Syndax Pharmaceuticals Reports First Quarter 2024 Financial Results and Provides Clinical and Business Update

May 8, 2024

– NDA filing for revumenib in R/R KMT2Ar acute leukemia granted Priority Review under RTOR; PDUFA action date set for September 26, 2024 –

– BLA filing for axatilimab in chronic GVHD granted Priority Review; PDUFA action date set for August 28, 2024 –

– Enrollment completed in AUGMENT-101 mNPM1 cohort; topline data expected in 4Q24 to potentially support sNDA filing in 1H25 –

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

WALTHAM, Mass., May 8, 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 March 31, 2024, and provided a business update.

"With the potential near-term approvals of revumenib and axatilimab in the third quarter as well as pivotal data from the mNPM1 cohort of the AUGMENT-101 trial in the fourth quarter, the Company is on track to have a historic year punctuated by major value-creating milestones," said Michael A. Metzger, Chief Executive Officer. "Syndax is unparalleled as a SMID cap oncology company with the potential launch of two first- and best-in-class agents into multi-billion-dollar markets with the opportunity for expansion beyond their initial indications. We remain keenly focused on laying the foundation and building an experienced team of experts to ensure our successful transition into a commercial organization."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- In March 2024, the Company announced that the FDA had granted Priority Review for the New Drug Application (NDA) filing for revumenib, a potent, selective small molecule menin inhibitor, for the treatment of adult and pediatric relapsed or refractory (R/R) KMT2A-rearranged (KMT2Ar) acute leukemia. The NDA filing is being reviewed under the FDA's Real-Time Oncology Review Program (RTOR) and has been assigned a Prescription Drug User Fee Act (PDUFA) target action date of September 26, 2024. RTOR allows for a more efficient review and close engagement between the sponsor and the FDA throughout the submission process, which historically has led to earlier approvals.
- In March 2024, the Company also announced the completion of enrollment in the AUGMENT-101 pivotal trial cohort of patients with R/R mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML). Topline data is expected in the fourth quarter of 2024 and could support a supplemental NDA (sNDA) filing for revumenib in R/R mNPM1 AML in the first half of 2025.
- Positive results from a subset of the pivotal AUGMENT-101 trial in pediatric patients with R/R KMT2Ar AML and acute lymphoid leukemia (ALL) treated with revumenib were featured in a plenary session at the American Society of Pediatric Hematology/Oncology (ASPHO) Annual Meeting in April 2024.
- Multiple Phase 1 combination trials of revumenib in mNPM1 and KMT2Ar acute leukemias are ongoing across the treatment landscape. The trials are expanding to validate recommended Phase 2 doses, with additional data expected in the second half of 2024. These trials include:
    - BEAT AML: Evaluating the combination of revumenib with venetoclax and Azacytidine in front-line AML patients. This trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial.
    - 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 the MD Anderson Cancer Center.
    - AUGMENT-102: Evaluating the combination of revumenib with fludarabine and cytarabine in patients with R/R acute leukemias.
- A Phase 1 trial of revumenib in combination with 7+3 chemotherapy followed by maintenance treatment in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias was initiated during the quarter.
- 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.
- Enrollment is ongoing in a Phase 1 proof-of-concept clinical trial of revumenib in patients with unresectable metastatic microsatellite stable colorectal cancer. The Company expects to provide an update on the trial in the second quarter of 2024.

Axatilimab

- In February, the Company announced that the FDA had accepted the Biologics License Application (BLA) filing for axatilimab, an anti-CSF-1R antibody, in patients with chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy. The application was granted Priority Review and assigned 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).
- Our partner Incyte plans to initiate two combination trials with axatilimab in chronic GVHD in 2024, including a Phase 2 combination trial with ruxolitinib and a Phase 3 combination trial with steroids.

Corporate Updates

- In March 2024, the Company announced the appointment of Steven Closter as Chief Commercial Officer. Mr. Closter brings to Syndax more than 30 years of commercial experience in the biopharmaceutical industry.

First Quarter 2024 Financial Results

As of March 31, 2024, Syndax had cash, cash equivalents, and short and long-term investments of $522.0 million and 85.3 million common shares and prefunded warrants outstanding.

First quarter 2024 research and development expenses increased to $56.5 million from $34.1 million for the comparable prior year period. The increase in research and development expenses was primarily due to increased clinical development and manufacturing costs, increased employee-related expenses and professional fees, and development milestone expense recognized in the current period.

First quarter 2024 selling, general and administrative expenses increased to $23.0 million from $12.0 million for the comparable prior year period. The increase in selling, general and administrative expenses was primarily due to increased employee-related expenses and professional fees as well as increased commercialization activities for revumenib and axatilimab.

For the three months ended March 31, 2024, Syndax reported a net loss attributable to common stockholders of $72.4 million, or $0.85 per share, compared to a net loss attributable to common stockholders of $41.1 million, or $0.59 per share, for the comparable prior year period.

Financial Guidance

For the second quarter of 2024, the Company expects research and development expenses to be $50 to $55 million and total operating expenses to be $80 to $85 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 an estimated $43 million in non-cash stock compensation expense.

The Company believes that it has sufficient cash runway 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 8:00 a.m. ET today, Wednesday, May 8, 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: Syndax1Q24
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX1Q24.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 KMT2A-rearranged (KMT2Ar), also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including ALL and AML, and mutant nucleophosmin (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 by the FDA and European Commission for the treatment of patients with AML 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, and Syndax's expected first quarter and full year research and development expenses, and expected first quarter and full year total operating expenses. 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)	March 31, 2024	December 31, 2023
Cash, cash equivalents, short and long-term investments	$ 521,994	$ 600,527
Total assets	$ 543,028	$ 612,880
Total liabilities	$ 51,139	$ 58,684
Total stockholders' equity	$ 491,889	$ 554,196
Common stock outstanding	84,979,686	84,826,632
Common stock and common stock equivalents*	98,658,929	96,316,640
*Common stock and common stock equivalents:		
Common stock	84,979,686	84,826,632
Common stock warrants (pre-funded)	285,714	285,714
Common stock and pre-funded stock warrants	85,265,400	85,112,346
Options to purchase common stock	11,872,530	10,684,858
Restricted Stock Units	1,520,999	519,436
Total common stock and common stock equivalents	98,658,929	96,316,640

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

(In thousands, except share and per share data)	Three Months Ended March 31, 2024	2023
Operating expenses:		
Research and development	$ 56,492	$ 34,054
Selling, general and administrative	23,022	11,961
Total operating expenses	79,514	46,015
Loss from operations	(79,514)	(46,015)
Other income (expense), net	7,114	4,889
Net loss	$ (72,400)	$ (41,126)

Net loss attributable to common stockholders	$ (72,400)	$ (41,126)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.85)	$ (0.59)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	85,213,200	69,438,890

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