Syndax Announces Orphan Drug Designation Granted to Axatilimab for Treatment of Idiopathic Pulmonary Fibrosis

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WALTHAM, Mass., April 8, 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 idiopathic pulmonary fibrosis (IPF).

"IPF is a serious, life-limiting orphan disease, and today's Orphan Drug Designation validates axatilimab's potential to serve as an effective therapeutic option for the currently-underserved patients living with this rare disease," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "Building on promising data demonstrating meaningful multiorgan clinical benefit in patients with chronic graft versus host disease, we are actively evaluating options to expand the axatilimab franchise into additional areas of high unmet need where the monocyte-macrophage lineage plays a key role in the fibrotic disease process. We look forward to providing updates on these plans in the coming months."

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.

About Idiopathic Pulmonary Fibrosis

Idiopathic Pulmonary Fibrosis (IPF) is a serious, life-limiting chronic lung disease characterized by fibrosis and scarring of lung tissue with a median survival of 3-5 years after diagnosis. Patients with IPF experience debilitating symptoms including progressive shortness of breath, particularly with exertion, chronic cough, fatigue, weakness, and chest discomfort. Currently approved drugs slow but do not halt disease progression and the only curative therapy is lung transplant, which is an option for less than 5% of patients. Estimates indicate that IPF could affect approximately 150,000 patients in the U.S. and approximately 260,000 patients across the seven major pharmaceutical markets (US, Japan, UK, Spain, Germany, Italy, and France).[1]

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. 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 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.

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 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 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] SMARTImmunology Insights. "Idiopathic Pulmonary Fibrosis." Presentation, March 2020.

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