Incyte Announces Japanese Approval of Pemazyre® (pemigatinib) for the Treatment of Patients with Myeloid/Lymphoid Neoplasms (MLNs)

March 27, 2023

WILMINGTON, Del. & TOKYO--(BUSINESS WIRE)--Mar. 27, 2023-- Incyte (Nasdaq:INCY) today announced that the Japanese Ministry of Health, Labour and Welfare (MHLW) has approved Pemazyre® (pemigatinib), a selective fibroblast growth factor receptor (FGFR) inhibitor, for the treatment of myeloid/lymphoid neoplasms (MLNs) with FGFR1 fusion (also known as 8p11 myeloproliferative syndrome). MLNs are a rare, aggressive group of cancers characterized by an over-production of myeloid cells, or bone tissue, with the tendency to rapidly progress to an acute myeloid leukemia (AML).

“The MHLW approval of Pemazyre in MLNs is an important step toward potentially providing a therapeutic option for Japanese patients with this rare condition,” said Lothar Finke, M.D., Ph.D., Group Vice President and General Manager, Incyte Asia. “Bringing this first and only approved therapy to MLN patients in Japan demonstrates Incyte’s commitment to finding solutions for critical unmet medical needs regardless of the size of the patient population – as is the case with MLNs, an extremely rare disease that is estimated to affect fewer than 100 patients worldwide.”

The approval was based on data from the Phase 2 FIGHT-203 study, a multicenter open-label, single-arm trial that evaluated the safety and efficacy of Pemazyre in 41 patients in myeloid or lymphoid neoplasms with FGFR1 fusion gene positive who received Pemazyre 13.5 mg orally once daily continuously or intermittently. The primary endpoint, investigator-assessed complete response rate, was 62.5% (95% CI: 45.8 - 77.3). The complete response rate in the continuous dosing population was 66.7% (95% CI: 46.0 - 83.5). The most common adverse reactions observed in patients receiving Pemazyre were hyperphosphatemia (70.7%), alopecia (56.1%), diarrhea (43.9%) and stomatitis (43.9%).

Previously, the MHLW granted Orphan Drug Designation (ODD) for Pemazyre – a designation granted to investigational compounds intended to treat rare diseases that affect fewer than 50,000 people in Japan, and for which there is a high medical need[1]. Designated orphan drugs are also eligible for priority review for marketing authorizations to ensure supply to clinical settings at the earliest opportunity[1]. The MLN ODD is the second such designation granted to Pemazyre by the MHLW, having also received ODD for cholangiocarcinoma, a type of biliary tract cancer.

MLNs with FGFR1 rearrangement are a form of very rare hematological cancers caused by a chromosomal abnormality (translocation) in which the chromosome breaks where the FGFR1 gene is located (position 11 in the short arm of chromosome 8: location 8p11) and fuses with fragments (genes) of other chromosomes. Various partner genes cause a constitutive activation of the FGFR1 tyrosine kinase, impacting cell proliferation and survival. These cancers are largely divided into two phases according to clinical presentation: the chronic phase, if diagnosed as myeloproliferative disorder or myelodysplastic syndrome, or acute phase, if diagnosed as acute leukemia. The prognosis is unfavorable, and while allogenic hematopoietic stem cell transplant is considered to be the only currently available treatment option that may achieve cure or long-term remission, no standard of care has been established.

About FIGHT-203

FIGHT-203 is a Phase 2, multicenter trial that enrolled patients 18 years and older with myeloid/lymphoid neoplasms (MLNs) with a fibroblast growth factor receptor 1 (FGFR1) rearrangement. Sponsored by Incyte, the study evaluated the safety and efficacy of pemigatinib for the treatment of adults with MLNs with FGFR1 rearrangement. Patients received pemigatinib 13.5 mg once daily in 21-day cycles, either on a continuous schedule (the approved recommended starting dosage for use in patients with MLNs with FGFR1 rearrangement) or as an intermittent schedule (14 days on, 7 days off, an unapproved dosage regimen in MLN with FGFR1 rearrangement). Pemigatinib was administered until disease progression or unacceptable toxicity or until patients were able to receive allo-HSCT. For more information about the study, please visit https://clinicaltrials.gov/ct2/show/NCT03011372.

About Pemigatinib

Pemazyre is a kinase inhibitor indicated in the United States 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[2]. This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Pemazyre is also the first targeted treatment approved for use in the United States for treatment of adults with relapsed or refractory myeloid/lymphoid neoplasms (MLNs) with FGFR1 rearrangement.

In Japan, Pemazyre is approved for the treatment of patients with unresectable biliary tract cancer (BTC) with a fibroblast growth factor receptor 2 (FGFR2) fusion gene, worsening after cancer chemotherapy and the treatment of myeloid/lymphoid neoplasms (MLNs) with FGFR1 rearrangement.

In Europe, Pemazyre is approved for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.

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, Europe and Japan.

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.

Pemazyre is a trademark of Incyte Corporation.

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.

For more information on Incyte Biosciences Japan G.K., please visit Incyte.jp.

Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including whether Pemazyre might provide a successful treatment option for patients with myeloid/lymphoid neoplasms with FGFR1 rearrangement, , contain predictions, estimates and other forward-looking statements.

These forward-looking statements are based on Incyte'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, MHLW and other regulatory authorities; Incyte's dependence on its relationships with its collaboration partners; the efficacy or safety of Incyte's products and the products of Incyte's collaboration partners; the acceptance of Incyte's products and the products of Incyte'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 Incyte's reports filed with the Securities and Exchange Commission, including its annual report for the year ending December 31, 2022. Incyte disclaims any intent or obligation to update these forward-looking statements.

Disclaimer

The drug information contained herein is intended for the disclosure of Incyte corporate information and is not intended to advertise or promote any medicinal product, including those under development.

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[1] Overview of Orphan Drug/Medical Device Designation System. Ministry of Health, Labour and Welfare. Available at: https://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/orphan_drug.html

[2] Pemazyre (pemigatinib) [Package Insert]. Wilmington, DE: Incyte; 2020.

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Source: Incyte