Syndax Pharmaceuticals Announces FDA Clearance of IND Application for Targeted Menin Inhibitor SNDX-5613 for Relapsed/Refractory Acute Leukemias

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-- SNDX-5613 is a potent, highly selective, oral inhibitor of the interaction of Menin with the Mixed Lineage Leukemia (MLL) protein --

-- Strong tumor growth inhibition and improved survival observed in preclinical models --

WALTHAM, Mass., July 10, 2019 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. (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 cleared the Company's Investigational New Drug (IND) application to begin a Phase 1/2 trial for SNDX-5613, a targeted Menin inhibitor, in patients with relapsed/refractory (R/R) acute leukemias.

"The FDA's acceptance of our IND for SNDX-5613 represents an important event in the development of targeted therapies for patients with acute leukemias," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "The advancement of SNDX-5613 builds on three decades of scientific investigation that explored the Menin-MLL-r interaction and its importance in a subset of genetically defined leukemias. Our preclinical results strongly support the therapeutic potential of SNDX-5613 for patients with MLL-r and NPM1 mutant leukemias, many of whom do not derive a durable benefit from existing treatments. We look forward to moving this program into the clinic."

The Phase 1/2 open-label trial will assess orally administered SNDX-5613 in patients with R/R acute leukemias. The Phase 1 portion of the study will assess the safety, tolerability and pharmacokinetics of SNDX-5613, and will seek to establish a recommended Phase 2 dose. The Phase 2 portion will evaluate efficacy, as defined by Complete Remission rate, across three expansion cohorts enrolling adult patients with MLL-rearranged (MLL-r) acute lymphoblastic leukemia (ALL), MLL-r acute myeloid leukemia (AML), and NPM1 mutant AML.

MLL rearrangements occur in approximately 80% of acute leukemia cases in infants and up to 10% of all leukemias. In preclinical models of MLL-r 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 pre-clinical models of NPM1 mutant AML, which represents the most frequent genetic abnormality in adult AML.

About Mixed Lineage Leukemia Rearranged (MLL-r)

Rearrangements of the MLL gene give rise to MLL-r acute leukemias, known to have a poor prognosis, with less than 55% of patients surviving past 5 years. MLL rearrangements produce fusion proteins that require interaction with the protein called Menin 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, which are routinely diagnosed through currently available cytogenetic or molecular diagnostic techniques, occur in approximately 80% of infant acute leukemias and up to 10% of all acute leukemias. There are currently no approved therapies indicated for MLL-r leukemias.

About NPM1 Mutant Acute Myeloid Leukemia

NPM1 mutant AML, which is distinguished by point mutations in the NPM1 gene that drive the leukemic phenotype, is the most common type of cytogenetically normal adult AML and represents approximately 30% of all adult AML cases. This subtype of AML has a 5-year overall survival rate of approximately 50%. Similar to MLL-r leukemias, NPM1 mutant AML is highly dependent on the expression of specific developmental genes, shown to be negatively impacted by inhibitors of the Menin-MLL interaction. NPM1 mutant AML is routinely diagnosed through currently available screening techniques. There are currently no approved therapies indicated for NPM1 mutant AML.

About Syndax Pharmaceuticals, Inc.

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. The Company's lead product candidate, entinostat, a once-weekly, oral, small molecule, class I HDAC inhibitor, is being tested in a phase 3 combination trial with exemestane for treatment of advanced HR+, HER2- breast cancer and has been evaluated in combination with 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, and SNDX-5613, a highly selective inhibitor of the Menin–MLL binding interaction. 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, 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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