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FDA Approves Incyte's Pemazyre™ (pemigatinib) as First Targeted Treatment for Adults with Previously Treated, Unresectable Locally Advanced or Metastatic Cholangiocarcinoma

April 17, 2020

- Pemazyre received Orphan Drug, Breakthrough Therapy and Priority Review designations based on the significant needs of people living with this devastating cancer -

- Investor conference call and webcast scheduled for Monday, April 20, 2020, at 8:00 a.m. ET -

WILMINGTON, Del.--(BUSINESS WIRE)--Apr. 17, 2020-- Incyte (Nasdaq:INCY) today announced that the U.S. Food and Drug Administration (FDA) has approved Pemazyre™ (pemigatinib), a kinase inhibitor indicated for the treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test. Pemazyre is the first and only FDA-approved treatment for this indication, which was approved under accelerated approval based on overall response rate and duration of response (DOR). Continued approval may be contingent on verification and description of clinical benefit in a confirmatory trial(s).

"Our research into FGFR2 fusions or rearrangements in cholangiocarcinoma and the development of Pemazyre as the first targeted treatment option demonstrates our commitment to translating scientific discovery into solutions that can positively impact patients' lives," said Hervé Hoppenot, Chief Executive Officer, Incyte. "We're proud to bring Pemazyre to patients and will make this new treatment available immediately."

The FDA approval was based on data from the FIGHT-202 study, a multi-center, open-label, single-arm study that evaluated Pemazyre as a treatment for adults with cholangiocarcinoma. In patients harboring FGFR2 fusions or rearrangements (Cohort A), Pemazyre monotherapy resulted in an overall response rate of 36% (primary endpoint), and median DOR of 9.1 months (secondary endpoint). Warnings and precautions for Pemazyre include eye problems such as dry or inflamed eyes, inflamed cornea, increased tears and a disorder of the retina; high levels of phosphate in the blood; and, for women who are pregnant, a risk of harm to the unborn baby or loss of pregnancy.

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.

"Although cholangiocarcinoma is considered a rare disease, it has been on the rise over the past three decades,"^{1,2} said Ghassan Abou-Alfa, M.D., Memorial Sloan Kettering Cancer Center. "It is encouraging to have a new targeted treatment option for patients who historically have had limited options after first-line chemotherapy or surgery, in which relapse rates remain high."

Cholangiocarcinoma is a rare cancer that forms in the bile duct. It is classified based on its anatomical origin: intrahepatic cholangiocarcinoma (iCCA) occurs in the bile duct inside the liver and extrahepatic cholangiocarcinoma occurs in the bile duct outside the liver. Patients with cholangiocarcinoma are often diagnosed at a late or advanced stage when the prognosis is poor.^{3,4} The incidence of cholangiocarcinoma varies regionally and ranges between 0.3-3.4 per 100,000 in North America and Europe.³ FGFR2 fusions or rearrangements occur almost exclusively in iCCA, where they are observed in 10-16% of patients.⁵⁻⁷ FGFRs play an important role in tumor cell proliferation and survival, migration and angiogenesis (the formation of new blood vessels). Activating fusions, rearrangements, translocations and gene amplifications in FGFRs are closely correlated with the development of various cancers.

"Today's approval of Pemazyre provides an exciting new treatment option for patients and will bring hope to those who typically face a difficult diagnosis journey and poor prognosis," said Stacie Lindsey, President, Cholangiocarcinoma Foundation.

Incyte is committed to supporting patients and removing barriers to access medicines. Eligible patients in the U.S. who are prescribed Pemazyre have access to IncyteCARES (Connecting to Access, Reimbursement, Education and Support), a comprehensive program offering patient support, including financial assistance and ongoing education and resources to eligible patients. More information is available at [Pemazyre.com](https://www.pemazyre.com).

The FDA is also expected to approve FoundationOne®CDx as the companion diagnostic for Pemazyre. FoundationOne CDx is Foundation Medicine's comprehensive genomic profiling assay and broad companion diagnostic platform approved for all solid tumors. If approved, this companion diagnostic would help identify patients with FGFR2 fusions or rearrangements who may be eligible for Pemazyre.

