Syndax Presents Positive Pediatric Data from Pivotal AUGMENT-101 Trial of Revumenib in Relapsed/Refractory KMT2Ar Acute Leukemia at ASPHO Plenary Session

April 8, 2024

– Consistent safety and efficacy profiles across adult and pediatric populations –

– Majority of pediatric patients who achieved an overall response proceeded to transplant; patients receiving post-transplant revumenib maintenance remained in remission as of data cutoff date –

– No treatment-related discontinuations or dose reductions –

WALTHAM, Mass., April 8, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (NASDAQ: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today announced the presentation of positive data from the pivotal AUGMENT-101 trial in pediatric patients with relapsed/refractory (R/R) KMT2A-rearranged (KMT2Ar) acute myeloid leukemia (AML) and acute lymphoid leukemia (ALL) treated with revumenib, a first-in-class menin inhibitor. The pediatric data was featured in a Plenary Session titled "Pivotal Phase 2 Results of AUGMENT-101 for Revumenib in KMT2Ar Acute Leukemia: Pediatric Experience" at the 2024 American Society of Pediatric Hematology/Oncology (ASPHO) Conference held April 3 – 6, 2024 in Seattle, Washington.

"We are pleased to present positive results from pediatric patients treated with revumenib in the AUGMENT-101 pivotal trial demonstrating impressive activity and consistency with the adult population," said Neil Gallagher, M.D., Ph.D., President, Head of Research and Development at Syndax. "As the only menin inhibitor with a formulation designed for the pediatric setting, we have an ongoing commitment to bringing this first- and best-in-class therapeutic agent to this important patient population in need of effective treatments."

The presented data include efficacy and safety findings for pediatric patients with R/R KMT2Ar acute leukemia from the pivotal Phase 2 portion of the AUGMENT-101 study. Of the 57 patients enrolled in AUGMENT-101 with central confirmation of their KMT2Ar status and sufficient follow-up to be included in the efficacy-evaluable population, 13 (23%) were less than 18 years old with a median age of 5 years. The pediatric patients were heavily pretreated with a median of 4 prior lines of therapy including 8 (62%) that received prior venetoclax, 2 (15%) that received CAR-T and 6 (46%) that received prior hematopoietic stem cell transplant (HSCT).

In this population, the complete remission (CR) or CR with partial hematological recovery (CRh) rate was 23% (3/13; 95% CI: 5.0, 53.8), with a median time to CR or CRh of 2.3 months (95% CI: 1.0, 3.9). The overall response rate[1] was 46% (6/13), and the composite response rate[2] (CRc) was 39% (5/13). Sixty percent (3/5) of CRc patients achieved minimal residual disease negative status. The median overall survival was 6.9 months (95% CI, 2.3–not reached). Four (67%) of the 6 patients who achieved an overall response underwent HSCT, two of whom did not achieve a CR or CRh prior to transplant. Half (2/4) of the patients who underwent HSCT received post-transplant maintenance with revumenib and had been in remission for 6 and 9 months at the time of the July 24, 2023 data cutoff.

Revumenib was well-tolerated, and the safety profile was consistent with the Company's previously reported data. In the safety-evaluable patient population, Grade 3 or greater treatment-related adverse events that occurred in greater than 10% of patients included febrile neutropenia (13%; 3/23) and decreased neutrophil count (13%; 3/23). Grade 3 or greater differentiation syndrome was observed in 9% (2/23) of patients and Grade 3 QTc prolongation was observed in 4% (1/23) of patients. No treatment-related discontinuations or dose reductions due to adverse events occurred in the trial.

A copy of the presentation is available in the Publications and Meeting Presentations section of Syndax's website.

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 with a CR/CRh rate of 23% (13/57; 95% confidence interval [CI]: [12.7, 35.8, one-sided p-value = 0.0036]) were presented at the 65th American Society of Hematology Annual Meeting. 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 by the FDA for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement. The New Drug Application (NDA) for revumenib for the treatment of adult and pediatric R/R KMT2Ar acute leukemia was granted Priority Review by the FDA with a Prescription Drug User Fee Act target action date of September 26, 2024, under the Real-Time Oncology Review Program.

About AUGMENT-101

AUGMENT-101 is a Phase 1/2 open-label trial designed to evaluate the safety, tolerability, pharmacokinetics, and efficacy of orally administered revumenib. The Phase 1 dose escalation portion of AUGMENT-101 included two cohorts based on concomitant treatment with a strong CYP3A4 inhibitor. Arm A enrolled patients not receiving a strong CYP3A4 inhibitor, while Arm B enrolled patients receiving a strong CYP3A4 inhibitor. The Phase 2 pivotal portion of AUGMENT-101 has enrolled R/R patients across the following trial populations: patients with mNPM1 AML, patients with KMT2Ar AML, and patients with KMT2Ar ALL. The primary endpoint for each of the cohorts is efficacy as measured by complete remission rate (CR + CRh) per protocol, with secondary endpoints including duration of response and overall survival (OS). Positive data from the pivotal AUGMENT-101 trial of revumenib in adult and pediatric patients with KMT2Ar AML and ALL served as the basis for the NDA submission that is currently under review by the FDA. Enrollment has been completed in the AUGMENT-101 pivotal trial cohort of patients with R/R mNPM1 AML and topline data is expected in the fourth quarter of 2024.

About KMT2A (MLL) Rearranged Acute Leukemia

Rearrangements of the KMT2A (mixed lineage leukemia or MLL) gene give rise to KMT2Ar acute leukemia that is known to have a poor prognosis. KMT2A genes produce fusion proteins that require interaction with the protein called menin to drive leukemic cancer growth. Disruption of the menin-KMT2Ar interaction has been shown to halt the growth of KMT2Ar leukemic cells. KMT2Ar acute leukemia can phenotypically appear as AML, ALL, or mixed phenotype acute leukemia (MPAL) and is routinely diagnosed through currently available cytogenetic or molecular diagnostic techniques. The median overall survival (OS) after standard of care first-line treatment, including intensive chemotherapy and transplant, is less than one year and the majority of patients suffer relapse within five years. With third line treatment or beyond, less than 5% of patients achieve complete remission, and the median OS is less than three months. There are currently no approved therapies indicated for KMT2A-rearranged acute leukemia.

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.

References

[1] Overall response rate includes CR, CRh, CRp, CRi, MLFS, and PR

[2] Composite response rate includes CR, CRh, CRp, CRi

CR = Complete remission
CRh = Complete remission with partial hematologic recovery
CRp = Complete remission with incomplete platelet recovery
CRi = Complete remission with incomplete count recovery
MLFS = Morphologic leukemia-free state
PR = Partial response

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