



Syndax Announces Results from the Pivotal AGAVE-201 Trial of Axatilimab in Chronic Graft-Versus-Host Disease to Be Featured in Plenary Session at the 65th ASH Annual Meeting

11.02.23

- Results support axatilimab's promising safety and efficacy profile, and reinforce its potential as a first-in-class CSF-1R monoclonal antibody in chronic graft-versus-host disease –
- Syndax and Incyte intend to file a Biologics License Application (BLA) by year-end 2023 –

WALTHAM, Mass., Nov. 2, 2023 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX), a clinical-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today announced that results from the pivotal AGAVE-201 trial of axatilimab, an anti-CSF-1R antibody, in adult and pediatric patients with chronic graft-versus-host disease (cGVHD), will be featured during the Plenary Scientific Session at the 65th American Society of Hematology (ASH) Annual Meeting being held December 9-12, 2023, in San Diego, California.

"Inclusion of AGAVE-201 in this year's ASH plenary session further supports our belief that axatilimab has the potential to serve as a highly differentiated therapeutic option for patients with chronic GVHD," said Michael A. Metzger, Chief Executive Officer. "We believe axatilimab's best-in-category profile and unique mechanism of action positions it as an important addition to the chronic GVHD treatment armamentarium, if approved. We look forward to sharing the full dataset next month."

The Company and its partner, Incyte, previously [announced](#) positive topline data from the pivotal AGAVE-201 trial of axatilimab in patients with cGVHD following two or more prior lines of therapy. All three dose cohorts, 0.3 mg/kg every two weeks, 1.0 mg/kg every two weeks, and 3.0 mg/kg every four weeks, met the primary endpoint. The overall response rate within the first six months of treatment at the 0.3 mg/kg dose was 74%, and 60% of these patients were still responding at one year. Furthermore, axatilimab was generally well tolerated, and the most common adverse events were consistent with on-target effects and prior trials. Syndax and Incyte expect to submit a BLA filing by year-end 2023.

Abstract Number: 1

Title: Safety and Efficacy of Axatilimab at 3 Different Doses in Patients with Chronic Graft-Versus-Host Disease (AGAVE-201)

Presenter: Daniel Wolff, M.D.

Session Name: Plenary Scientific Session

Session Date: Sunday, December 10, 2023

Session Time: 2:00 – 4:00 PM PT

Presentation Time: 2:00 PM PT

Axatilimab Preclinical Data

In addition, preclinical data detailing the anti-inflammatory and anti-fibrotic mechanism through which axatilimab is thought to impact the disease process in cGVHD will be featured during a poster session.

Details for the presentation are as follows:

Abstract Number: 2540

Title: Axatilimab Ameliorates Inflammation and Fibrosis by Targeting the Macrophages in a Preclinical Model of Chronic GVHD

Presenter: Anamika Bajpai, Ph.D.

Session Name: 201. Granulocytes, Monocytes, and Macrophages: Poster II

Session Date: Sunday, December 10, 2023

Presentation Time: 6:00 – 8:00 PM PT

A copy of each abstract is now available online via the ASH website at www.hematology.org.

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 tissue³.

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). 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 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 mg/kg every two weeks or 3 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://clinicaltrials.gov/ct2/show/NCT04710576>.

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 or follow the Company on [Twitter](#) and [LinkedIn](#).

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," "could," "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 submission 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.

References

1. SmartAnalyst 2020 SmartImmunity 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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† View original content: <https://www.prnewswire.com/news-releases/syndax-announces-results-from-the-pivotal-agave-201-trial-of-axatilimab-in-chronic-graft-versus-host-disease-to-be-featured-in-plenary-session-at-the-65th-ash-annual-meeting-301975502.html>

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