Syndax Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Business Update

March 3, 2025

– $7.7 million in Revuforj® (revumenib) net product revenue in initial five weeks of launch –

– Launched Niktimvo™ (axatilimab-csfr) in the U.S. in late January, in partnership with Incyte –

– sNDA filing for revumenib in R/R mNPM1 AML expected in 2Q25 based on positive pivotal data from AUGMENT-101 trial –

– $692.4 million in cash, cash equivalents and investments expected to fund the company to profitability –

– Company to host a conference call today at 8:00 a.m. ET –

NEW YORK, March 03, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results for the fourth quarter and full year ended December 31, 2024, and provided a business update.

"We are off to a strong start with the U.S. launch of Revuforj and are very encouraged by the early patient demand, breadth of prescribing, and coverage from payers. Our early results are consistent with the high unmet need in R/R KMT2A-rearranged acute leukemia and reflect strong execution across our entire organization," said Michael A. Metzger, Chief Executive Officer. "With two first-in-class medicines on the market that address major unmet needs and a robust development strategy underway for expansion, Syndax is well-positioned to unlock the multi-billion-dollar potential of both medicines."

Recent Business Highlights and Anticipated Milestones

Revuforj® (revumenib)

- Achieved $7.7 million in Revuforj net product revenue in the fourth quarter of 2024, the first partial quarter (initial five weeks) of the U.S. launch. The Company estimates that approximately one-third of the net revenue represents inventory at specialty pharmacies and specialty distributors and the remainder represents patient demand. Revuforj was launched in the U.S. in late November 2024, following the FDA's approval on November 15, 2024 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 (KMT2Ar).[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 pivotal AUGMENT-101 trial of revumenib. The Company expects to submit a supplemental NDA (sNDA) filing for revumenib in R/R mNPM1 AML in the second quarter of 2025, followed by a potential FDA approval around year-end 2025. The Company also expects to publish the pivotal data and submit the publication for consideration to be included in the NCCN Guidelines in the second quarter of 2025.
- 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. In the expanded analysis, 26% (20/77; 95% CI: 17%, 37%) achieved a complete remission (CR) plus CR with partial hematological recovery (CRh) and the median duration of CR/CRh response was 4.7 months.
- 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. Consistent with previously reported data, the updated analysis showed that revumenib provides durable responses and robust rates of overall response, minimal residual disease (MRD) negativity, and hematopoietic stem cell transplantation (HSTC). With seven months of additional follow-up, the median duration of CR/CRh extended to 13 months among the 13 CR/CRh responders included in the interim analysis presented at ASH 2023.
- Multiple trials evaluating revumenib in mNPM1 and KMT2Ar acute leukemia across the treatment landscape are ongoing. 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. The trial is now enrolling a cohort of newly diagnosed patients.
  - Intensive chemotherapy: A Phase 1 trial evaluating the combination of revumenib with intensive chemotherapy (7+3) followed by revumenib maintenance treatment in newly diagnosed mNPM1 or KMT2Ar acute leukemia patients. The company expects to report Phase 1 data in the second half of 2025.
  - Break *Through* Cancer: A Phase 2 trial studying whether the combination of revumenib and venetoclax can eliminate MRD in patients with AML and extend progression-free survival. The trial is being conducted by Break *Through* Cancer, a collaboration between leading U.S. cancer research centers.
  - INTERCEPT: A Phase 1 trial evaluating the use of novel therapies, including revumenib, to target MRD and early relapse in AML. The trial is being conducted by the Australasian Leukaemia and Lymphoma Group as part of the INTERCEPT AML master clinical trial. Data that showed 54% (6/11) of patients had MRD reduction at any time, including 36% (4/11) who achieved MRD negativity, were presented at the 66th ASH Annual Meeting.
- The Company is initiating 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.
- The Company plans to initiate multiple trials of revumenib in combination with standard of care regimens in newly diagnosed acute leukemia patients who are fit to receive intensive chemotherapy, starting in the second half of 2025.

- The Company is evaluating revumenib in patients with R/R metastatic microsatellite stable (MSS) colorectal cancer (CRC). The Phase 1b portion of this proof-of-concept trial is ongoing.

