Syndax Highlights Recent Accomplishments and Anticipated 2025 Milestones at the 43rd Annual J.P. Morgan Healthcare Conference

January 13, 2025

– Launched Revuforj® (revumenib) for treatment of R/R acute leukemia with a KMT2A translocation in adult and pediatric patients one year and older –

– Revumenib added to NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for AML and ALL –

– Niktimvo™ (axatilimab-csfr) approved by U.S. FDA for treatment of chronic GVHD after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg –

– sNDA filing for revumenib in R/R mNPM1 AML expected in 1H25 based on positive pivotal data from AUGMENT-101 trial –

WALTHAM, Mass., Jan. 13, 2025 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX) will present at the 43rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 14th at 10:30 a.m. PT/1:30 p.m. ET. Ahead of the presentation, Syndax highlighted its recent accomplishments and anticipated 2025 milestones.

"Building on a transformative 2024 with the FDA approvals of Revuforj® and Niktimvo™, we are focused on executing two outstanding U.S. launches for these first-in-class, practice-changing medicines," said Michael A. Metzger, Chief Executive Officer. "Syndax is well-positioned for continued success and long-term growth with two approved drugs launching into multi-billion-dollar markets, a clear strategy to expand into additional indications, and a strong cash position expected to fund operations through profitability."

2024 Key Accomplishments

Revumenib:

- Launched Revuforj (revumenib), the first and only U.S. Food and Drug Administration (FDA) approved menin inhibitor, in late November 2024. Revuforj was approved by the FDA under the Agency's Real-Time Oncology Review (RTOR) program for the treatment of relapsed or refractory (R/R) acute leukemia with a KMT2A translocation in adult and pediatric patients one year and older.
- Revumenib was added to the latest NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for acute myeloid leukemia (AML) and acute lymphoblastic leukemia (ALL) as a category 2A recommendation for R/R acute leukemia with a KMT2A rearrangement.[1]
- Announced that the primary endpoint was met in the protocol-defined efficacy population of 64 adults with R/R mNPM1 AML in the Phase 2 cohort of the AUGMENT-101 trial of revumenib.
- Reported additional positive results from a post-hoc efficacy analysis of all 77 R/R mNPM1 AML patients who met the efficacy evaluable criteria in the Phase 2 cohort of AUGMENT-101.
- Published data from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in adult and pediatric patients with R/R KMT2A-rearranged (KMT2Ar) acute leukemia in the *Journal of Clinical Oncology*.
- Presented a larger data set with longer follow-up from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in R/R KMT2Ar acute leukemia at the 66th American Society of Hematology (ASH) Annual Meeting.
- Presented data from multiple ongoing trials evaluating revumenib in mNPM1 and KMT2Ar acute leukemia across the treatment landscape. These trials include:
  - BEAT AML: A Phase 1 trial evaluating the combination of revumenib with venetoclax and azacitidine in newly diagnosed mNPM1 or KMT2Ar AML patients. The trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. Updated data from the trial showed an overall response rate (ORR)[2] of 100% (37/37) and a composite complete remission (CRc) rate of 95% (35/37).
  - SAVE: A Phase 1/2 trial evaluating an all-oral combination of revumenib with venetoclax and decitabine/cedazuridine in pediatric and adult patients with R/R AML or mixed-lineage acute leukemia (MPAL) harboring either mNPM1, KMT2Ar, or NUP98r alterations. The trial is being conducted by investigators from MD Anderson Cancer Center. Updated data that showed an ORR of 82% (27/33) and a CR/CRh rate of 48% (16/33) were presented at the 66th ASH Annual Meeting.

