Syndax Announces Completion of Enrollment in AUGMENT-101 Pivotal Trial Cohort of Patients with Relapsed/Refractory mNPM1 Acute Myeloid Leukemia

March 28, 2024

– Topline data expected in 4Q24 could support sNDA filing in 1H25 –

– Revumenib has the potential to address $2 billion mNPM1 and KMT2Ar R/R acute leukemia U.S. market opportunity –

WALTHAM, Mass., March 28, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today announced completion of enrollment in the AUGMENT-101 pivotal trial cohort of patients with relapsed/refractory (R/R) mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML). Topline data is expected in the fourth quarter of 2024 and could support a supplemental New Drug Application (sNDA) filing for revumenib in R/R mNPM1 AML in the first half of 2025.

"We are thrilled to announce that we are one step closer to potentially expanding the therapeutic reach of revumenib in genetically defined acute leukemias," said Neil Gallagher, M.D., Ph.D., President, Head of Research and Development at Syndax. "We look forward to reporting topline data for this pivotal cohort in the fourth quarter of this year, which will follow closely behind a potential first approval of KMT2A acute leukemia in the third quarter."

Michael A. Metzger, Chief Executive Officer, added, "With revumenib and axatilimab, two first-and best-in-class drugs, expected to launch in 2024 and the potential to expand beyond first approvals and into additional indications, Syndax is well positioned to deliver on its mission to improve the lives of cancer patients and create meaningful long-term value for shareholders."

Sixty-four (64) adult and up to 20 pediatric patients with mNPM1 AML have been enrolled into the pivotal portion of AUGMENT-101, a pivotal trial designed to evaluate the safety, tolerability, pharmacokinetics, and efficacy of orally administered revumenib. The primary endpoint for the trial is efficacy as measured by complete remission (CR) or a CR with partial hematological recovery (CRh) rate (CR + CRh) per protocol, with secondary endpoints including duration of response (DOR) and overall survival (OS).

A new drug application (NDA) is under review by the U.S. Food and Drug Administration (FDA) for revumenib in R/R KMT2Ar acute leukemia with a Prescription Drug User Fee Act (PDUFA) action date of September 26, 2024. The NDA submission is supported by positive data from the AUGMENT-101 pivotal trial cohort of revumenib in adult and pediatric patients with KMT2Ar AML and acute lymphoblastic leukemia (ALL).

About Revumenib

Revumenib is a potent, selective, small molecule inhibitor of the menin-KMT2A binding interaction that is being developed for the treatment of KMT2A-rearranged, also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including ALL and AML, and NPM1-mutant AML. Positive topline results from the Phase 2 AUGMENT-101 trial in R/R KMT2Ar acute leukemia showing the trial met its primary endpoint were presented at the 65th American Society of Hematology Annual Meeting and data from the Phase 1 portion of AUGMENT-101 in acute leukemia was published in Nature. Revumenib was granted Orphan Drug Designation by the FDA and European Commission for the treatment of patients with AML, and Fast Track designation by the FDA for the treatment of adult and pediatric patients with R/R acute leukemias harboring a KMT2A rearrangement or NPM1 mutation. Revumenib was granted Breakthrough Therapy Designation (BTD) by the FDA for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement.

About NPM1-Mutant Acute Myeloid Leukemia

NPM1-mutant AML, which is distinguished by 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 KMT2A-rearranged acute leukemia, NPM1-mutant AML is highly dependent on the expression of specific developmental genes shown to be negatively impacted by inhibitors of the menin-KMT2A 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

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include revumenib, a highly selective inhibitor of the menin–KMT2A binding interaction, and axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor. For more information, please visit. For more information, please visit www.syndax.com or follow the Company on X (formerly Twitter) and LinkedIn.

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 "anticipate," "believe," "could," "estimate," "expects," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative or plural of those terms, 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, the reporting of clinical data for Syndax's product candidates, and the potential use of its 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; 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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