Syndax Reports Second Quarter 2025 Financial Results and Provides Business Update

August 4, 2025

– $28.6 million of Revuforj® (revumenib) net revenue, representing 43% growth over 1Q25 –

– $36.2 million of Niktimvo™ (axatilimab-csfr) net revenue reported by Incyte in first full quarter of launch;$9.4 million in collaboration revenue reported by Syndax –

– sNDA in R/R mNPM1 AML granted Priority Review; PDUFA action date of October 25, 2025 –

– $517.9 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, Aug. 04, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today reported its financial results for the second quarter ended June 30, 2025, and provided a business update.

“Syndax reported another remarkable quarter. With strong Revuforj and Niktimvo sales momentum along with our expectation for stable expenses over the next few years and more than $500 million in cash, we are well on our way to becoming a profitable company with two blockbuster products,” said Michael A. Metzger, Chief Executive Officer. “Notably, Revuforj net revenue increased 43% quarter-over-quarter, even with approximately a third of patients pausing treatment to receive a stem cell transplant. Revuforj is poised for continued growth as we further penetrate the KMT2A population and as patient recontinuations following stem cell transplantation build meaningfully. Importantly, we are nearing another major inflection point with the anticipated approval of Revuforj as the first therapy for R/R mNPM1 AML. Longer-term, our leadership in the menin space positions us to be first to the frontline and meaningfully expand the franchise.”

Mr. Metzger continued, “Additionally, Niktimvo is off to a very fast start generating $50 million in net revenue in just the first five months of the launch. It is already profitable to Syndax and we expect increasing profitability margins as revenues continue to grow. The impressive results demonstrate the benefit of delivering a novel approach to treating chronic GVHD and the potential for Niktimvo to transform patient care and make a substantial financial impact for Syndax.”

Recent Business Highlights and Anticipated Milestones

Revuforj® (revumenib)

- Revuforj achieved $28.6 million in net revenue in the second quarter of 2025, representing a 43% increase over the first quarter of 2025. Revuforj was launched in the U.S. in 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.

- Announced that the U.S. FDA granted Priority Review to the Company’s supplemental New Drug Application (sNDA) for Revuforj for the treatment of R/R mutant NPM1 (mNPM1) acute myeloid leukemia (AML). The sNDA is being reviewed under the FDA's Real-Time Oncology Review (RTOR) program and has been assigned a Prescription Drug User Fee Act (PDUFA) target action date of October 25, 2025.

- Presented new Revuforj data in R/R mNPM1 and NUP98-rearranged (NUP98r) AML from the AUGMENT-101 trial at the European Hematology Association (EHA) Annual Congress Meeting in June 2025.
  - Additional results from all efficacy-evaluable R/R mNPM1 AML patients in the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib (n=77) show an overall response rate (ORR)[1] of 48% (37/77). The complete remission (CR) plus complete remission with partial hematologic recovery (CRh) rate was 26% (20/77). Median duration of CR/CRh response was 4.7 months. Among patients with CR/CRh assessed for measurable residual disease (MRD), 63% (12/19) were MRD negative. Subpopulation analysis showed that a median overall survival (OS) of 23.3 months (95% CI: 8.4-NR) was observed among the 37 patients who achieved an overall response, based on a Kaplan-Meier estimate from this single-arm trial.

  - Among patients with R/R NUP98r AML included in the Phase 1 portion of AUGMENT-101, the ORR was 60% (3/5). One patient who proceeded to transplant resumed revumenib post-transplant and was in maintenance cycle 10 as of the data cutoff date. The safety profile of revumenib in patients with R/R NUP98r AML was consistent with previous reports observed in KMT2Ar or mNPM1 AML.

- Published pivotal data from patients with R/R mNPM1 AML in the Phase 2 portion of the AUGMENT-101 trial in the journal *Blood* in May 2025. The results published from the primary efficacy analysis (n=64) are consistent with the data presented at the EHA Annual Congress Meeting in June 2025 from the larger efficacy-evaluable population (n=77).

