy, Washington, D.C., Sept 9, 2014

With telaprevir, Vertex made huge advances in the treatment of another leading infectious disease, hepatitis C. But Vertex has been notably absent from TB research and development; VXc-486 presents a welcome opportunity to rectify this neglect of one of the world's deadliest infectious diseases.

As such, we ask Vertex to:

1) commit resources to advancing VXc-486's preclinical development, and clinical development should the compound merit further study;
2) collaborate with other TB drug developers and research consortia to make VXc-486 available for combination studies;
3) create an access strategy, which includes plans for compassionate use, affordable pricing, and widespread registration, should VXc-486 enter clinical trials;
4) develop research and access plans in conjunction with representatives of TB-affected communities, such as the Global TB Community Advisory Board, per the Good Participatory Practice Guidelines for TB Drug Research.[2]

With the urgent need for improved treatment options for TB, we cannot afford to leave a promising compound unstudied and inaccessible.

We look forward to discussing with you about Vertex's plans for developing VXc-486, and request a teleconference with you in the near future. We request by October 14, 2015 your response to this letter with suggested dates and times for a call (preferably in the morning Boston time to accommodate our various time zones of residence). Please direct any responses to Wim Vandevelde at wim@eatg.org.

Thank you and best regards,
The Global TB Community Advisory Board

[2] http://www.cptrinitiative.org/downloads/resources/GPP-TB%20Oct1%202012%20FINAL.pdf