Syndax Reports First Quarter 2025 Financial Results and Provides Business Update

May 5, 2025

– $20.0 million in Revuforj® (revumenib) net revenue in first full quarter of launch –

– $13.6 million in Niktimvo™ (axatilimab-csfr) net revenue (reported by Incyte) in first partial quarter of launch–

– Submitted sNDA for revumenib in R/R mNPM1 AML –

– Initiated a pivotal frontline trial of revumenib plus ven/aza in mNPM1 and KMT2Ar AML –

– $602.1 million in cash, cash equivalents and investments expected to fund the company to profitability –

– Company to host a conference call today at 4:30 p.m. ET –

NEW YORK, May 05, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today reported its financial results for the first quarter ended March 31, 2025, and provided a business update.

"I'm very pleased to report an outstanding quarter in which Revuforj and Niktimvo generated a combined $34 million in net sales. We believe this success is a reflection of excellent commercial execution and the clinical profile of these first- and best-in-class medicines," said Michael A. Metzger, Chief Executive Officer. "We've also made excellent progress advancing our pipeline, including most notably the recent submission of our sNDA for R/R mNPM1 AML and the initiation of the first pivotal frontline trial of a menin inhibitor in combination with venetoclax and azacitidine for mNPM1 and KMT2Ar AML. With a solid financial position and highly skilled team, we are poised to deliver two successful product launches while aggressively advancing our development strategy designed to unlock the multi-billion-dollar opportunities for both drugs."

Recent Business Highlights and Anticipated Milestones

Revuforj® (revumenib)

- Achieved $20.0 million in Revuforj net revenue in the first quarter of 2025, the first full quarter of the U.S. launch. Revuforj was launched in the U.S. in late November 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.

- Completed the submission of a supplemental New Drug Application (sNDA) to the U.S. FDA in April 2025, seeking Priority Review for the approval of Revuforj for the treatment of R/R mutant NPM1 (mNPM1) AML. The sNDA was submitted under the FDA's Real-Time Oncology Review (RTOR) program, which allows for a more efficient review and close engagement between the sponsor and FDA throughout the submission process. The sNDA is supported by the previously reported positive pivotal data from the AUGMENT-101 trial.

- Submitted the pivotal R/R mNPM1 AML data from the AUGMENT-101 trial for publication. The manuscript has been accepted and the publication is expected imminently.

- Opened enrollment in the EVOLVE-2 trial, a pivotal, randomized, double-blind, placebo-controlled trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed mNPM1 or KMT2A-rearranged (KMT2Ar) AML patients who are unfit for intensive chemotherapy in the first quarter of 2025. The trial is being conducted in collaboration with the HOVON network, a leading cooperative clinical trial group with extensive experience studying novel therapies for hematologic malignancies.

- 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 that showed an overall response rate (ORR)[1] of 100% (37/37) and a composite complete remission (CRc) rate of 95% (35/37) were reported at the Company's investor event at the 66 th ASH Annual Meeting. The Company anticipates that an update on the trial will be available at a medical meeting in the second quarter of 2025.

  - 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 data in the fourth quarter 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 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)

- Niktimvo achieved $13.6 million in net revenue in the first quarter of 2025, the first partial quarter of the U.S. launch. Niktimvo was launched in the U.S. in late January 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). Syndax and Incyte co-commercialize Niktimvo, and Syndax records 50% of the Niktimvo net profit/loss, defined as net product revenue minus the cost of sales and commercial expenses.

- Presented a post-hoc analysis evaluating the effects of prior lines of therapy on clinical outcomes for patients with chronic GVHD in the AGAVE-201 trial of axatilimab. 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 data were presented at the 2025 Tandem Meetings of the American Society for Transplantation and Cellular Therapy and the Center for International Blood and Marrow Transplantation Research.

- Two trials evaluating axatilimab in combination with standard of care therapies in newly diagnosed chronic GVHD patients are ongoing, including:
  - 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.

  - A pivotal Phase 3, randomized, double-blind, placebo-controlled, multi-center trial of axatilimab in combination with corticosteroids in patients ≥ 12 years of age with newly diagnosed 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 the second half of 2026.

First Quarter 2025 Financial Results

As of March 31, 2025, Syndax had cash, cash equivalents, and short and long-term investments of $602.1 million and 86.3 million common shares and prefunded warrants outstanding.

In the first quarter of 2025, the first full quarter of the U.S. launch, Revuforj net revenue was $20.0 million. Cost of sales for the first quarter of 2025 was $0.9 million.

