Syndax Reports Second Quarter 2024 Financial Results and Provides Clinical and Business Update

August 1, 2024

– Axatilimab BLA in refractory chronic GVHD is under Priority Review; PDUFA action date of August 28, 2024 –

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

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

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

WALTHAM, Mass., Aug. 1, 2024 /PRNewswire/ -- Syndax Pharmaceuticals (Nasdaq: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results for the quarter ended June 30, 2024, and provided a business update.

"This is an exciting time for Syndax as we transition to a commercial stage company," said Michael A. Metzger, Chief Executive Officer. "We've made significant progress advancing our pipeline this quarter, including the presentation of updated revumenib combination data from the BEAT AML and AUGMENT-102 trials and additional axatilimab data from the AGAVE-201 trial at EHA. We are excited to continue building on this momentum as we look ahead to the approval of both first-in-class assets and sharing pivotal AUGMENT-101 data in mNPM1 AML this year."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- The New Drug Application (NDA) for revumenib, a highly selective 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. On July 29, 2024, the Company announced that the FDA extended the Prescription Drug User Fee Act (PDUFA) target action date for the revumenib NDA from September 26, 2024 to December 26, 2024 to provide FDA additional time to conduct a full review of supplemental information provided by the Company in response to the FDA's requests.
- 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.
- Multiple Phase 1 combination trials of revumenib in mNPM1 and KMT2Ar acute leukemias are ongoing across the treatment landscape. These trials include:
    - BEAT AML: Evaluating the combination of revumenib with venetoclax and azacitidine in front-line AML patients. This 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.
    - SAVE: 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 trial is expanding to validate the recommended Phase 2 doses, with additional data expected in the second half of 2024.
    - Intensive chemotherapy: 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 Phase 1 trial is designed to identify the recommended Phase 2 dose for this combination to support further development.
- 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 presented updated data from the AUGMENT-102 trial evaluating the combination of revumenib with fludarabine and cytarabine in patients with R/R acute leukemias at the EHA 2024 Congress. Treatment with the combination in R/R mNPM1, NUP98-rearranged (NUP98r) or KMT2Ar acute leukemia patients resulted in a 52% (14 of 27 pts) CRc rate.
- The Company announced that it advanced into the Phase 1b portion of its Phase 1/2 proof-of-concept trial of revumenib in patients with R/R metastatic microsatellite stable (MSS) colorectal cancer (CRC) based on initial data from the Phase 1a portion of the trial.

Axatilimab

- The Biologics License Application (BLA) for axatilimab, an anti-CSF-1R antibody, for patients with chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy, is under FDA Priority Review with a PDUFA action date of August 28, 2024.
- 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).
- The Company's partner, Incyte, plans to initiate two combination trials with axatilimab in earlier lines of treatment for chronic GVHD in the second half of 2024, including a Phase 2 combination trial with ruxolitinib and a Phase 3 combination trial with steroids.
- The Company presented additional positive data from the AGAVE-201 pivotal trial evaluating axatilimab monotherapy in patients with refractory chronic GVHD at the EHA 2024 Congress. Responses were noted in all fibrosis-dominant organs and the clinical activity was supported by clinician-reported and patient-reported changes in organ-specific symptoms, such as improvements in swallowing, shortness of breath, skin and joints, and sclerotic skin.

Corporate Updates

- In May 2024, the Company announced the appointment of Aleksandra Rizo, M.D., Ph.D to its Board of Directors. Dr. Rizo has extensive clinical development experience and a track record of successfully leading the development of several oncology drugs from discovery through commercialization.

Second Quarter 2024 Financial Results

As of June 30, 2024, Syndax had cash, cash equivalents, and short- and long-term investments of $454.6 million and 85.3 million common shares and prefunded warrants outstanding.

Second quarter 2024 research and development expenses increased to $48.7 million from $34.8 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.

Second quarter 2024 selling, general and administrative expenses increased to $29.1 million from $14.9 million for the comparable prior year period. The increase was driven by a greater level of pre-commercialization activities for revumenib and axatilimab as well as higher employee-related expenses and professional fees.

For the three months ended June 30, 2024, Syndax reported a net loss attributable to common stockholders of $68.1 million, or $0.80 per share, compared to a net loss attributable to common stockholders of $44.6 million, or $0.64 per share, for the comparable prior year period.

