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January 7, 2024

***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):

<b>Description of New Prescription Drug</b>	<b>NDC Number</b>	<b>Date of Commercial Availability</b>	<b>WAC (Wholesale Acquisition Cost, as of the Date of Commercial Availability)</b>
CASGEVY™	51167-0290-09	December 8, 2023	\$2,200,000.00

**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 CASGEVY towards a limited number of physicians and health care professionals who are located at Authorized Treatment Centers (ATCs) 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 CASGEVY’s FDA approved label.

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



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Specific activities related to people living with sickle cell disease (SCD) and their caregivers include print distribution of patient materials to ATCs care centers and digital advertising (e.g., CASGEVY’s product website and social media pages). Vertex does not engage in traditional direct-to-consumer advertising (e.g., television or mass media). While not part of Vertex’s marketing plan, the company also operates a patient support program, known as Vertex Connects™, that provides educational resources, communications, and support to eligible patients who have been prescribed CASGEVY.

### **Pricing Plan**

When determining the price of CASGEVY, we applied a value-based approach that recognizes the holistic value of a one-time therapy:

- Lifetime clinical benefit of CASGEVY as a one-time treatment for patients with SCD facing significant early mortality
- Enables access for all eligible patients regardless of payer type without excessive hurdles to therapy
- Reduced economic impact of lifelong health care expenses and long-term value delivered across healthcare systems and society
- Investment required for ongoing discovery and development of new medicines

The list price of CASGEVY reflects the transformative clinical and economic value that a one-time treatment that eliminates severe vaso-occlusive crises (VOCs) brings to patients and their families, as well as the resulting long-term value delivered across the healthcare ecosystem and society.

- In clinical trials, 93.5% of patients with SCD achieved the primary efficacy endpoint of freedom from VOCs for at least 12 consecutive months.
- List price is within the range of traditional cost-effectiveness thresholds based on external economic modeling.

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

There are approximately 120,000 prevalent cases of Sickle Cell Disease (SCD) in the US. Among prevalent cases of SCD, approximately 16,000 have severe disease defined by recurrent vaso-occlusive crises, meet key age criteria, and would be considered for gene therapy.

### **Was the drug granted breakthrough therapy designation by the federal Food and Drug Administration (FDA) prior to final approval?**

No, CASGEVY was not 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?**



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Yes, the Food and Drug Administration (FDA) granted CASGEVY a priority review with a Prescription Drug User Fee Action (PDUFA) date of December 8, 2023.

**The date and price of acquisition if the drug was not developed by the manufacturer.**

N/A

For any questions concerning this notification please contact: [Vrtx\\_StateDrugPrice@vrtx.com](mailto:Vrtx_StateDrugPrice@vrtx.com).

Sincerely,  
Vertex Pharmaceuticals Incorporated