Syndax Pharmaceuticals Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Clinical and Business Update

February 27, 2024

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

– NDA for revumenib in R/R KMT2Ar acute leukemia submitted under FDA's RTOR program; PDUFA date assignment expected in 1Q24 –

– Enrollment in AUGMENT-101 mNPM1 patient cohort expected to complete in late 1Q/early 2Q; topline data in 4Q24 –

– Initiated revumenib Phase 1 combination trial with 7+3 chemotherapy in newly diagnosed acute leukemias –

– Axatilimab Phase 2 IPF trial underway –

– $600 million in cash and cash equivalents expected to provide runway through 2026 –

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

WALTHAM, Mass., Feb. 27, 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 fourth quarter and full year ended December 31, 2023, and provided a clinical and business update.

"2023 was a landmark year for Syndax marked by execution across all of our major clinical, regulatory, and operational milestones," said Michael A. Metzger, Chief Executive Officer. "With the axatilimab BLA filing under FDA Priority Review and receipt of a PDUFA action date for revumenib anticipated later this quarter, preparations are well underway for the potential launch of both first- and best-in-class drugs later this year. We are committed to maximizing the full potential of these therapies beyond their initial approvals through expansion into earlier lines of therapy in combination with standard of care agents and into new indications. With a strong cash position that is expected to fund operations through 2026, Syndax is well positioned for continued success and long-term growth."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- On December 29, 2023, the Company submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) 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 submission is being reviewed under the FDA's Real-Time Oncology Review (RTOR) program, and the Company expects to receive a Prescription Drug User Fee Act (PDUFA) action date from the FDA this quarter.
- The Company expects to complete enrollment in the AUGMENT-101 pivotal trial cohort of patients with R/R mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML) later this quarter or early in the second quarter of 2024. 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 the Phase 1 and Phase 2 portions of the Company's AUGMENT-101 trial were featured throughout multiple sessions at the 65th American Society of Hematology (ASH) Annual Meeting in December 2023, including results from the pivotal portion of the trial, which were highlighted as a late-breaking oral presentation.
- At the ASH Annual Meeting and during the Company's accompanying investor event, investigators presented compelling data from multiple Phase 1 combination trials of revumenib in mNPM1 and KMT2Ar acute leukemia 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 azacitidine 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.
- In February 2024, the Company initiated 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.
- 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 unable 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

- The FDA has 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 has been granted Priority Review and assigned a PDUFA action date of August 28, 2024.
- Results from the pivotal AGAVE-201 trial were featured in the Plenary Scientific Session at the 65th ASH Annual Meeting in December 2023.
- The Company exercised its option under its collaboration agreement with Incyte to co-commercialize axatilimab in the U.S. and will provide 30% of the commercial effort.
- Enrollment is ongoing in a randomized, double-blinded, placebo-controlled Phase 2 trial of axatilimab in patients with idiopathic pulmonary fibrosis (IPF).
- Incyte plans to initiate two combination trials with axatilimab in chronic GVHD in mid-2024, including a Phase 2 combination trial with ruxolitinib and a Phase 3 combination trial with steroids.

Corporate Update

In December of 2023, Syndax closed an underwritten public offering of 12,432,431 shares of its common stock at $18.50 per share. This total included the exercise in full by the underwriters of their option to purchase up to 1,621,621 additional shares of common stock. Additionally, the Company issued and sold 2,719,744 shares pursuant to its ATM facility. Aggregate net proceeds from these offerings were approximately $258.1 million after deducting underwriting discounts and sales agent commissions and estimated offering expenses payable by Syndax.

Fourth Quarter and Full Year 2023 Financial Results

As of December 31, 2023, Syndax had cash, cash equivalents, and short and long-term investments of $600.5 million and 85.1 million common shares and prefunded warrants outstanding.

Fourth quarter 2023 research and development expenses increased to $55.1 million from $31.8 million, and for the full year increased to $163.0 million compared to $118.5 million for 2022. The year-over-year increase in research and development expenses was primarily due to increased employee-related expenses and professional fees as well as increased clinical and manufacturing expenses.

Fourth quarter 2023 selling, general and administrative expenses increased to $22.8 million from $10.2 million, and for the full year increased to $66.9 million compared to $33.3 million for 2022. The year-over-year 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 December 31, 2023, Syndax reported a net loss attributable to common stockholders of $72.5 million, or $1.00 per share, compared to a net loss attributable to common stockholders of $39.2 million, or $0.62 per share, for the comparable prior year period. For the year ended December 31, 2023, Syndax reported a net loss attributable to common stockholders of $209.4 million or $2.98 per share, compared to a net loss attributable to common stockholders of $149.3 million or $2.46 per share for the comparable prior year period.

Financial Guidance

For the first quarter of 2024, the Company expects research and development expenses to be $56 to $62 million and total operating expenses to be $82 to $88 million. For the full year of 2024, the Company expects 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 4:30 p.m. ET today, Tuesday, February 27, 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: Syndax4Q23
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX4Q23.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, also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including ALL and AML, and NPM1-mutant 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 recently 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 BTD 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 graft-versus-host disease (GVHD) and idiopathic pulmonary fibrosis (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 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 colony stimulating factor 1 (CSF-1) receptor. For more information, please visit www.syndax.com or follow the Company on 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 sufficiency of the Company's cash runway to fund its research, clinical development and commercial operations through 2026, 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)	December 31, 2023	December 31, 2022
Cash, cash equivalents, short and long-term investments	$ 600,527	$ 481,271
Total assets	$ 612,880	$ 497,236
Total liabilities	$ 58,684	$ 29,787
Total stockholders' equity	$ 554,196	$ 467,449
Common stock outstanding	84,826,632	68,111,385
Common stock and common stock equivalents*	96,316,640	77,460,706
*Common stock and common stock equivalents:		
Common stock	84,826,632	68,111,385
Common stock warrants (pre-funded)	285,714	1,142,856
Common stock and pre-funded stock warrants	85,112,346	69,254,241
Options to purchase common stock	10,684,858	7,981,677
Restricted Stock Units	519,436	224,788
Total common stock and common stock equivalents	96,316,640	77,460,706

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

(In thousands, except share and per share data)	Three Months Ended December 31, 2023	Three Months Ended December 31, 2022	Year Ended December 31, 2023	Year Ended December 31, 2022
Operating expenses:				
Research and development	$ 55,126	$ 31,841	$ 163,032	$ 118,499
Selling, general and administrative	22,779	10,192	66,922	33,258
Total operating expenses	77,905	42,033	229,954	151,757
Loss from operations	(77,905)	(42,033)	(229,954)	(151,757)
Other income (expense), net	5,432	2,839	20,594	2,419
Net loss	$ (72,473)	$ (39,194)	$ (209,360)	$ (149,338)
Net loss attributable to common stockholders	$ (72,473)	$ (39,194)	$ (209,360)	$ (149,338)
Net loss per share attributable to common stockholders--basic and diluted	$ (1.00)	$ (0.62)	$ (2.98)	$ (2.46)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	72,520,784	63,192,750	70,370,519	60,760,906

View original content:https://www.prnewswire.com/news-releases/syndax-pharmaceuticals-reports-fourth-quarter-and-full-year-2023-financial-results-and-provides-clinical-and-business-update-302073241.html

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