Vertex Pharmaceuticals Incorporated
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November 21st, 2019

BY ELECTRONIC DELIVERY

Subject: Notification Pursuant to 18 V.S.A. § 4637(c)

To the Office of the Attorney General of Vermont:

Vertex Pharmaceuticals Incorporated ("Vertex") hereby notifies the Office of the Attorney General of Vermont of the following new prescription drug, pursuant to 18 V.S.A. § 4637(c):

Description of New Prescription Drug	**NDC Number**	**Date of Commercial Availability**	**WAC (Wholesale Acquisition Cost, as of the Date of Commercial Availability)**
TRIKAFTA™ (elexacaftor, tezacaftor and ivacaftor tablets; ivacaftor tablets)	51167-0331-01	October 25, 2019	$23,896.13 for a 28-day supply

A description of the marketing and pricing plans used in the launch of the new drug in the United States and internationally.

Marketing Plan

Vertex's commercial field team in the United States is composed of a small number of individuals, and we focus our United States marketing activities for TRIKAFTA towards a limited number of physicians and health care professionals who are located at Cystic Fibrosis (CF)-focused accredited centers in the United States.

The objective of these activities is to raise awareness and understanding about the approved indication, dosing, efficacy and safety data that are consistent with TRIKAFTA's FDA approved label.

Specific activities related to physicians and health care professionals include print distribution by the commercial field team to CF care centers, digital advertising (e.g., TRIKAFTA's product website), and other educational programs.

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Specific activities related to people living with CF and their caregivers include print distribution of patient materials to CF care centers and digital advertising (e.g., TRIKAFTA's product website and social media pages). Vertex does not engage in traditional direct-to-consumer advertising (e.g., television or mass media). Vertex also operates a comprehensive patient support program, known as Vertex Guidance & Patient Support (Vertex GPS™), which helps eligible patients who have been prescribed TRIKAFTA, access their medication and help them stay on track with treatment.

Pricing Plan

At Vertex, our mission is ambitious: to discover medicines that can prevent, cure or fundamentally change the outcomes of serious diseases for patients and their families. Our pricing philosophy supports this mission and reflects the transformative clinical value of our medicines, our commitment to patient access and the investment required to bring new medicines to CF and other serious diseases.

When determining the price of TRIKAFTA, we considered a combination of factors, including:

- Efficacy of the medicine and the benefit to patients of treating the underlying cause of CF, therefore having the potential to modify the course of the disease
- Ease for patients to access medicine
- Impact on a serious orphan disease that places significant burden on the healthcare system and society
- Commitment by Vertex to invest in the resources required to support the discovery and development of new medicines for those waiting for treatments and cures

TRIKAFTA is a significant clinical advancement in the treatment of people with CF aged 12 years and older with at least one *F508del* mutation. The benefits and risks of TRIKAFTA were evaluated in two Phase 3 studies. In both studies, people taking TRIKAFTA experienced significant improvement in lung function and CF respiratory symptoms.

- One study compared TRIKAFTA with an active comparator, tezacaftor/ivacaftor and ivacaftor, in people with two *F508del* mutations. The results showed a significant incremental improvement in lung function and CF respiratory symptoms in the people taking TRIKAFTA.
- The other study compared TRIKAFTA with placebo in people with an *F508del* mutation and another mutation defined in the study. The results showed significant improvement in lung function, reduction in pulmonary exacerbations, and improvements in CF respiratory symptoms in the people taking TRIKAFTA.

The estimated volume of patients that may be prescribed the drug.

Based on patient registry data (2017), in the United States, up to 17,300 total patients aged 12 years and older may be eligible for treatment with TRIKAFTA.

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Was the drug granted breakthrough therapy designation by the federal Food and Drug Administration (FDA) prior to final approval?
Yes, TRIKAFTA was granted breakthrough therapy designation by the Food and Drug Administration prior to approval.

Did the drug receive a priority review by the federal FDA prior to final approval?
Yes, the Food and Drug Administration (FDA) granted TRIKAFTA a priority review with a Prescription Drug User Fee Action (PDUFA) date of March 19, 2020. The FDA approved TRIKAFTA on October 21, 2019, approximately 5 months earlier than the PDUFA date that had been assigned under priority review.

The date and price of acquisition if the drug was not developed by the manufacturer.
N/A - TRIKAFTA was developed by Vertex Pharmaceuticals Incorporated.

For any questions concerning this notification please contact: Vrtx_StateDrugPrice@vrtx.com.

Sincerely,
Vertex Pharmaceuticals Incorporated