- Multiple trials evaluating revumenib in mNPM1 and KMT2Ar acute leukemia across the treatment landscape are ongoing. These trials include:
  - EVOLVE-2: A pivotal, Phase 3, randomized, double-blind, placebo-controlled trial evaluating revumenib in combination with venetoclax and azacitidine in newly diagnosed mNPM1 AML patients who are unfit for intensive chemotherapy. 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.

  - BEAT AML: A Phase 1 trial evaluating the combination of revumenib with venetoclax and azacitidine in newly diagnosed older adults (≥60 years) with mNPM1 or KMT2Ar AML. The trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. Updated data that showed a 67% (29/43) CR rate and 100% (37/37) flow-MRD negativity rate among evaluable responders were published in the *Journal of Clinical Oncology* and simultaneously presented in an oral presentation at the EHA Annual Congress Meeting in

June 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 supporting pivotal dose selection.

- 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.

- Start-up activities are underway for two trials, known as the REVEAL trials, that will evaluate revumenib in combination with standard of care regimens in newly diagnosed acute leukemia patients with mNPM1 or KMT2A-rearranged AML who are fit to receive intensive chemotherapy, with trial initiation expected in the fourth quarter of 2025.

- The Company is evaluating revumenib in patients with R/R metastatic microsatellite stable (MSS) colorectal cancer (CRC). The Company expects to report data from the trial by the end of 2025.

Niktimvo™ (axatilimab-csfr)

- Niktimvo achieved $36.2 million in net revenue in the second quarter of 2025, the first full quarter of the U.S. launch, representing significant growth compared to the $13.6 million in net revenue in the first quarter of 2025. Niktimvo was launched in the U.S. in late January 2025 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 are co-commercializing Niktimvo. Syndax records 50% of the Niktimvo net commercial profit/loss, defined as net product revenue minus the cost of sales and commercial expenses. For the second quarter of 2025, Syndax's share of the Niktimvo product contribution, reported as Collaboration revenue, was $9.4 million.

- Presented data at the EHA Annual Congress Meeting in June 2025 highlighting the robust and rapid responses observed in different organs and subgroups of patients with chronic GVHD in the pivotal AGAVE-201 trial that supported the FDA approval of Niktimvo.

- 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 MAXPIRe, 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 the fourth quarter of 2025 with topline data anticipated in the second half of 2026.

Corporate Updates

- In May 2025, the Company announced the appointment of Dr. Nick Botwood as Head of Research and Development and Chief Medical Officer. Dr. Botwood brings 25 years of industry experience leading drug development, R&D strategy, and global commercialization of novel oncology therapeutics.
- In June 2025, the Company announced the resignation of William Meury from its Board of Directors after seven years of service due to his acceptance of the role of President and Chief Executive Officer and a member of the Board of Directors at Incyte, the Company's collaboration partner for Niktimvo.

Second Quarter 2025 Financial Results

As of June 30, 2025, Syndax had cash, cash equivalents, and short and long-term investments of $517.9 million and 86.3 million common shares and prefunded warrants outstanding.

Total revenue for the second quarter of 2025 was $38.0 million, which consisted of $28.6 million in Revuforj net product revenue and $9.4 million in Niktimvo collaboration revenue. The collaboration revenue is derived from the $36.2 million in Niktimvo net revenue that was previously reported by the Company's partner Incyte. 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.

Second quarter 2025 research and development expenses increased to $62.2 million from $48.7 million for the comparable prior year period. The increase was due to an increase in revumenib-related costs primarily driven by multiple trials evaluating revumenib in mNPM1 and KMT2Ar acute leukemia across the treatment landscape, milestone paid to AbbVie upon submission of the sNDA, and medical affairs activities supporting Revuforj

as well as increased personnel costs to help support commercialization.

