Incyte Announces U.S. Food and Drug Administration Grants Priority Review for Axatilimab for the Treatment of Chronic Graft-Versus-Host Disease

February 27, 2024

- Priority Review acceptance based on positive results of AGAVE-201 study

WILMINGTON, Del.--(BUSINESS WIRE)--Feb. 27, 2024-- Incyte (Nasdaq:INCY) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the Biologics License Application (BLA) for axatilimab, an anti-CSF-1R antibody, for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy. The Prescription Drug User Fee Act (PDUFA) date for the FDA decision is August 28, 2024.

The BLA is supported by positive data from the AGAVE-201 trial (NCT04710576), recently highlighted in a Plenary Scientific Session at the American Society of Hematology Annual Meeting 2023, which showed that treatment with axatilimab resulted in clinically meaningful results and was generally well-tolerated, with a safety profile that was manageable and consistent with the mechanism of action of CSF-1R inhibition.

Axatilimab is being developed by Incyte and Syndax Pharmaceuticals (Nasdaq:SNDX) as part of an exclusive worldwide co-development and co-commercialization license agreement.

“Despite recent advancements in the treatment of patients with chronic GVHD, there remains a significant unmet need for patients who progressed on earlier lines of therapy,” said Hervé Hoppenot, Chief Executive Officer, Incyte. “Axatilimab’s novel mechanism offers a differentiated treatment approach which may help patients suffering from this devastating disease. We look forward to working closely with the FDA and our partners at Syndax on the review of our application for axatilimab for this indication.”

The FDA grants Priority Review designation to applications for medicines that, if approved, would treat a serious condition and provide significant improvements in the safety or effectiveness of the treatment.

About Chronic Graft-Versus-Host Disease

Chronic graft-versus-host disease (GVHD), an immune response of the donor-derived hematopoietic cells against recipient tissues, is a serious, potentially life-threatening complication of allogeneic hematopoietic stem cell transplantation which can last for years. Chronic GVHD is estimated to develop in approximately 40% of transplant recipients, and affects approximately 14,000 patients in the U.S.[1,2]. Chronic GVHD typically manifests across multiple organ systems, with skin and mucosa being commonly involved, and is characterized by the development of fibrotic tissue[3].

About Axatilimab

Axatilimab is an investigational monoclonal antibody that targets colony stimulating factor-1 receptor, or CSF-1R, a cell surface protein thought to control the survival and function of monocytes and macrophages. In pre-clinical models, inhibition of signaling through the CSF-1 receptor has been shown to reduce the number of disease-mediating macrophages along with their monocyte precursors, which has been shown to play a key role in the fibrotic disease process underlying diseases such as chronic GVHD and idiopathic pulmonary fibrosis (IPF). Phase 1/2 data of axatilimab in chronic GVHD demonstrating its broad activity and tolerability were last presented at the 63rd American Society of Hematology Annual Meeting and data were published in the Journal of Clinical Oncology. Additionally, positive topline results and additional data from the Phase 2 AGAVE-201 trial highlighted in a Plenary Scientific Session at the American Association of Hematology Annual Meeting 2023 were announced. Axatilimab was granted Orphan Drug Designation by the U.S. FDA for the treatment of patients with chronic GVHD and IPF.

In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab. Axatilimab is being developed under an exclusive worldwide license from UCB entered into between Syndax and UCB in 2016.

About AGAVE-201 (NCT04710576)

The global Phase 2 AGAVE-201 dose-ranging trial evaluated the efficacy, safety, and tolerability of axatilimab in 241 adult and pediatric patients with recurrent or refractory active chronic GVHD whose disease had progressed after two prior therapies. Patients were randomized to one of three treatment groups that investigated a distinct dose of axatilimab administered at 0.3 mg/kg every two weeks, 1.0 mg/kg every two weeks or 3.0 mg/kg every four weeks. The trial's primary endpoint is the proportion of patients in each dose group who achieved an objective response as defined by 2014 NIH Consensus Criteria for chronic GVHD by cycle 7 day 1. Secondary endpoints include duration of response, percent reduction in daily steroids dose, organ specific response rates and validated quality-of-life assessments using the Modified Lee Symptom Scale.

For more information about AGAVE-201, visit https://www.clinicaltrials.gov/study/NCT04710576.

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.

Incyte Forward-looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding the AGAVE-201 trial, expectations regarding the BLA for axatilimab, and the potential for axatilimab to become a treatment option for chronic graft-versus-host disease, 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 U.S. FDA and other regulatory authorities outside of the United States; the efficacy or safety of Incyte and its partners' products; the acceptance of Incyte and its partners' products in the marketplace; market competition; sales, marketing, manufacturing and distribution requirements; and other risks detailed from time to time in Incyte's reports filed with the Securities and Exchange Commission, including its annual report on form 10-K for the year ended December 31, 2023. Incyte disclaims any intent or obligation to update these forward-looking statements.

1 SmartAnalyst 2020 SmartImmunology Insights chronic GVHD report.

2 Bachier, CR. et al. ASH annual meeting 2019; abstract #2109 Epidemiology and Real-World Treatment of Chronic Graft-Versus-Host Disease Post Allogeneic Hematopoietic Cell Transplantation: A U.S. Claims Analysis.

3 Kantar 2020 GVHD Expert Interviews N=32 interviews.

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Incyte:
Media
media@incyte.com

Investors
ir@incyte.com

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