Syndax Reports Third Quarter 2024 Financial Results and Provides Business Update

November 5, 2024

– New revumenib and Niktimvo ™ clinical data will be highlighted at 66th ASH Annual Meeting –

– mNPM1 AML topline data from AUGMENT-101 expected in 4Q24; potential sNDA filing in 1H25 –

– Revumenib NDA in R/R KMT2Ar acute leukemia is being reviewed under RTOR; PDUFA action date of December 26, 2024 –

– Niktimvo 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 –

– $350 million royalty funding agreement for Niktimvo expected to fund Company through profitability –

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

WALTHAM, Mass., Nov. 5, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results for the quarter ended September 30, 2024, and provided a business update.

"This has been a historic period for Syndax as we transitioned to a commercial-stage company with the approval of Niktimvo. With the recently completed royalty financing, we expect to be funded through profitability and we are well positioned to maximize the potential of our pipeline," said Michael A. Metzger, Chief Executive Officer. "We have a very exciting quarter ahead with the anticipated FDA approval and U.S. launch of revumenib for adults and pediatrics with R/R KMT2Ar acute leukemia, as well as the expected readout of topline pivotal data from patients with R/R mNPM1 AML. Our commercial organization is well-prepared to launch revumenib and leverage our first-to-market position to drive long-term value creation."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- The New Drug Application (NDA) for revumenib, an oral menin inhibitor, for the treatment of adult and pediatric relapsed or refractory (R/R) KMT2A-rearranged (KMT2Ar) acute leukemia was granted Priority Review and is being reviewed under the U.S. FDA's Real-Time Oncology Review (RTOR) Program with a Prescription Drug User Fee Act (PDUFA) target action date of December 26, 2024.
- The Company expects to report topline data from the AUGMENT-101 pivotal trial cohort of patients with R/R mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML) in the fourth quarter of 2024. Positive data could support a supplemental NDA (sNDA) filing for revumenib in R/R mNPM1 AML in the first half of 2025.
- The Company announced that data from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in adult and pediatric patients with R/R KMT2Ar AML and acute lymphoid leukemia (ALL) have been published in the *Journal of Clinical Oncology*.
- The Company announced that a larger data set and longer follow-up from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in R/R KMT2Ar acute leukemia will be presented at the upcoming 66th American Society of Hematology (ASH) Annual Meeting. The larger efficacy population (n=97) includes the 57 patients from the previously reported interim efficacy analysis plus an additional 40 patients. Consistent with previously reported data, the updated analysis shows 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 the potential to expand revumenib use across the mNPM1 and KMT2Ar acute leukemia treatment landscape are ongoing. These trials include:
    - BEAT AML: A Phase 1 trial evaluating the combination of revumenib with venetoclax and azacitidine in front-line AML patients. The trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. The Company presented updated positive data from the trial at the European Hematology Association (EHA) 2024 Congress, showing a 96% (23 of 24 pts) composite complete remission (CRc) rate in patients with newly diagnosed mNPM1 or KMT2Ar AML. The BEAT AML trial is expanding to validate the recommended Phase 2 dose of the combination of revumenib with venetoclax and azacitidine. The company anticipates that an update on the trial will be available in the fourth quarter of 2024.
    - SAVE: A Phase 1 trial 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 MD Anderson Cancer Center. The Company announced that updated data showing an 88% ORR (23 of 26 pts) in R/R patients with KMT2Ar, mNPM1, or NUP98r leukemias will be presented at the upcoming 66th ASH Annual Meeting. In

addition to the R/R cohort, a frontline cohort is now enrolling patients.
    - 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. Preliminary results from the first eight mNPM1 patients treated with revumenib will be presented at the upcoming 66th ASH Annual Meeting.
    - Intensive chemotherapy: A Phase 1 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. The trial is designed to identify the recommended Phase 2 dose for this combination to support further development.
    - 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.
- The Company plans to 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 by year-end 2024.
- The Company is evaluating revumenib in patients with R/R metastatic microsatellite stable (MSS) colorectal cancer (CRC). The trial is currently enrolling patients in the Phase 1b portion of its Phase 1/2 proof-of-concept trial.

Niktimvo™ (axatilimab-csfr)

- Niktimvo received U.S. Food and Drug Administration (FDA) approval 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). The Company anticipates that Niktimvo will be launched in the U.S. no later than early first quarter 2025. In the U.S., Niktimvo will be co-commercialized by Syndax and Incyte.
- The Company announced Niktimvo was added to the latest NCCN Clinical Practice Guidelines in Oncology (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. Treatments are classified as category 2A when there is uniform NCCN consensus that the intervention is appropriate, based on lower level evidence.
- The Company announced that results from the pivotal Phase 2 AGAVE-201 trial of Niktimvo in adult and pediatric patients with recurrent/refractory active chronic GVHD who had received at least two prior lines of systemic therapy were published in the *New England Journal of Medicine.*
- The Company announced a secondary analysis of overall and organ-specific responses from the pivotal Phase 2 AGAVE-201 trial of Niktimvo in adult and pediatric patients with recurrent/refractory active chronic GVHD who had received at least two prior lines of systemic therapy will be presented 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.
- Enrollment is ongoing in 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), now referred to as the MAXPIRe trial (NCT06132256). The Company expects to report topline data from the trial in 2026.
- The Company's partner, Incyte, is now recruiting patients for 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 (NCT06388564). A Phase 3 trial of axatilimab in combination with steroids for the treatment of chronic GVHD is currently in preparation.

