Syndax Announces Preclinical Results Supporting Development of its Portfolio of Menin Inhibitors In Mixed Lineage Leukemias

December 9, 2019

- Data provide detailed look at potential underlying mechanism of action within cells -

- Results support ongoing development of SNDX-5613, Company's lead highly selective Menin inhibitor, for treatment of MLL-rearranged acute leukemias -

WALTHAM, Mass., Dec. 9, 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 the publication of a preclinical report demonstrating that selective inhibition of the Menin-MLL interaction, provides consistent anti-proliferative and anti-leukemic activity across multiple mixed lineage leukemia rearranged (MLLr) samples. The article, "A Menin-MLL inhibitor induces specific chromatin changes and eradicates disease in models of MLL-rearranged leukemia," was published in the December 9 issue of *Cancer Cell*; the article is also available online.

This study, which was led by researchers at Dana-Farber Cancer Institute and Children's Cancer Institute, Sydney, Australia, examined the activity of VTP-50469, a close analog of the clinical lead SNDX-5613, against a range of MLLr harboring cell lines and patient-derived xenograft (PDX) models. Cell lines carrying MLL rearrangements were selectively responsive to VTP-50469, triggering disruption of menin containing transcription complexes and causing changes to gene expression that induced terminal differentiation and cell death. In PDX models, of both acute lymphoblastic leukemia (ALL) and acute myeloid leukemia (AML) subtypes of MLLr, single agent treatment with the Menin-MLL interaction inhibitor significantly reduced leukemia burden and led to profound survival benefit, with many mice remaining disease free more than one year after treatment.

"The newly developed Menin-MLL inhibitor demonstrated remarkable single-agent activity in PDX models of human MLL-rearranged leukemia including disease eradication," said Scott A. Armstrong, M.D., Ph.D., President, Dana-Farber/Boston Children's Cancer and Blood Disorders Center, and Chairman, Department of Pediatric Oncology, Dana-Farber Cancer Institute and senior author of the study. "This level of activity is unusual for single agent treatments in leukemia models."

"For patients with genetically-defined acute leukemias, there exists a dire unmet need for novel and effective therapeutic options," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "We are encouraged by these preclinical data, which continue to support our belief that SNDX-5613, our lead Menin inhibitor, has the potential to overcome the limitations of currently approved regimens, many of which do not yield a durable benefit. We are hopeful that these findings will translate positively in our ongoing Phase 1/2 AUGMENT-101 trial, for which we continue to expect initial data in 2020."

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 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
craig.Heit@gcihealth.com
Tel 347.451.4733

SNDX-G

View original content:http://www.prnewswire.com/news-releases/syndax-announces-preclinical-results-supporting-development-of-its-portfolio-of-menin-inhibitors-in-mixed-lineage-leukemias-300971580.html

SOURCE Syndax Pharmaceuticals