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 affect approximately 50% of patients after allogeneic hematopoietic stem cell transplantation and can lead to significant morbidity and mortality.

The AGAVE-201 trial, sponsored by Syndax and Incyte, enrolled 241 patients with relapsed and refractory chronic GVHD who had received two or more prior systemic therapies. The trial was designed to evaluate the efficacy and safety of axatilimab, a novel antibody targeting inflammation and fibrosis through the inhibition of disease-associated macrophages. This inhibition leads to the resolution of fibrosis and inflammation, which are key drivers of chronic GVHD.

The AGAVE-201 trial met its primary endpoint across all dose cohorts with 74% of patients at the 0.3 mg/kg dose achieving a complete or partial response within the first six months of treatment. This result is significant as it underscores the efficacy of axatilimab patients with chronic GVHD.

In the overall trial population, 33% of patients experienced at least one grade ≥3 treatment-emergent adverse event (TEAE), with 15.5% experiencing adverse events leading to discontinuation of treatment. The recommended dose of axatilimab for future trials in chronic GVHD is 0.3 mg/kg every two weeks.

The AGAVE-201 pivotal trial enrolled 241 patients with relapsed and refractory chronic GVHD who had received two or more prior lines of systemic therapy. These data are featured in the Plenary Scientific Session at the 65th Annual Meeting of the American Society of Hematology (ASH 2023), held December 9-12, 2023, in San Diego and virtually.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20231210634247/en/

Incyte and Syndax expect to file a Biologics License Application (BLA) for axatilimab by year-end 2023.
develop in approximately 40% of transplant recipients, and affects approximately 14,000 patients in the U.S.\textsuperscript{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\textsuperscript{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 graft-versus-host disease (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 from the Phase 2 AGAVE-201 trial showing the trial met its primary endpoint were recently announced. Axatilimab was granted Orphan Drug Designation by the U.S. Food and Drug Administration 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**

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 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](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](https://www.incyte.com) and follow @Incyte.

**About Syndax**

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company’s pipeline include revumenib, a highly selective inhibitor of the Menin–KMT2A binding interaction, and axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor. For more information, please visit [www.syndax.com](http://www.syndax.com) or follow the Company on [Twitter](https://twitter.com/SyndaxPharma) and [LinkedIn](https://www.linkedin.com/company/syndax-pharmaceuticals/).

**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 submission of a BLA for axatilimab by year-end 2023, 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 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 and its quarterly report on Form 10-Q for the quarter ended September 30, 2023.

**Syndax Forward-looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as “may,” “will,” “expect,” “plan,” “anticipate,” “estimate,” “intend,” “believe” and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. These forward-looking statements are based on Syndax's expectations and assumptions as of the date of this press release. Each of these forward-looking statements involves risks and uncertainties. Actual results may differ materially from these forward-looking statements. Forward-looking statements contained in this press release include, but are not limited to, statements about the progress, timing, clinical development and scope of clinical trials, the reporting of clinical data for Syndax's product candidates, the potential filing of a BLA by year-end 2023, and the potential use of our product candidates to treat various cancer indications and fibrotic diseases. Many factors may cause differences between current expectations and actual results, including: unexpected safety or efficacy data observed during preclinical or clinical trials; clinical trial site activation or enrollment rates that are lower than expected; changes in expected or existing competition; changes in the regulatory environment; failure of Syndax's collaborators to support or advance collaborations or product candidates; and unexpected litigation or other disputes. Other factors that may cause Syndax's actual results to differ from those expressed or implied in the forward-looking statements in this press release are discussed in Syndax's filings with the U.S. Securities and Exchange Commission, including the "Risk Factors" sections contained therein. Except as required by law, Syndax assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

1 SmartAnalyst 2020 Smart Immunology Insights chronic GVHD report.


3 Kantar 2020 GVHD Expert Interviews N=32 interviews.