Financial Guidance

For the third quarter of 2024, the Company expects research and development expenses to be $70 to $75 million and total operating expenses to be $105 to $110 million. For the full year of 2024, the Company continues to expect research and development expenses to be $240 to $260 million and total operating expenses to be $355 to $375 million, which includes milestone payments that the Company expects to become due as well as an estimated $43 million in non-cash stock compensation expense.

The Company continues to believe it has sufficient capital to fund its research, clinical development and commercial operations through 2026.

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, Thursday, August 1, 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: Syndax2Q24
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX2Q24.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 a potent, selective, small molecule inhibitor of the menin-KMT2A binding interaction that is being developed for the treatment of KMT2Ar, also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including acute lymphoid leukemia (ALL) and AML, and mNPM1 AML. Positive topline results from the Phase 2 AUGMENT-101 trial in R/R KMT2Ar acute leukemia showing the trial met its primary endpoint were presented at the 65th American Society of Hematology Annual Meeting, and data from the Phase 1 portion of AUGMENT-101 in acute leukemia was published in Nature. Revumenib was granted Orphan Drug Designation for the treatment of AML and ALL by the FDA and for the treatment of AML by the European Commission, and Fast Track designation by the FDA for the treatment of adult and pediatric patients with R/R acute leukemias harboring a KMT2A rearrangement or NPM1 mutation. Revumenib was granted Breakthrough Therapy Designation by the FDA for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement.

About Axatilimab

Axatilimab is an investigational monoclonal antibody that targets colony stimulating factor-1 receptor, or CSF-1R, a cell surface protein thought to control the survival and function of monocytes and macrophages. In pre-clinical models, inhibition of signaling through the CSF-1 receptor has been shown to reduce the number of disease-mediating macrophages along with their monocyte precursors, which has been shown to play a key role in the fibrotic disease process underlying diseases such as chronic GVHD and IPF. Positive topline results from the Phase 2 AGAVE-201 trial showing the trial met its primary endpoint were recently presented at the 65th American Society of Hematology Annual Meeting, and Phase 1/2 data of axatilimab in chronic GVHD were published in the Journal of Clinical Oncology. Axatilimab was granted Orphan Drug Designation by the U.S. Food and Drug

Administration for the treatment of patients with chronic GVHD and IPF. In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab. Syndax has exercised its option under the collaboration agreement to co-commercialize axatilimab in the U.S. and will provide 30% of the commercial effort. Axatilimab is being developed under an exclusive worldwide license from UCB entered into between Syndax and UCB in 2016.

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 clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include revumenib, a highly selective inhibitor of the menin–KMT2A binding interaction, and axatilimab, a monoclonal antibody that blocks the CSF-1 receptor. 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 third quarter and full year research and development expenses, and expected third quarter and 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)	June 30, 2024	December 31, 2023
Cash, cash equivalents, short and long-term investments	$ 454,613	$ 600,527
Total assets	$ 476,947	$ 612,880
Total liabilities	$ 42,609	$ 58,684
Total stockholders' equity	$ 434,338	$ 554,196
Common stock outstanding	85,028,629	84,826,632
Common stock and common stock equivalents*	99,026,763	96,316,640
*Common stock and common stock equivalents:		
Common stock	85,028,629	84,826,632
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	85,314,343	85,112,346
Options to purchase common stock	12,226,274	10,684,858
Restricted Stock Units	1,486,146	519,436
Total common stock and common stock equivalents	99,026,763	96,316,640

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

(In thousands, except share and per share data)	Three Months Ended June 30, 2024	2023	Six Months Ended June 30, 2024	2023
Revenue				
Milestone and license revenue	$ 3,500	$ -	$ 3,500	$ -
Total revenue	3,500	-	3,500	-
Operating expenses:				
Research and development	$ 48,655	$ 34,764	$ 105,147	$ 68,819
Selling, general and administrative	29,061	14,914	52,083	26,875
Total operating expenses	77,716	49,678	157,230	95,694
Loss from operations	(74,216)	(49,678)	(153,730)	(95,694)
Other income (expense), net	6,153	5,063	13,267	9,953
Net loss	$ (68,063)	$ (44,615)	$ (140,463)	$ (85,741)
Net loss attributable to common stockholders	$ (68,063)	$ (44,615)	$ (140,463)	$ (85,741)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.80)	$ (0.64)	$ (1.65)	$ (1.23)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	85,274,829	69,638,427	85,244,015	69,539,209

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