Second quarter 2025 selling, general and administrative expenses increased to $43.8 million from $29.1 million for the comparable prior year period. The 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 June 30, 2025, Syndax reported a net loss attributable to common stockholders of $71.8 million, or $0.83 per share, compared to a net loss attributable to common stockholders of $68.1 million, or $0.80 per share, for the comparable prior year period.

Financial Guidance

For the third quarter of 2025, the Company expects total research and development plus selling, general and administrative expenses to be $95 to $100 million, excluding non-cash stock compensation expense. For the full year of 2025, the Company expects total research and development plus selling, general and administrative expenses to be $370 to $390 million, excluding non-cash stock compensation expense. The full year 2025 guidance represents no change to prior guidance, which was $415 to $435 including $45 million in estimated non-cash stock compensation expense. The Company is not providing revenue guidance at this time.

Syndax expects that its operating expense base will remain stable over the next few years. As a result, 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, August 4, 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: Syndax2Q25
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://sndx-2q25.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.

The FDA has granted Priority Review to Syndax's supplemental New Drug Application (sNDA) seeking the approval of revumenib for the treatment of R/R mNPM1 AML. The sNDA is being reviewed 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. The sNDA is supported by positive pivotal data from the AUGMENT-101 trial of revumenib in patients with R/R mNPM1 AML. Results from this population were published in the journal *Blood* and presented at the 2025 European Hematology Association (EHA) Annual Congress Meeting.

Additionally, multiple trials of revumenib are ongoing or planned across the treatment landscape, including in combination with standard of care therapies in newly diagnosed patients with mNPM1 or KMT2Ar AML.

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 third quarter and full year research and development expenses, and expected third 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)	June 30, 2025	December 31, 2024
Cash, cash equivalents, short and long-term investments	$ 517,860	$ 692,404
Total assets	$ 596,149	$ 724,816
Total liabilities	$ 438,725	$ 436,692
Total stockholders' equity	$ 157,424	$ 288,124
Common stock outstanding	86,059,077	85,694,443
Common stock and common stock equivalents*	102,855,472	98,972,323
*Common stock and common stock equivalents:		
Common stock	86,059,077	85,694,443
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	86,344,791	85,980,157
Options to purchase common stock	13,933,003	11,688,079
Restricted Stock Units	2,577,678	1,304,087
Total common stock and common stock equivalents	102,855,472	98,972,323

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

(In thousands, except share and per share data)	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024

Revenue				
Product revenue, net	$ 28,600	—	$ 48,642	—
Collaboration revenue, net	9,358	—	9,111	—
Milestone and license revenue	—	3,500	—	3,500
Total revenue	37,958	3,500	57,753	3,500
Operating expenses:				
Cost of product sales	$ 1,279	—	$ 2,164	—
Research and development	62,227	48,655	123,863	105,147
Selling, general and administrative	43,805	29,061	84,836	52,083
Total operating expenses	107,311	77,716	210,863	157,230
Loss from operations	(69,353)	(74,216)	(153,110)	(153,730)
Other income (expense), net	(2,494)	6,153	(3,583)	13,267
Net loss	$ (71,847)	$ (68,063)	$ (156,693)	$ (140,463)
Net loss attributable to common stockholders	$ (71,847)	$ (68,063)	$ (156,693)	$ (140,463)
Net loss per share:				
Basic loss per share attributable to common stockholders	$ (0.83)	$ (0.80)	$ (1.82)	$ (1.65)
Diluted loss per share attributable to common stockholders	$ (0.83)	$ (0.80)	$ (1.82)	$ (1.65)
Weighted-average common shares used in calculating:				
Basic loss per share attributable to common stockholders	86,337,237	85,274,829	86,255,020	85,244,015
Diluted loss per share attributable to common stockholders	86,337,237	85,274,829	86,225,020	85,244,015

Source: Syndax Pharmaceuticals, Inc.