Syndax Announces FDA Priority Review of NDA for Revumenib for the Treatment of Relapsed/Refractory KMT2Ar Acute Leukemia

March 26, 2024

– PDUFA action date set for September 26, 2024 –

– NDA being reviewed under FDA's RTOR program –

WALTHAM, Mass., March 26, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (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 Priority Review for its New Drug Application (NDA) for revumenib, the Company's first-in-class menin inhibitor, for the treatment of adult and pediatric relapsed or refractory (R/R) KMT2A-rearranged (KMT2Ar) acute leukemia. The NDA filing is being reviewed under the FDA's Real-Time Oncology Review Program (RTOR) and has been assigned a Prescription Drug User Fee Act (PDUFA) target action date of September 26, 2024. RTOR allows for a more efficient review and close engagement between the sponsor and the FDA throughout the submission process, which historically has led to earlier approvals.

"The receipt of Priority Review for the revumenib NDA filing is a significant milestone as we transition to a leading commercial-stage oncology company with the planned launches of two first- and best-in class drugs in 2024," said Michael A. Metzger, Chief Executive Officer. "With two regulatory filings now under FDA Priority Review, our team is focused on commercial preparations to enable Syndax's continued success as we enter this next stage of growth."

The NDA submission is supported by positive data from the pivotal AUGMENT-101 trial of revumenib in adult and pediatric patients with KMT2Ar acute myeloid leukemia (AML) and acute lymphoid leukemia (ALL). As previously reported, the trial met its primary endpoint at the protocol-defined interim analysis with a complete remission (CR) or a CR with partial hematological recovery (CRh) rate of 23% (13/57; 95% confidence interval [CI]: [12.7, 35.8, one-sided p-value = 0.0036]) among the 57 efficacy evaluable patients in the pooled KMT2Ar acute leukemia population. 70% of patients who achieved a CR/CRh and were assessed for minimal residual disease (MRD) were MRD negative. Additionally, 63% (36/57) of the efficacy-evaluable patients achieved an overall response, 39% (14/36) of whom underwent hematopoietic stem cell transplant (HSCT), with 50% (7/14) restarting revumenib as post-transplant maintenance at the time of the data cutoff.

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 Real-Time Oncology Review (RTOR)

RTOR provides a more efficient review process for oncology drugs to ensure that safe and effective treatments are available to patients as early as possible, while improving review quality and engaging in early iterative communication with the applicant. Specifically, it allows for close engagement between the sponsor and the FDA throughout the submission process and it enables the FDA to review individual sections of modules of a drug application rather than requiring the submission of complete modules or a complete application prior to initiating review. Additional information about RTOR can be found at: https://www.fda.gov/about-fda/oncology-center-excellence/real-time-oncology-review-pilot-program

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 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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