In the first quarter of 2025, the first partial quarter of the U.S. launch, the Company's partner, Incyte, reported $13.6 million in Niktimvo net revenue. Syndax records 50% of the Niktimvo net commercial profit/loss, defined as net product revenue (recorded by Incyte) minus the cost of sales and commercial expenses. For the first quarter of 2025, Niktimvo posted a net commercial loss and Syndax's share of the collaboration loss amounted to $0.2 million.

First quarter 2025 research and development expenses increased to $61.6 million from $56.5 million for the comparable prior year period. The year-over-year increase was due to an increase in axatilimab-related costs primarily driven by the IPF trial, the frontline chronic GVHD trial with ruxolitinib being conducted in partnership with Incyte, and a $10.0 million milestone payment as a result of the first patient dosed in the Phase 3 trial of axatilimab in combination with corticosteroids. The higher expenses were also driven by an increase in personnel costs and other expenses related to increased R&D support for ongoing clinical trials, sNDA activities, and medical affairs in support of commercialization. These activities were partially offset by a decrease in revumenib-related costs, primarily driven by an $8.0 million milestone expense in the 2024 period and a reduction in CMC expense due to the capitalization of inventory for commercial use.

First quarter 2025 selling, general and administrative expenses increased to $41.0 million from $23.0 million for the comparable prior year period. The year-over-year increase was primarily due to increased employee-related expenses and professional fees to support increased sales and marketing-related expenses related to the U.S. commercial launch of Revuforj.

For the three months ended March 31, 2025, Syndax reported a net loss attributable to common stockholders of $84.8 million, or $0.98 per share, compared to a net loss attributable to common stockholders of $72.4 million, or $0.85 per share, for the comparable prior year period.

Financial Guidance

For the second quarter of 2025, the Company expects research and development expenses to be $70 to $75 million and total research and development plus selling, general and administrative expenses to be $110 to $115 million. For the full year of 2025, the Company continues to expect 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 4:30 p.m. ET today, Monday, May 5, 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: Syndax1Q25
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://sndx-1q25.open-exchange.net

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 and the Company submitted a supplemental NDA for revumenib in R/R mNPM1 AML in April 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 the Real-Time Oncology Review Program (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.

About Syndax

Syndax Pharmaceuticals is a commercial-stage biopharmaceutical company advancing innovative 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 second quarter and full year research and development expenses, and expected second 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.

References

1. 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

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

(In thousands)	March 31, 2025	December 31, 2024
Cash, cash equivalents, short and long-term investments	$ 602,135	$ 692,404
Total assets	$ 640,707	$ 724,816
Total liabilities	$ 425,648	$ 436,692
Total stockholders' equity	$ 215,059	$ 288,124
Common stock outstanding	86,047,032	85,694,443
Common stock and common stock equivalents*	102,455,254	98,972,323
*Common stock and common stock equivalents:		
Common stock	86,047,032	85,694,443
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	86,332,746	85,980,157
Options to purchase common stock	13,528,527	11,688,079
Restricted Stock Units	2,593,981	1,304,087
Total common stock and common stock equivalents	102,455,254	98,972,323

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

(In thousands, except share and per share data)	Three Months Ended March 31, 2025	2024
Revenue:		
Product revenue	$ 20,042	$ —
Total revenue	20,042	—
Operating expenses:		
Cost of product sales	$ 885	$ —
Research and development	61,636	56,492
Selling, general and administrative	41,031	23,022
Collaboration loss	247	—
Total operating expenses	103,799	79,514
Loss from operations	(83,757)	(79,514)
Other income (expense), net:		
Royalty interest expense	(8,049)	—
Other interest expense	(2)	(55)
Interest income	7,183	7,256
Other income (expense), net	(221)	(87)
Total other income (expense), net	(1,089)	7,114
Net loss	$ (84,846)	$ (72,400)
Net loss attributable to common stockholders	$ (84,846)	$ (72,400)

Net loss per share:

Basic loss per share attributable to common stockholders	$	(0.98)	$	(0.85)
Diluted loss per share attributable to common stockholders	$	(0.98)	$	(0.85)
Weighted-average common shares used in calculating:				
Basic loss per share attributable to common stockholders		86,171,889		85,213,200
Diluted loss per share attributable to common stockholders		86,171,889		85,213,200

Source: Syndax Pharmaceuticals, Inc.