Syndax Highlights Recent Updates and Anticipated 2024 Milestones

January 2, 2024

– BLA submitted for axatilimab in chronic graft-versus-host disease; Syndax exercised option to co-commercialize axatilimab in the U.S. with Incyte –

– NDA submitted for revumenib in R/R KMT2Ar acute leukemia under U.S. FDA's RTOR program –

– Completion of enrollment in AUGMENT-101 mNPM1 patient cohort expected in late 1Q/early 2Q with topline data anticipated in 4Q24 –

– Completion of $230 million follow-on offering in December extends runway through 2026 –

WALTHAM, Mass., Jan. 2, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today highlighted recent updates and anticipated 2024 milestones.

"With positive pivotal data readouts for both revumenib and axatilimab and subsequent presentations at the American Society of Hematology Annual Meeting, 2023 was truly a landmark year for Syndax," said Michael A. Metzger, Chief Executive Officer. "We are working with the FDA during their review of our regulatory submissions while diligently preparing for the potential launch of two first- and best-in-class products in the U.S. in 2024. We look forward to continuing to broadly expand revumenib and axatilimab beyond their first approvals and into additional indications which have the potential to deliver meaningful clinical benefit."

Recent Company Updates and Planned 2024 Milestones

Revumenib:

- The Company announced the submission of a New Drug Application (NDA) under the U.S. Food and Drug Administration (FDA) Real-time Oncology Review (RTOR) program to the FDA for revumenib, a first-in-class menin inhibitor, for the treatment of adult and pediatric relapsed or refractory (R/R) KMT2A-rearranged (KMT2Ar) acute leukemia on December 29, 2023. As per RTOR guidance, the submission is not deemed complete until the FDA issues a PDUFA date, which Syndax expects to receive in the first quarter. RTOR allows for close engagement between the sponsor and the FDA throughout the submission process and enables the FDA to review individual modules of a drug application rather than requiring a complete application prior to initiating its review.
- The Company expects to complete enrollment in the AUGMENT-101 pivotal trial cohort of patients with R/R mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML) later this quarter or early in the second quarter. The Company plans to report topline data in the fourth quarter of 2024, which could support a regulatory filing for revumenib in an additional indication of R/R mNPM1 AML.
- At the 65th American Society of Hematology (ASH) Annual Meeting in December 2023, the Company highlighted positive results in multiple presentations from the Phase 1 and 2 portions of the AUGMENT-101 trial, including the pivotal AUGMENT-101 results that were featured as a late-breaking oral presentation.
- Investigators presented data from multiple Phase 1 combination trials of revumenib in mNPM1 and KMT2Ar acute leukemia across the treatment landscape at the ASH Annual Meeting and the Company's corporate event. The trials are expanding to validate the recommended Phase 2 dose and the Company expects to have additional data on the trials in the second half of 2024. These include:
    - BEAT AML: Evaluating the combination of revumenib with venetoclax and azacitidine in front-line AML patients. This trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial.
    - SAVE: Evaluating the all-oral combination of revumenib with venetoclax and decitabine/cedazuridine in R/R AML or mixed phenotype acute leukemias. The trial is being conducted by investigators from the MD Anderson Cancer Center and continues to accrue patients.
    - AUGMENT-102: Evaluating the combination of revumenib with fludarabine and cytarabine in patients with R/R acute leukemias.
- The Company plans to initiate a trial of revumenib in combination with 7+3 cytarabine and daunorubicin chemotherapy followed by maintenance treatment in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias this quarter.
- The Company plans to initiate a pivotal trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias by the end of 2024.
- Enrollment is ongoing in a Phase 1 proof-of-concept clinical trial of revumenib in patients with unresectable metastatic microsatellite stable colorectal cancer. The Company expects to provide an update on the trial in the first half of 2024.

Axatilimab:

- The Biologics License Application (BLA) for axatilimab, an anti-CSF-1R antibody, in adult and pediatric patients six years or older with chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy was submitted to the FDA on December 28, 2023.
- Results from the pivotal Phase 2 AGAVE-201 trial were featured in the Plenary Scientific Session at the 65th ASH Annual

Meeting in December 2023.
- Syndax announced today that it has exercised its option under the Company's 2021 collaboration agreement with Incyte to co-commercialize axatilimab in the U.S.
- Syndax announced today the randomized, double-blind and placebo-controlled Phase 2 trial to assess the efficacy, safety and tolerability of axatilimab in patients with idiopathic pulmonary fibrosis (IPF) is open for enrollment.
- Additionally, Incyte plans to initiate two combination trials with axatilimab in cGVHD in mid-2024, including a Phase 2 combination trial with ruxolitinib and a Phase 3 combination trial with steroids.

Corporate

- In the fourth quarter of 2023, Syndax issued 12,432,431 shares of its common stock at $18.50 per share. Additionally in the quarter, the Company sold 2,719,744 shares from its ATM facility. Aggregate net proceeds from these offerings were approximately $258.1 million after deducting underwriting discounts and commissions and estimated offering expenses payable by Syndax. With these proceeds, the Company now believes that it has sufficient cash runway to fund its clinical and commercial operations through 2026.

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 recently 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 BTD by the FDA for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement.

About Axatilimab

Axatilimab is an investigational monoclonal antibody that targets colony stimulating factor-1 receptor, or CSF-1R, a cell surface protein thought to control the survival and function of monocytes and macrophages. In pre-clinical models, inhibition of signaling through the CSF-1 receptor has been shown to reduce the number of disease-mediating macrophages along with their monocyte precursors, which has been shown to play a key role in the fibrotic disease process underlying diseases such as chronic graft-versus-host disease (GVHD) and idiopathic pulmonary fibrosis (IPF). Positive topline results from the Phase 2 AGAVE-201 trial showing the trial met its primary endpoint were recently presented at the 65th American Society of Hematology Annual Meeting and Phase 1/2 data of axatilimab in chronic GVHD were published in the Journal of Clinical Oncology. Axatilimab was granted Orphan Drug Designation by the U.S. Food and Drug Administration for the treatment of patients with chronic GVHD and IPF. In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab. Axatilimab is being developed under an exclusive worldwide license from UCB entered into between Syndax and UCB in 2016.

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 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 "may," "will," "expect," "plan," "anticipate," "estimate," "intend," "could," "believe" and similar expressions such as "look forward" (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, the sufficiency of the Company's cash runway, and the potential use of our 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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