Syndax Announces First Patient Dosed in Phase 1/2 AUGMENT-101 Trial of SNDX-5613 for the Treatment of Adults with Relapsed/Refractory Acute Leukemias

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- Company expects to report initial clinical data in 2020 -

WALTHAM, Mass., Nov. 6, 2019 /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 first patient has been dosed in AUGMENT-101, a Phase 1/2 clinical trial evaluating SNDX-5613, Syndax's potent, highly selective oral Menin inhibitor, in patients with relapsed/refractory (R/R) acute leukemias.

"Dosing of the first patient in AUGMENT-101 represents an important milestone, both for the SNDX-5613 program and, importantly, for patients with genetically-defined acute leukemias, many of whom often do not achieve durable benefit from currently available treatment regimens," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "Preclinical data strongly support our belief that SNDX-5613 has the potential to address a significant unmet need as a treatment for patients with MLL-r and NPM1 mutant leukemias."

AUGMENT-101 is a Phase 1/2 open-label trial designed to evaluate the efficacy, safety, tolerability and pharmacokinetics of orally administered SNDX-5613. The Phase 1 dose escalation portion of AUGMENT-101 will enroll adults with R/R acute leukemias and establish a recommended Phase 2 dose. The Phase 2 portion will evaluate efficacy, as defined by Complete Response rate (per International Working Group response criteria), across three expansion cohorts: MLL-rearranged (MLL-r) acute lymphoblastic leukemia (ALL), MLL-r AML and NPM1 mutant AML. The Company expects to report initial clinical data from the trial in 2020.

Additional information about the AUGMENT-101 trial is available via Clinicaltrials.gov (NCT 04065399).

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 MLL-rearranged (MLL-r) acute leukemias, including acute lymphoblastic leukemia (ALL) and acute myeloid leukemia (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 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 acute leukemias.

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.

Syndax Contacts

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

Media Contact
Craig Heit
GCI Health
craig.Heit@gcihealth.com
Tel 347.451.4733

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