Conference Call Information

Incyte will host an investor conference call and webcast on Monday, April 20, 2020, at 8:00 a.m. ET. The webcast will be available via investor.incyte.com.

To access the conference call, please dial 877-407-3042 for domestic callers or +1-201-389-0864 for international callers. When prompted, provide the conference identification number 13700772.

If you are unable to participate, a replay of the conference call will be available for 30 days. The replay dial-in number for the United States is 877-660-6853 and the dial-in number for international callers is +1-201-612-7415. To access the replay, you will need the conference identification number 13700772.

About FIGHT-202

The FIGHT-202 Phase 2 multi-center, open-label, single-arm study (NCT02924376) evaluated the safety and efficacy of Pemazyre – a selective fibroblast growth factor receptor (FGFR) inhibitor – in adult (age ≥18 years) patients with previously treated, locally advanced or metastatic

cholangiocarcinoma with documented FGFR2 fusion or rearrangement.

Patients were enrolled into one of three cohorts – Cohort A (FGFR2 fusions or rearrangements), Cohort B (other FGF/FGFR genetic alterations) or Cohort C (no FGF/FGFR genetic alterations). All patients received 13.5 mg Pemazyre orally once daily (QD) on a 21-day cycle (two weeks on/one week off) until radiological disease progression or unacceptable toxicity.

The primary endpoint of FIGHT-202 was overall response rate (ORR) in Cohort A, assessed by independent review per RECIST v1.1. Secondary endpoints include ORR in Cohorts B, A plus B, and C; and duration of response (DOR).

For more information about FIGHT-202, visit <https://clinicaltrials.gov/ct2/show/NCT02924376>.

About FIGHT

The FIGHT (**F**ibroblast **G**rowth factor receptor in oncology and **H**ematology **T**rials) clinical trial program includes ongoing Phase 2 and 3 studies investigating the safety and efficacy of Pemazyre therapy across several FGFR-driven malignancies. Phase 2 monotherapy studies include FIGHT-202, as well as FIGHT-201 investigating Pemazyre in patients with metastatic or surgically unresectable bladder cancer, including with activating FGFR3 mutations or fusions/rearrangements; FIGHT-203 in patients with myeloproliferative neoplasms with activating FGFR1 fusions/rearrangements; FIGHT-207 in patients with previously treated, locally-advanced/metastatic or surgically unresectable solid tumor malignancies harboring activating FGFR mutations or fusions/rearrangements, irrespective of tumor type. FIGHT-205 is a Phase 2 study investigating Pemazyre plus pembrolizumab combination therapy and Pemazyre monotherapy in patients with previously untreated, metastatic or unresectable bladder cancer harboring FGFR3 mutations or fusions/rearrangements who are not eligible to receive cisplatin. FIGHT-302 is a Phase 3 study investigating Pemazyre as a first-line treatment for patients with cholangiocarcinoma with FGFR2 fusions or rearrangements.

About Pemazyre™ (pemigatinib)

Pemazyre is a kinase inhibitor indicated for the treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test.

Pemazyre is a potent, selective, oral inhibitor of FGFR isoforms 1, 2 and 3 which, in preclinical studies, has demonstrated selective pharmacologic activity against cancer cells with FGFR alterations.

Pemazyre is marketed by Incyte in the United States. Incyte has granted Innovent Biologics, Inc. rights to develop and commercialize pemigatinib in hematology and oncology in Mainland China, Hong Kong, Macau and Taiwan. Incyte has retained all other rights to develop and commercialize pemigatinib outside of the United States.

Additionally, Incyte's marketing authorization application (MAA) seeking the approval of pemigatinib for patients with cholangiocarcinoma in the EU has been validated by the European Medicines Agency (EMA) and is currently under review for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that is relapsed or refractory after at least one line of systemic therapy.

Pemazyre is a trademark of Incyte Corporation.

Important Safety Information

Before you take PEMAZYRE, tell your healthcare provider about all of your medical conditions, including if you:

- have vision or eye problems
- have problems swallowing tablets
- are pregnant or plan to become pregnant. PEMAZYRE can harm your unborn baby or cause loss of your pregnancy (miscarriage). You should not become pregnant during treatment with PEMAZYRE.