Niktimvo™ (axatilimab-csfr)

- Launched Niktimvo in the U.S. in late January, in partnership with Incyte. Niktimvo is approved by the U.S. FDA 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).
- 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 chronic GVHD at the 66th ASH Annual Meeting. The data demonstrated rapid responses and symptom improvement in inflammatory and fibrotic manifestations of chronic GVHD in heavily pretreated patients.
- Presented a post-hoc analysis evaluating the effects of prior lines of therapy on clinical outcomes for patients with chronic GVHD who received axatilimab in the AGAVE-201 trial at the 2025 Tandem Meetings of the American Society for Transplantation and Cellular Therapy and the Center for International Blood and Marrow Transplantation Research. The data show that overall response rates were consistent with axatilimab regardless of the number of prior lines of therapy and that organ-specific responses were noted regardless of the last prior therapy.
- 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 of axatilimab in combination with corticosteroids as initial treatment for chronic GVHD.
- Enrollment is ongoing in the MAXPIRe trial, a Phase 2, 26-week randomized, double-blinded, placebo-controlled trial of axatilimab on top of standard of care in patients with idiopathic pulmonary fibrosis (IPF). The company expects to complete enrollment in the trial in 2025 with topline data anticipated in 2026.

Corporate Update

- The Company announced a $350 million royalty funding agreement with Royalty Pharma based on U.S. net sales of Niktimvo. Under the agreement, Syndax received $350 million in exchange for a 13.8% capped synthetic royalty on U.S. net sales of Niktimvo.

Fourth Quarter and Full Year 2024 Financial Results

As of December 31, 2024, Syndax had cash, cash equivalents, and short and long-term investments of $692.4 million and 86.0 million common shares and pre-funded warrants outstanding.

Fourth quarter 2024 net product revenue for Revuforj was $7.7 million, the first partial quarter of the U.S. launch. Cost of sales for the fourth quarter 2024 was $0.8 million.

Fourth quarter 2024 research and development expenses increased to $65.5 million from $55.1 million, and for the full year increased to $241.6 million compared to $163.0 million for 2023. The year-over-year increase was primarily due to increased clinical, medical and pre-commercial manufacturing expenses as well as increased employee-related expenses and professional fees.

Fourth quarter 2024 selling, general and administrative expenses increased to $37.7 million from $22.8 million, and for the full year increased to $120.9 million compared to $66.9 million for 2023. The year-over-year increase was primarily due to increased employee-related expenses and professional fees to support commercial readiness as well as increased sales and marketing related expenses related to the U.S. commercial launch of Revuforj.

For the three months ended December 31, 2024, Syndax reported a net loss attributable to common stockholders of $94.2 million, or $1.10 per share, compared to a net loss attributable to common stockholders of $72.5 million, or $1.00 per share, for the comparable prior year period. For the year ended December 31, 2024, Syndax reported a net loss attributable to common stockholders of $318.8 million or $3.72 per share, compared to a net loss attributable to common stockholders of $209.4 million or $2.98 per share for the comparable prior year period.

Financial Guidance

For the first quarter of 2025, the Company expects research and development expenses to be $65 to $70 million and total research and development plus selling, general and administrative expenses to be $105 to $110 million. For the full year of 2025, the Company expects research and development expenses to be $260 to $280 million and total research and development plus selling, general and administrative expenses to be $415 to $435 million, which includes an estimated $45 million in non-cash stock compensation expense. The Company is not providing revenue guidance at this time.

Syndax expects that its cash, cash equivalents and short- and long-term investments, combined with its anticipated product revenue and interest income, will enable the company to reach profitability.

Conference Call and Webcast

In connection with the earnings release, Syndax's management team will host a conference call and live audio webcast at 8:00 a.m. ET today, Monday, March 3, 2025.

The live audio webcast and accompanying slides may be accessed through the Events & Presentations page in the Investors section of the Company's website. Alternatively, the conference call may be accessed through the following:

Conference ID: Syndax4Q24
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX4Q24.cfm

For those unable to participate in the conference call or webcast, a replay will be available on the Investors section of the Company's website at www.syndax.com approximately 24 hours after the conference call and will be available for 90 days following the call.

