Syndax Announces Orphan Drug Designation Granted to Axatilimab for Treatment of Chronic Graft Versus Host Disease

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WALTHAM, Mass., March 31, 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 that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to axatilimab, its anti-CSF-1R monoclonal antibody, for the treatment of patients with chronic graft versus host disease (cGVHD).

"Receipt of Orphan Drug Designation underscores axatilimab's potential to serve as a safe and effective intervention for patients with cGVHD," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "Through its ability to inhibit monocyte derived macrophages, which play a key role in the fibrotic disease process, we believe axatilimab could represent a meaningful therapeutic approach for cGVHD, as well as other fibrotic diseases. As previously announced, our pivotal Phase 2 AGAVE-201 trial is now underway in patients with cGVHD, with topline results expected in 2023."

The FDA's Office of Orphan Drug Products grants Orphan Drug Designation to support drug candidates in development for underserved patient populations or rare disorders that affect fewer than 200,000 people in the U.S. Orphan Drug Designation qualifies a candidate for various development incentives, including tax credits for eligible clinical trials, waiver of application fees, and market exclusivity for seven years upon FDA approval.

At the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition in December 2020, Syndax reported updated data from its Phase 1 trial of axatilimab in patients with cGVHD which demonstrated deep, durable responses and multiorgan clinical benefit in patients refractory to multiple therapeutic agents. The Company recently announced that the pivotal Phase 2 AGAVE-201 trial, which will evaluate the safety and efficacy of three doses and schedules of axatilimab in patients with cGVHD, is now underway. 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 report topline data in 2023.

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 data has demonstrated deep, durable responses and multiorgan clinical benefit in patients refractory to multiple therapeutic agents to date, and is currently being evaluated in a pivotal Phase 2 AGAVE-201 trial in patients with cGVHD.

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.

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.

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[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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