Females who can become pregnant:

- Your healthcare provider should do a pregnancy test before you start treatment with PEMAZYRE.
- You should use an effective method of birth control during treatment and for 1 week after your final dose of PEMAZYRE. Talk to your healthcare provider about birth control methods that may be right for you.
- Tell your healthcare provider right away if you become pregnant or think that you may be pregnant.

Males with female partners who can become pregnant:

- You should use effective birth control when sexually active during treatment with PEMAZYRE and for 1 week after your final dose of PEMAZYRE.
- are breastfeeding or plan to breastfeed. Do not breastfeed during treatment and for 1 week after your final dose of PEMAZYRE.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

What are the possible side effects of PEMAZYRE?

PEMAZYRE may cause serious side effects, including:

- **Eye problems.** Certain eye problems are common with PEMAZYRE but can also be serious. Eye problems include dry eye or inflamed eyes, inflamed cornea (front part of the eye), increased tears, and a disorder of the retina (an internal part of the eye). You will need to see an eye specialist for a complete eye exam before you begin treatment with PEMAZYRE, every 2 months for the first 6 months, and then every 3 months during treatment with PEMAZYRE.
 - You should use artificial tears or substitutes, hydrating or lubricating eye gels as needed, to help prevent or treat dry eyes.
 - **Tell your healthcare provider right away if you develop any changes in your vision during treatment with PEMAZYRE, including: blurred vision, flashes of light, or see black spots.** You may need to see an eye specialist right away.

- **High phosphate levels in your blood (hyperphosphatemia).** Hyperphosphatemia is common with PEMAZYRE but can also be serious. Your healthcare provider will check your blood phosphate levels during treatment with PEMAZYRE.
 - Your healthcare provider may prescribe changes in your diet or phosphate lowering therapy, or change, interrupt, or stop PEMAZYRE if needed.
 - Tell your healthcare provider right away if you develop any muscle cramps, or numbness or tingling around your mouth.

The most common side effects of PEMAZYRE include:

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| <ul style="list-style-type: none"> • hair loss • diarrhea • nails separate from the bed or poor formation of the nail • feeling tired • change in sense of taste • nausea • constipation • mouth sores | <ul style="list-style-type: none"> • dry eyes • dry mouth • decrease in appetite • vomiting • joint pain • stomach-area (abdominal) pain • low phosphate in blood • back pain • dry skin |
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These are not all the possible side effects of PEMAZYRE. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects.

You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to Incyte Medical Information at 1-855-463-3463.

General information about the safe and effective use of PEMAZYRE.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use PEMAZYRE for a condition for which it is not prescribed. Do not give PEMAZYRE to other people, even if they have the same symptoms you have. It may harm them. If you would like more information, talk with your healthcare provider. You can ask your pharmacist or healthcare provider for information that is written for healthcare professionals. **Keep PEMAZYRE and all medicines out of the reach of children.**

Please see the Full Prescribing Information for PEMAZYRE at www.pemazyre.com.

About Incyte

Incyte is a Wilmington, Delaware-based, global biopharmaceutical company focused on finding solutions for serious unmet medical needs through the discovery, development and commercialization of proprietary therapeutics. For additional information on Incyte, please visit incyte.com and follow [@Incyte](https://twitter.com/Incyte).

Forward Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding whether confirmatory studies may be needed or may be successful, how long the approval of Pemazyre will be contingent and whether and when it might become final, whether and when the companion diagnostic will be approved by the FDA and whether it will help identify patients, the other studies in the FIGHT clinical trial program and the European MAA filing for pemigatinib, contain predictions, estimates and other forward-looking statements. These forward-looking statements are based on the Company's current expectations and subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: unanticipated delays; further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials; determinations made by the FDA; the Company's dependence on its relationships with its collaboration partners; the efficacy or safety of the Company's products and the products of the Company's collaboration partners; the acceptance of the Company's products and the products of the Company's collaboration partners in the marketplace; market competition; sales, marketing, manufacturing and distribution requirements; greater than expected expenses; expenses relating to litigation or strategic activities; and other risks detailed from time to time in the Company's reports filed with the Securities and Exchange Commission, including its Form 10-K for the year ending December 31, 2019. The Company disclaims any intent or obligation to update these forward-looking statements.

References

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