Syndax Pharmaceuticals to Present Updated Data from SNDX-5613 and Axatilimab Clinical Programs During Oral Sessions at 63rd ASH Annual Meeting

November 4, 2021

- Robust clinical activity with durable responses and no discontinuations due to treatment-related adverse events observed in Phase 1 portion of AUGMENT-101 trial of SNDX-5613 in relapsed or refractory patients with genetically-defined acute leukemias -

- Phase 2 results highlight broad efficacy and tolerability of axatilimab at 1mg/kg in patients with relapsed or refractory cGVHD -

WALTHAM, Mass., Nov. 4, 2021 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. (Nasdaq: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today announced that updated data from both of its ongoing SNDX-5613 and axatilimab programs will be featured during oral sessions at the 63rd American Society of Hematology (ASH) Annual Meeting and Exposition being held December 11-14, 2021. SNDX-5613 is the Company's highly selective oral menin inhibitor. Axatilimab is Syndax's anti-CSF-1R monoclonal antibody.

"We are pleased to share that both our innovative pipeline programs will be highlighted during oral presentation sessions at the upcoming ASH Annual Meeting," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "For SNDX-5613, updated data from the Phase 1 portion of AUGMENT-101 continue to demonstrate robust clinical activity and a well-tolerated safety profile in heavily pretreated patients with mixed lineage leukemia rearranged (MLLr) or nucleophosmin (NPM1c) mutations."

"We are also excited to share updates from the Phase 1/2 trial of axatilimab for the treatment of chronic graft-versus-host disease (cGVHD). With additional patients and longer follow up, we have observed a high rate of durable responses and multiorgan clinical benefit in patients refractory to multiple therapeutic agents. We are encouraged by the continued trend we are seeing, notably in those treated in the 1 mg/kg Phase 2 expansion cohort, and look forward to presenting additional updated data for both programs in December."

SNDX-5613

A copy of the abstract published today can be viewed here. The oral presentation will include updated Phase 1 data from additional patients as of a more recent cutoff date, as well as further details on durability and complete response (CR) or CR with partial hematologic recovery (CRh) rate and mutational status.

The abstract highlights data from the Phase 1 portion of the Company's Phase 1/2 AUGMENT-101 trial of SNDX-5613 in patients with MLLr and NPM1c mutant relapsed/refractory (R/R) acute leukemias as of a June 29, 2021 data cutoff date. Of the 45 patients with MLLr or mNPM1 mutant leukemia who received at least one dose of SNDX-5613, the composite complete response (CRc: CR+CRh+CRp+CRi/MLFS) rate was 44% (n=20/45), with 14 of the 20 (70%) patients with a CRc showing no evidence of minimal residual disease (MRD-). The CR/CRh rate in this population was 22% (n=10/45). At the time of the data cut for the abstract, the median follow up was only 3.2 months and the median duration of response for patients achieving a CR/CRh was 5.2 months. Thirteen patients remained on treatment as of the data cutoff date. SNDX-5613 was generally safe and well-tolerated, with no study discontinuations due to treatment-related adverse events.

Oral Presentation Details:

- **Title**: Safety and Efficacy of Menin Inhibition in Patients (Pts) with MLL-Rearranged and NPM1 Mutant Acute Leukemia: A Phase (Ph) 1, First-in-Human Study of SNDX-5613 (AUGMENT 101)
- **Presenter**: Eytan Stein, M.D.
- **Session Name:** 616: Acute Myeloid Leukemias: Investigational Therapies, Excluding Transplantation and Cellular Immunotherapies: Targeted Therapies and Novel Therapies Session
- **Session Date:** Monday, December 13, 2021
- **Session Time:** 2:45 – 4:15 p.m. ET
- **Presentation Time:** 3:15 p.m. ET
- **Abstract/Publication Number**: 699

Axatilimab

A copy of the abstract published today can be viewed here. The oral presentation will include additional follow up on all patients enrolled.

The abstract published today highlights data from 40 patients treated in the Company's Phase 1/2 trial of axatilimab in patients with cGVHD as of a June 28, 2021 data cutoff date. A total of 38 patients were evaluable for response and demonstrated an overall response rate (ORR) of 66% (25/38). Thirty-two patients were treated at two of the doses being tested in the Company's ongoing AGAVE-201 global pivotal study, and 30 were evaluable for response at the time of the data cutoff. A best ORR (CR+partial response) of 75% (18/24) at 1mg/kg every two weeks and 50% (3/6) at 3mg/kg every four weeks was observed, with responses noted across organ systems including difficult to treat manifestations such as lung, skin, and joints and fascia. Axatilimab was generally safe and well-tolerated. Enrollment is ongoing in the pivotal Phase 2 AGAVE-201 trial of axatilimab in patients with cGVHD, with topline data expected in 2023.

Oral Presentation Details:

- **Title**: Safety, Tolerability, and Efficacy of Axatilimab, a CSF-1R Humanized Antibody, for Chronic Graft-Versus-Host

Disease after 2 or More Lines of Systemic Treatment

- **Presenter**: Stephanie Lee, M.D, M.P.H.
- **Session Name:** 722: Allogeneic Transplantation: Acute and Chronic GVHD, Immune Reconstitution: Treatment of Acute and Chronic Graft vs. Host Disease
- **Session Date:** Saturday, December 11, 2021
- **Session Time:** 2:00 – 3:30 p.m. ET
- **Presentation Time:** 3:00 p.m. ET
- **Abstract/Publication Number**: 263

About SNDX-5613

SNDX-5613 is a potent, selective, small molecule inhibitor of the menin-MLL binding interaction that is being developed for the treatment of mixed lineage leukemia rearranged (MLLr) acute leukemias including acute lymphoblastic leukemia (ALL) and acute myeloid leukemia (AML), and NPM1 mutant AML. In preclinical models of MLLr acute leukemias, SNDX-5613 demonstrated robust, dose-dependent inhibition of tumor growth, resulting in a marked survival benefit. Menin-MLL interaction inhibitors have also demonstrated robust treatment benefit in multiple preclinical models of NPM1 mutant AML, which represents the most frequent genetic abnormality in adult AML. SNDX-5613 is currently being evaluated in the Company's AUGMENT-101 Phase 1/2 open-label clinical trial for the treatment of relapsed/refractory (R/R) acute leukemias. SNDX-5613 was granted Orphan Drug Designation by the U.S. Food and Drug Administration for the treatment of patients with AML, and Fast Track designation for the treatment of adult and pediatric patients with relapsed or refractory acute leukemias harboring a mixed lineage leukemia rearranged MLLr or NPM1 mutation.

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 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 design, progress, timing, clinical development and scope of clinical trials, plans for initiating future clinical trials, reporting of clinical data for Syndax's product candidates, the association of data with treatment outcomes, 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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View original content:https://www.prnewswire.com/news-releases/syndax-pharmaceuticals-to-present-updated-data-from-sndx-5613-and-axatilimab-clinical-programs-during-oral-sessions-at-63rd-ash-annual-meeting-301416641.html

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