Syndax Pharmaceuticals Announces Additional Positive Data from Phase 1 Trial of Axatilimab in Patients with cGVHD

December 6, 2020

- Data featured during oral session at the 62nd ASH Annual Meeting demonstrate deep, durable responses and multiorgan clinical benefit in patients refractory to multiple therapeutic agents -

- Enrollment in pivotal AGAVE-201 trial on track to start by year-end -

- Company to host conference call and webcast today at 2:00 p.m. ET-

WALTHAM, Mass., Dec. 6, 2020 /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 data from the Company's Phase 1 trial of axatilimab, its anti-CSF-1R monoclonal antibody, in patients with chronic graft versus host disease (cGVHD). The data will be featured today during an oral presentation at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition. A copy of the presentation will be available via Syndax's website at http://www.syndax.com/science/publications/.

"cGVHD is a highly underserved area in dire need of innovative new therapeutic options," said Mukta Arora, M.D., M.S., Professor of Medicine, Division of Hematology, Oncology and Transplantation at the University of Minnesota Medical School. "With sustained responses across several organ systems and clinically meaningful symptom improvements, findings from this trial suggest that axatilimab has the potential to play an important role in the cGVHD treatment paradigm. I look forward to seeing axatilimab explored further in the upcoming pivotal AGAVE-201 trial."

"The compelling data reported today demonstrate durable responses across all dose levels in several organs, including multiple organ-specific complete responses, and continue to support axatilimab's potential to serve as a novel, safe, and effective therapy for patients with cGVHD," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "We believe that axatilimab is the only agent in development for cGVHD that specifically targets the monocyte-macrophage lineage, which plays a key role in the fibrotic disease process underlying cGVHD and other diseases. The breadth and depth of clinical benefit observed in this trial may be attributed to this unique activity of axatilimab. We are pleased to confirm that we are on track to commence enrollment in the pivotal Phase 2 AGAVE-201 trial by year-end, with topline data anticipated in 2023."

As of October 30, 2020 (data cutoff), a total of 15 patients were enrolled in the Phase 1 portion of the trial across five dose cohorts. Of 14 evaluable patients, responses were observed in ~60% (n=8) of patients with refractory disease who received a median of four prior systemic therapies, including ibrutinib, ruxolitinib and belumosudil (formerly KD-025). Deep and sustained responses were observed at all dose levels in several organs, including the esophagus (n=1/1), lower gastrointestinal (GI) tract (n=1/1), mouth (n=5/9), joints/fascia (n=6/11), lungs (n=2/5), skin (n=4/10), and eyes (n=4/12). Of note, clinical benefit was seen in difficult to treat sclerodermatous cGVHD, and complete responses were observed in multiple organs, including the esophagus, lower GI tract, mouth, and eyes. As of the data cutoff date, 67% of evaluable patients (n=8/12) experienced a clinically meaningful improvement in symptoms, as measured by at least a 7-point decrease in Lee Symptom Scale score.

Axatilimab was generally safe and well-tolerated. The most common observed 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 remains ongoing in the Phase 2 portion of the Phase 1/2 trial at a dose of 1 mg/kg every two weeks.

The Company plans to commence a pivotal Phase 2 trial, AGAVE-201, to assess the safety and efficacy of different doses and schedules of axatilimab for the treatment of patients with cGVHD. The primary endpoint will assess objective response rate based on the 2014 NIH consensus criteria for GVHD, with key secondary endpoints including duration of response and improvement in modified Lee Symptom Scale score. The Company expects to begin enrollment by year-end, with topline data anticipated in 2023.

Conference Call and Webcast Details

The Company will host a conference call and webcast today, Sunday, December 6, 2020 at 2:00 p.m. ET. The event will feature a summary of the ASH data presentation, as well as a review of select patient case studies, and a discussion on how axatilimab may fit into the current and evolving treatment landscape. Expert participants will include lead author of the ASH presentation, Mukta Arora, M.D., M.S., Professor of Medicine, Division of Hematology, Oncology and Transplantation at the University of Minnesota Medical School, and co-author, Geoffrey Hill, M.D., José Carreras/E. Donnall Thomas Endowed Chair for Cancer Research and Director of The Immunotherapy Integrated Research Center at Fred Hutchinson Cancer Research Center.

The live audio webcast and accompanying slides may be accessed through the Events & Presentations page in the Investors section of the Company's website at www.syndax.com. Alternatively, the conference call may be accessed through the following:

Conference ID: 8698086
Domestic Dial-in Number: (855) 251-6663
International Dial-in Number: (281) 542-4259
Live webcast: https://edge.media-server.com/mmc/p/ddupdib6

For those unable to participate in the live conference call or webcast, a replay will be available on the Investors section of the Company's website, www.syndax.com.

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. cGVHD is estimated to develop in approximately 40% of transplant recipients, and affects approximately 14,000 patients in the U.S.[1,2] cGVHD 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, and block the development of cutaneous and pulmonary cGVHD. Axatilimab is currently being evaluated in a Phase 1/2 clinical trial in patients with cGVHD, and has demonstrated compelling clinical activity and a well-tolerated safety profile. Syndax plans to commence a pivotal trial, AGAVE-201, by the end of 2020.

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. 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 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 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:http://www.prnewswire.com/news-releases/syndax-pharmaceuticals-announces-additional-positive-data-from-phase-1-trial-of-axatilimab-in-patients-with-cgvhd-301186859.html

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