**State Price Transparency Reporting**

**PEMAZYRE New Drug Reporting**

**State:** Vermont

**Due Date:** May 20th

**Trigger:** Shipment of PEMAZYRE on April 20th

**New Drug Notification Date:** April 22nd

**Reporting Method: Email submission to** [AGO.highcostprescriptiondrugs@vermont.gov](mailto:AGO.highcostprescriptiondrugs@vermont.gov)

1. **Description of domestic and international marketing and pricing plans**

To market PEMAZYRE, Incyte designed activities to increase awareness and understanding with healthcare providers about the first FDA-approved product for these patients. Marketing activities will include education and training provided by our existing sales force and by contracted speakers to health care providers. There is a consumer-directed website to educate patients on the disease state and PEMAZYRE, and Incyte intends to limit other direct-to-consumer advertising activities given the limited patient population.

At Incyte, we are driven by rigorous science and committed to ensuring patients have access to our innovative medicines. We responsibly price our drugs by balancing the value of the outcomes and innovation they bring to patients and the health care system within market and societal expectations.

* Cholangiocarcinoma (CCA) is an orphan disease with limited treatment options.
* PEMAZYRE is the first treatment innovation for patients with cholangiocarcinoma since chemotherapy was approved 25 years ago
* It is a targeted treatment approved in the United States for previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with an FGFR2 fusion or other rearrangement as detected by an FDA-approved test.

1. **Estimated number of patients who may be prescribed the drug:**

Cholangiocarcinoma (CCA) is a rare disease with a poor prognosis. Published epidemiology studies vary in findings and suggest that the incidence rate of CCA ranges between 2.2 to 2.8 per 100,000 lives. CCA is a molecular target-rich disease with FGFR2 fusions and rearrangements representing between 10-16% of intrahepatic CCA patients. FGFR2 fusions and rearrangements are rare in extrahepatic CCA. Patients with unresectable, previously treated iCCA with an FGFR2 fusion or rearrangement translate to approximately 1 per 1 million lives. The rarity of the disease and FGFR2 fusions and rearrangements, including the ranges reported in published literature, raise uncertainty in the accuracy of forecast projections.

1. **Whether the drug was granted breakthrough therapy designation or priority review by the FDA prior to final approval**

The FDA granted PEMAZYRE Breakthrough Therapy designation for the treatment of patients with previously treated advanced/metastatic or unresectable FGFR2 translocated cholangiocarcinoma. Additionally, the FDA granted PEMAZYRE Orphan Drug designation for the treatment of cholangiocarcinoma, and the New Drug Application (NDA) for PEMAZYRE was reviewed under the FDA’s Priority Review program.

1. **Date and price of acquisition if the drug was not developed by Incyte**

n/a