Axatilimab:

- Received U.S. Food and Drug Administration (FDA) approval for Niktimvo (axatilimab-csfr) for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs).
- Announced axatilimab-csfr was added to the latest NCCN Guidelines as a category 2A recommendation for the treatment of GVHD after the failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.
- Published results from the pivotal Phase 2 AGAVE-201 trial of axatilimab in adult and pediatric patients with recurrent/refractory active chronic GVHD in the *New England Journal of Medicine*.
- Presented a secondary analysis of overall and organ-specific responses from the pivotal Phase 2 AGAVE-201 trial of axatilimab in adult and pediatric patients with recurrent/refractory active chronic GVHD who had received at least two prior lines of systemic therapy at the 66th ASH Annual Meeting.
- Initiated enrollment in the MAXPIRe trial, a 26-week randomized, double-blinded, placebo-controlled Phase 2 trial of axatilimab on top of standard of care in patients with idiopathic pulmonary fibrosis (IPF).
- The Company's partner, Incyte, initiated a Phase 2, open-label, randomized, multicenter trial of axatilimab in combination with ruxolitinib in patients ≥12 years of age with newly diagnosed chronic GVHD.
- The Company's partner, Incyte, initiated a Phase 3, randomized, double-blind, placebo-controlled, multi-center trial that will investigate the use of axatilimab in combination with corticosteroids as initial treatment for chronic GVHD.

Corporate:

- Announced a $350 million royalty funding agreement with Royalty Pharma based on U.S. net sales of Niktimvo. Syndax expects that its cash, cash equivalents and marketable securities, together with the $350 million from the royalty funding agreement and anticipated product revenue and interest income, will enable the company to reach profitability.

Expected 2025 Key Milestones

Revumenib:

- Maximize U.S. adoption of Revuforj as the preferred menin inhibitor, leveraging our first mover advantage and robust clinical data.
- Submit a supplemental NDA (sNDA) filing for revumenib in R/R mNPM1 AML in the first half of 2025, followed by a potential FDA approval around year-end 2025.
- Publish pivotal data from AUGMENT-101 trial in R/R mNPM1 AML in the first half of 2025.
- Initiate a pivotal trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed mNPM1 or KMT2Ar acute leukemia patients unfit to receive intensive chemotherapy in the first quarter of 2025.
- Report Phase 1 data from a trial evaluating the combination of revumenib with intensive chemotherapy (7+3) followed by revumenib maintenance treatment in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias in the second half of 2025.
- Initiate multiple frontline trials evaluating revumenib in combination with intensive chemotherapy, starting in 2025.
- Present additional data at medical congresses from ongoing trials of revumenib in combination with standard-of-care agents.

Axatilimab:

- Launch Niktimvo in the U.S. in early first quarter of 2025. In the U.S., Niktimvo will be co-commercialized by Syndax and Incyte.
- Complete enrollment in MAXPIRe Phase 2 IPF trial in 2025 with topline data expected in 2026.

Presentation at the 43rd Annual J.P. Morgan Healthcare Conference

- Syndax will webcast its presentation from the 43rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 14, 2025 at 10:30 a.m. PT (1:30 p.m. ET). A live webcast of the fireside chat can be accessed from the Investor section of the Company's website at www.syndax.com, where a replay of the event will also be available for a limited time.

About Syndax

Syndax Pharmaceuticals is a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include Revuforj® (revumenib), an FDA-approved menin inhibitor, and Niktimvo™ (axatilimab-csfr), an FDA-approved monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor. Fueled by our commitment to reimagining cancer care, Syndax is working to unlock the full potential of its pipeline and is conducting several clinical trials across the continuum of treatment. For more information, please visit www.syndax.com/ or follow the Company on X 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, the acceptance of Syndax and its partners' products in the marketplace, sales, marketing, manufacturing and distribution requirements, 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 to Revuforj's commercial availability, 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. NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Acute Myeloid Leukemia (Version 1.2025 – December 20, 2024); NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Acute Lymphoblastic Leukemia (Version 3.2024 – December 20, 2024); NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Pediatric Acute Lymphoblastic Leukemia (Version 2.2025 – December 16, 2024). NCCN makes no warranties of any kind whatsoever regarding their content, use or application and disclaims any responsibility for their application or use in any way.

2. Overall response rate (ORR) includes CR, CRh, CRp, CRi, MLFS, and PR; Composite complete remission (CRc) includes CR, CRh, CRp, and 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

Syndax Contact

Sharon Klahre
Syndax Pharmaceuticals, Inc.
sklahre@syndax.com
Tel 781.684.9827

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