About Revuforj® (revumenib)

Revuforj (revumenib) is an oral, first-in-class menin inhibitor that is FDA approved for the treatment of relapsed or refractory (R/R) acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in adult and pediatric patients one year and older.

Revumenib is in development for the treatment of R/R acute myeloid leukemia (AML) with a nucleophosmin 1 mutation (mNPM1). Positive pivotal data from the AUGMENT-101 trial in this population with revumenib as a monotherapy were recently reported. The Company expects to file a supplemental NDA filing for revumenib in R/R mNPM1 AML in the second quarter of 2025. Additionally, multiple trials of revumenib in combination with standard-of-care agents in mNPM1 AML or KMT2A-rearranged acute leukemia are ongoing across the treatment landscape, including in newly diagnosed patients.

Revumenib was previously granted Orphan Drug Designation for the treatment of AML, ALL and acute leukemias of ambiguous lineage (ALAL) by the U.S. FDA and for the treatment of AML by the European Commission. The U.S. FDA also granted Fast Track designation to revumenib for the treatment of adult and pediatric patients with R/R acute leukemias harboring a KMT2A rearrangement or NPM1 mutation and Breakthrough Therapy Designation for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement.

About Niktimvo™ (axatilimab-csfr)

Niktimvo (axatilimab-csfr) is a first-in-class colony stimulating factor-1 receptor (CSF-1R)-blocking antibody approved for use in the U.S. 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).

In 2016, Syndax licensed exclusive worldwide rights to develop and commercialize axatilimab from UCB. In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab in chronic GVHD and any future indications.

Axatilimab is being studied in frontline combination trials in chronic GVHD, including a Phase 2 combination trial with ruxolitinib (NCT06388564) and a Phase 3 combination trial with steroids (NCT06585774). Axatilimab is also being studied in an ongoing Phase 2 trial in patients with idiopathic pulmonary fibrosis (NCT06132256).

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, the potential use of its product candidates to treat various cancer indications and fibrotic diseases, and Syndax's expected first quarter and full year research and development expenses, and expected first quarter and full year total operating expenses, including its estimated non-cash stock compensation expense. 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 or Niktimvo'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.

Niktimvo is a trademark of Incyte.

All other trademarks are the property of their respective owners.

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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SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands)	December 31, 2024	December 31, 2023
Cash, cash equivalents, short and long-term investments	$ 692,404	$ 600,527
Total assets	$ 724,816	$ 612,880
Total liabilities	$ 436,692	$ 58,684
Total stockholders' equity	$ 288,124	$ 554,196
Common stock outstanding	85,694,443	84,826,632
Common stock and common stock equivalents*	98,972,323	96,316,640
*Common stock and common stock equivalents:		
Common stock	85,694,443	84,826,632
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	85,980,157	85,112,346
Options to purchase common stock	11,688,079	10,684,858
Restricted Stock Units	1,304,087	519,436
Total common stock and common stock equivalents	98,972,323	96,316,640

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(In thousands, except share and per share data)	Three Months Ended December 31, 2024	Three Months Ended December 31, 2023	Year Ended December 31, 2024	Year Ended December 31, 2023
Revenue				
Total revenue				
Net product sales	$ 7,680	$ -	$ 7,680	$ -
Milestone and license revenue	-	-	16,000	-
Total revenue	7,680	-	23,680	-
Operating expenses:				
Cost of product sales	$ 826	$ -	$ 826	$ -
Research and development	65,529	55,126	241,647	163,032
Selling, general and administrative	37,690	22,779	120,879	66,922
Total operating expenses	104,045	77,905	363,352	229,954
Loss from operations	(96,365)	(77,905)	(339,672)	(229,954)
Other income (expense), net	2,195	5,432	20,914	20,594
Net loss	$ (94,170)	$ (72,473)	$ (318,758)	$ (209,360)
Net loss attributable to common stockholders	$ (94,170)	$ (72,473)	$ (318,758)	$ (209,360)
Net loss per share attributable to common stockholders--basic and diluted	$ (1.10)	$ (1.00)	$ (3.72)	$ (2.98)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	85,791,198	72,520,784	85,622,065	70,370,519

Source: Syndax Pharmaceuticals, Inc.