Incyte Announces Acceptance and Priority Review of NDA for Pemigatinib as a Treatment for Patients with Cholangiocarcinoma

November 27, 2019

WILMINGTON, Del.--(BUSINESS WIRE)--Nov. 27, 2019-- Incyte (Nasdaq:INCY) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review its New Drug Application (NDA) for pemigatinib, a selective fibroblast growth factor receptor (FGFR) inhibitor, as a treatment for patients with previously treated, locally advanced or metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements.

The NDA submission is based on data from the FIGHT-202 study evaluating pemigatinib as a treatment for patients with previously treated, locally advanced or metastatic cholangiocarcinoma. Study results, recently presented at the European Society for Medical Oncology (ESMO) 2019 Congress, demonstrated that in patients harboring FGFR2 fusions or rearrangements (Cohort A), pemigatinib monotherapy resulted in an overall response rate (ORR) of 36 percent (primary endpoint), and median duration of response (DOR) of 7.5 months (secondary endpoint) with a median follow-up of 15 months. Adverse events were manageable and consistent with the mechanism of action of pemigatinib.

“There is a significant need for new therapies for patients with cholangiocarcinoma, who have limited treatment options beyond first-line chemotherapy and often face a poor prognosis,” said Peter Langmuir, M.D., Group Vice President, Targeted Therapeutics, Incyte. “We are very pleased that the FDA has accepted our NDA for Priority Review which we believe represents an important step toward providing the first treatment option for patients with previously treated, locally advanced or metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements. We intend to work closely with the FDA to bring this innovative targeted therapy to patients suffering from this devastating disease as soon as possible.”

The FDA grants Priority Review to medicines that may offer a major advance in treatment where none currently exists. This designation shortens the review period to eight months compared to 12 months for Standard Review. The Prescription Drug User Fee Act (PDUFA) target action date is May 30, 2020.

Cholangiocarcinoma is a rare cancer that forms in the bile duct. It is classified based on its 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.[1,2] The incidence of cholangiocarcinoma varies regionally and ranges between 0.3 – 3.4 per 100,000 in North America and Europe.[1] FGFR2 fusions or rearrangements occur almost exclusively in iCCA, where they are observed in 10-16 percent of patients.[3-5]

About FIGHT-202

The FIGHT-202 Phase 2, open-label, multicenter study (NCT02924376) is evaluating the safety and efficacy of pemigatinib – a selective fibroblast growth factor receptor (FGFR) inhibitor – in adult (age ≥ 18 years) patients with previously treated, locally advanced or metastatic cholangiocarcinoma with documented FGF/FGFR status.

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 pemigatinib 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 is 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; progression free survival (PFS), overall survival (OS), duration of response (DOR), disease control rate (DCR) and safety in all cohorts.

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

About FIGHT

The FIGHT (**FI**broblast **G**rowth factor receptor in oncology and **H**ematology **T**rials) clinical trial program includes ongoing Phase 2 and 3 studies investigating safety and efficacy of pemigatinib therapy across several FGFR-driven malignancies. Phase 2 monotherapy studies include FIGHT-202, as well as FIGHT-201 investigating pemigatinib 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 pemigatinib plus pembrolizumab combination therapy and pemigatinib 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 recently initiated Phase 3 study investigating pemigatinib as a first-line treatment for patients with cholangiocarcinoma with FGFR2 fusions or rearrangements.

About FGFR and Pemigatinib

Fibroblast growth factor receptors (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.

Pemigatinib 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. The U.S. Food and Drug Administration (FDA) has granted pemigatinib Breakthrough Therapy designation for the treatment of previously treated, advanced/metastatic or unresectable FGFR2 translocated cholangiocarcinoma. The FDA's Breakthrough Therapy designation is designed to expedite the development and review of drugs for serious conditions that have shown encouraging early clinical results and may demonstrate substantial improvements over available medicines. Additionally, the FDA granted pemigatinib Orphan Drug designation for the treatment of cholangiocarcinoma, a designation granted to investigational compounds intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people.

About Incyte

Incyte Corporation is a Wilmington, Delaware-based biopharmaceutical company focused on the discovery, development and commercialization of proprietary therapeutics. For additional information on Incyte, please visit the Company's website at www.incyte.com.

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Forward Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding whether or when pemigatinib might be approved in the U.S. for the treatment of, and whether or when pemigatinib might provide a treatment option for, patients with previously treated, locally advanced or metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements, and the FIGHT clinical trial program. 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-Q for the quarter ending September 30, 2019. The Company disclaims any intent or obligation to update these forward-looking statements.

[1] Banales JM, et al. Nat Rev Gastroenterol Hepatol. 2016;13:261–280.
[2] Uhlig J, et al. Ann Surg Oncol. 2019;26:1993–2000.
[3] Graham RP, et al. Hum Pathol. 2014;45:1630–1638.
[4] Farshidfar F, et al. Cell Rep. 2017;18(11):2780–2794.
[5] Ross JS et al. The Oncologist. 2014;19:235–242.

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