SNDX-5613 Granted FDA Fast Track Designation for the Treatment of Relapsed/Refractory Acute Leukemias

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WALTHAM, Mass., June 28, 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 Fast Track Designation (FTD) to SNDX-5613 for the treatment of adult and pediatric patients with relapsed or refractory acute leukemias harboring a mixed lineage leukemia rearranged (MLLr) or nucleophosmin (NPM1) mutation. SNDX-5613 is the Company's highly selective, oral menin inhibitor.

"Genetically-defined acute leukemias represent an underserved area marked by particularly poor prognosis and limited therapeutic options," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "As we move toward initiating our pivotal study, receipt of FTD from the FDA underscores SNDX-5613's potential to meaningfully improve outcomes for patients with MLLr and NPM1 mutant acute leukemias."

About Fast Track Designation

Fast Track Designation is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fulfill an unmet medical need, enabling drugs to reach patients earlier. The FDA created this process to help deliver important new drugs to patients earlier and it covers a broad range of serious illnesses. These clinical programs may also be eligible to apply for Accelerated Approval and Priority Review if relevant criteria are met.

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 Mixed Lineage Leukemia Rearranged Acute Leukemias

Rearrangements of the MLL gene give rise to mixed lineage leukemia rearranged (MLLr) acute leukemias known to have a poor prognosis, with less than 25% of adult patients surviving past five years. MLL rearrangements produce fusion proteins that require interaction with the protein called menin to drive leukemic cancer growth. Disruption of the menin-MLLr interaction has been shown to halt the growth of MLLr leukemic cells. MLLr 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 MLLr leukemias.

About NPM1 Mutant Acute Myeloid Leukemia

NPM1 mutant acute myeloid leukemia (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 five year overall survival rate of approximately 50%. Similar to mixed lineage leukemia rearranged (MLLr) 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 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 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 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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