Syndax Pharmaceuticals Announces Updated Positive Data Demonstrating Broad Activity and Tolerability of Axatilimab in Patients with Chronic Graft-Versus-Host Disease

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- 68% overall response rate and broad multiorgan clinical benefit observed in highly refractory patients treated at doses being assessed in ongoing AGAVE-201 pivotal study -

- Axatilimab well-tolerated at all doses and schedules -

WALTHAM, Mass., Dec. 11, 2021 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. ("Syndax," the "Company" or "we") (Nasdaq: SNDX), a clinical-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today announced updated positive data from its Phase 1/2 trial of axatilimab in patients with recurrent or refractory chronic graft-versus-host disease (cGVHD) despite two or more prior lines of therapy. Axatilimab is the Company's anti-CSF-1R monoclonal antibody. The data are being featured during an oral session at the 63rd American Society of Hematology (ASH) Annual Meeting on Saturday, December 11, 2021 at 3:05 p.m. ET.

"There exists an urgent need for novel and effective therapies in patients with cGVHD," said Michael Meyers, M.D, M.P.H., Chief Medical Officer of Syndax. "The broad activity and tolerability observed underscores axatilimab's potential to play a meaningful role in the cGVHD treatment landscape, and further supports the importance of the ongoing AGAVE-201 trial."

"Durable responses and multiorgan clinical benefit reported today from the ongoing pivotal Phase 2 AGAVE-201 trial continue to support axatilimab's potential to serve as an intervention for patients with cGVHD," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "We are committed to maximizing the clinical impact of axatilimab across multiple lines of treatment in cGVHD as well as additional fibrotic diseases where the monocyte-macrophage lineage plays a vital role, such as idiopathic pulmonary fibrosis. We look forward to providing updates on our progress in the coming months."

A total of 40 patients with refractory disease who received a median of four prior systemic therapies, including ibrutinib, ruxolitinib, and belumosudil, were treated in the Company's Phase 1/2 trial of axatilimab. As of an October 22, 2021 data cutoff date, 31 patients treated at two of the doses being tested in the Company's ongoing AGAVE-201 global pivotal study were evaluable for response. A best ORR (complete response + partial response) of 72% (18/25) at 1mg/kg every two weeks and 50% (3/6) at 3mg/kg every four weeks was observed, for an ORR of 68% (21/31). Of note, responses were observed across a range of organ systems with difficult to treat manifestations such as lung (5/15), skin (3/28), and joints and fascia (16/24). Fifty-three percent of patients reported clinically meaningful improvement in their symptoms via the Lee Symptom Scale. As of the data cutoff date, 43% (17/40) of patients remained on treatment.

Axatilimab was well-tolerated with a favorable safety profile. The most common adverse events were consistent with on-target effects on liver enzyme pharmacology. There was no incidence of cytomegalovirus (CMV) or other viral reactivation, and no apparent increases in risk for infection.

Enrollment is ongoing in the Company's global pivotal Phase 2 AGAVE-201 trial of axatilimab in patients with cGVHD, with topline data expected in 2023. The trial will evaluate the safety and efficacy of three doses and schedules of axatilimab. The primary endpoint will assess objective response rate based on the 2014 NIH consensus criteria for cGVHD, with key secondary endpoints including duration of response and improvement in modified Lee Symptom Scale score.

A copy of today's presentation will be available in the Publications and Meeting Presentations section of Syndax's website.

About Chronic Graft-Versus-Host Disease

Chronic graft-versus-host disease (cGVHD), 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 (HSCT) 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 graft-versus-host disease (cGVHD) and idiopathic pulmonary fibrosis (IPF). Axatilimab data has demonstrated deep, durable responses and multiorgan clinical benefit in patients with cGVHD refractory to multiple therapeutic agents, and is currently being evaluated in the global pivotal Phase 2 AGAVE-201 trial in patients with cGVHD. Axatilimab was granted Orphan Drug Designation by the U.S. Food and Drug Administration for the treatment of patients with cGVHD and IPF. In September 2021, Syndax and Incyte entered into an exclusive worldwide collaboration and license agreement to develop and commercialize axatilimab. Axatilimab is being developed under an exclusive worldwide license from UCB entered into between Syndax and UCB in 2016.

About Syndax Pharmaceuticals, Inc.

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. The Company's pipeline includes SNDX-5613, a highly selective inhibitor of the Menin–MLL binding interaction, axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor, and entinostat, a class I HDAC inhibitor. For more information, please visit www.syndax.com or follow the Company on Twitter and LinkedIn.

Syndax's Cautionary Note on 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 and the reporting of clinical data for Syndax's product candidates, 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, the COVID-19 pandemic may disrupt our business and that of the third parties on which we depend, including delaying or otherwise disrupting our clinical trials and preclinical studies, manufacturing and supply chain, or impairing employee productivity, 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 in our Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on September 15, 2021, as well as in other filings we may make with the SEC in the future. 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.

Reference

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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View original content:https://www.prnewswire.com/news-releases/syndax-pharmaceuticals-announces-updated-positive-data-demonstrating-broad-activity-and-tolerability-of-axatilimab-in-patients-with-chronic-graft-versus-host-disease-301442638.html

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