Syndax Pharmaceuticals Announces Presentation of Preclinical Data from Menin-MLL Program at the 60th American Society of Hematology Annual Meeting

- Preclinical data provide further support for the clinical application of Company's Menin-MLL program in acute leukemias-

WALTHAM, Mass., December 3, 2018 (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 the presentation of preclinical data from the Company's Menin-Mixed Lineage Leukemia (MLL) inhibitor program at the 60th American Society of Hematology (ASH) Annual Meeting in San Diego, California.

"Acute leukemias characterized by MLL-rearrangements (MLL-r) and nucleophosmin (NPM1) mutations represent areas of high unmet medical need, with 5-year survival rates falling below 50%," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "Data presented on VTP-50469, a Syndax Menin-MLL inhibitor, provides further support for developing this class of molecules for specific, genetically-defined acute leukemias. The robustness and consistency of the accumulated preclinical data seen with our Menin-MLL inhibitors provide support for an anticipated IND filing on our lead compound, SNDX-5613, in the second quarter of 2019."

In an oral presentation at ASH, Hannah Uckelmann, Ph.D., Dana-Farber Cancer Institute, presented new preclinical data demonstrating that NPM1 mutant progenitor cells can act as drivers of leukemic transformation, and that VTP-50469 can block their pathological capacity by disrupting the menin-MLL interaction. In addition, data generated using a mouse PDX model of NPM1 mutant acute myeloid leukemia (AML) demonstrated that single agent treatment with VTP-50469 eradicated pre-leukemic NPM1 mutant cells and prevented leukemia development, resulting in a marked survival benefit. These data lend further support to the potential therapeutic utility of menin inhibitors in the setting of NPM1 mutant AML.

Furthermore, during a Scientific Spotlight Session at ASH, Scott Armstrong, M.D., Ph.D., Dana-Farber Cancer Institute, provided an overview of the multiple complexes that influence gene expression in MLL-r leukemias and NPM1 AML. Additional preclinical data generated in his laboratory further substantiate that inhibition of the Menin-MLL interaction can decrease leukemic burden and prolong survival in mouse PDX models of both MLL-r and NPM1 mutant leukemias. These data build on prior encouraging preclinical results presented at the American Association for Cancer Research Annual Meeting in April, and further underscore the potential therapeutic utility of menin inhibitors as modulators of critical epigenetic mechanisms in acute human leukemias, including subsets of both acute lymphoblastic leukemia (ALL) and AML.

Oral Presentation:

Title: MLL-Menin Inhibition Reverses Pre-Leukemic Progenitor Self-Renewal Induced By NPM1 Mutations and Prevents AML Development
Presenter: Hannah Uckelmann, Ph.D., Research Fellow at the Dana-Farber Cancer Institute
Publication Number: 546

Scientific Spotlight Session:

Title: Targeting Chromatin Complexes in MLL Rearranged Leukemia
Presenter: Scott Armstrong, M.D., Ph.D., Chairman of the Department of Pediatric Oncology at the Dana-Farber Cancer Institute, Associate Chief of the Division Hematology/Oncology at Boston Children's Hospital, and the David G. Nathan Professor of Pediatrics at Harvard Medical School

Both presentations are available in the Publications section of the Company's website, www.syndax.com.

About MLL Rearranged Leukemias

Rearrangements of the MLL gene give rise to an acute leukemia, MLL-r. MLL-r occurs in ~80% of infant acute leukemias and up to 10% of adult acute leukemias. It is associated with a poor prognosis, with less than 50% of infants with MLL-r surviving past 5 years. MLL rearrangements produce fusion proteins that require interaction with a protein called Menin in order to drive leukemic cancer growth. Disruption of the Menin-MLL-r interaction has been shown to halt the growth of MLL-r leukemic cells. MLL-r leukemias are routinely diagnosed through currently available cytogenetic screening techniques in leukemic cells, but there are currently no approved therapies indicated for MLL-r leukemias.

About NPM1c Acute Myeloid Leukemia

NPM1c represents another discrete form of acute myeloid leukemia (AML) distinguished by point mutations in the NPM1 gene that drives the leukemic phenotype. NPM1c is the most common type of cytogenetically normal AML and represents ~30% of all diagnosed AML. This subtype of AML has a poor prognosis, with a 5-year overall survival rate of ~50%. Similar to MLL-r leukemias, NPM1c AML is highly dependent on the expression of specific developmental genes, shown to be negatively impacted by inhibitors of the menin-MLL1 interaction. NPM1c AML is routinely diagnosed through currently available screening techniques in leukemic cells, but there are currently no approved therapies indicated for NPM1c AML.

About Syndax Pharmaceuticals, Inc.

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. The Company is developing its lead product candidate, entinostat, a once-weekly, oral, small molecule, class I HDAC inhibitor, in combination with exemestane and several approved PD-1/PD-(L)1 antagonists. The Company's pipeline also includes SNDX-6352, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor, as well as a portfolio of potent and selective inhibitors targeting the binding interaction of Menin with MLL-r (including its lead candidate SNDX-5613). 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 studies, clinical trial site activation or enrollment rates that are lower than expected, changes in expected or existing competition, changes in the regulatory environment, 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.

Syndax Contacts

Investor Contact
Melissa Forst
Argot Partners
melissa@argotpartners.com
Tel 212.600.1902

Media Contact
David Rosen
Argot Partners
david.rosen@argotpartners.com
Tel 212.600.1902

SNDX-G