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% royalty on U.S. net sales of Niktimvo. Royalty payments to Royalty Pharma will cease upon reaching a multiple of 2.35x.

Third Quarter 2024 Financial Results

As of September 30, 2024, Syndax had cash, cash equivalents, and short- and long-term investments of $399.6 million and 85.6 million common shares and prefunded warrants outstanding.

Third quarter 2024 research and development expenses increased to $71.0 million from $39.1 million for the comparable prior year period. The increase was primarily due to greater clinical development expenses, higher pre-commercial manufacturing costs, and increased employee-related expenses and professional fees.

Third quarter 2024 selling, general and administrative expenses increased to $31.1 million from $17.3 million for the comparable prior year period. The increase was driven by a greater level of commercial readiness activities for revumenib and axatilimab as well as higher employee-related expenses and professional fees.

For the three months ended September 30, 2024, Syndax reported a net loss attributable to common stockholders of $84.1 million, or $0.98 per share, compared to a net loss attributable to common stockholders of $51.1 million, or $0.73 per share, for the comparable prior year period.

Financial Guidance

For the full year of 2024, the Company expects research and development expenses to be $245 to $250 million (prior guidance $240 million to $260 million) and total operating expenses to be $365 to $370 million (prior guidance $355 million to $375 million), which includes milestone payments that the Company expects to become due as well as an estimated $41 million (prior guidance $43 million) in non-cash stock compensation expense.

Syndax expects that its cash, cash equivalents and marketable securities, together with the $350 million from the sale of a portion of the Niktimvo

royalty and anticipated product revenue and interest income, enables 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, Tuesday, November 5, 2024.

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: Syndax3Q24
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX3Q24.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 Revumenib

Revumenib is an oral, small molecule inhibitor of the menin-KMT2A binding interaction that is being developed for the treatment of KMT2A-rearranged (KMT2Ar), also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including acute lymphoid leukemia (ALL) and acute myeloid leukemia (AML), and mNPM1 AML. The *Journal of Clinical Oncology* published results from the Phase 2 AUGMENT-101 trial of revumenib in R/R KMT2Ar acute leukemia showing the trial met its primary endpoint.

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 anti-CSF-1R 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 the U.S., Niktimvo will be co-commercialized by Syndax and Incyte. Incyte has exclusive commercialization rights for Niktimvo outside of the U.S.

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 – a Phase 2 combination trial with ruxolitinib (NCT06388564) is underway and a Phase 3 combination trial with steroids is in preparation. 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-pilot-program

About Syndax

Syndax Pharmaceuticals is a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include revumenib, a selective 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 (formerly 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 "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 potential use of its product candidates to treat various cancer indications and fibrotic diseases, Syndax's expected full year research and development expenses, expected full year total operating expenses, and Syndax's expectations for liquidity and future operations. 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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SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands)	September 30, 2024	December 31, 2023
Cash, cash equivalents, short and long-term investments	$ 399,636	$ 600,527
Total assets	$ 425,811	$ 612,880
Total liabilities	$ 59,379	$ 58,684
Total stockholders' equity	$ 366,432	$ 554,196
Common stock outstanding	85,285,488	84,826,632
Common stock and common stock equivalents*	99,238,167	96,316,640
*Common stock and common stock equivalents:		
Common stock	85,285,488	84,826,632
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	85,571,202	85,112,346
Options to purchase common stock	12,205,960	10,684,858
Restricted Stock Units	1,461,005	519,436
Total common stock and common stock equivalents	99,238,167	96,316,640

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

(In thousands, except share and per share data)	Three Months Ended September 30, 2024	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2024	Nine Months Ended September 30, 2023
Revenue				
Milestone and license revenue	$ 12,500	$ -	$ 16,000	$ -
Total revenue	12,500	-	16,000	-
Operating expenses:				
Research and development	$ 70,971	$ 39,087	$ 176,118	$ 107,906
Selling, general and administrative	31,106	17,268	83,189	44,143
Total operating expenses	102,077	56,355	259,307	152,049
Loss from operations	(89,577)	(56,355)	(243,307)	(152,049)
Other income (expense), net	5,451	5,209	18,718	15,162
Net loss	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Net loss attributable to common stockholders	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Net loss per share attributable to common stockholders--basic and diluted	$ (84,126) / $ (0.98)	$ (51,146) / $ (0.73)	$ (224,589) / $ (2.63)	$ (136,887) / $ (1.97)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	85,433,569	69,855,766	85,307,660	69,645,888

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