Syndax Pharmaceuticals Reports Third Quarter 2023 Financial Results and Provides Clinical and Business Update

11.02.23

– NDA submission initiated for revumenib in R/R KMT2Ar acute leukemia under RTOR –

– mNPM1 final efficacy data from the Phase 1 portion of AUGMENT-101 demonstrates a 36% CR/CRh rate –

– Revumenib and axatilimab U.S. registrational filings on track for year-end 2023 completion and potential 2024 approvals –

– Axatilimab to be featured in plenary session at 65th ASH Annual Meeting –

– Revumenib pivotal monotherapy results, combination with venetoclax and post-transplant maintenance data to be highlighted at 65th ASH Annual Meeting –

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

WALTHAM, Mass., Nov. 2, 2023 /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 September 30, 2023, and provided a business update.

"Syndax continues to make excellent progress against our key milestones and corporate priorities and is well positioned to potentially launch two first- and best-in-class blockbuster therapies in 2024," said Michael A. Metzger, Chief Executive Officer. "We are also pleased to announce final Phase 1 mNPM1 data with a 36% CR/CRh rate, which demonstrates revumenib's ability as a monotherapy with excellent safety and tolerability to drive patients with mNPM1 AML to durable MRD negative remissions. We recently shared topline pivotal data for revumenib in R/R KMT2Ar acute leukemia, our second positive pivotal result over the past few months. We look forward to providing additional monotherapy and combination data, which further highlight the compelling clinical profile and utility of each asset, next month at the ASH Annual Meeting."

New Data Announcement

The Company announced today positive data from the Phase 1 portion of the AUGMENT-101 trial of revumenib in a total of 14 patients with relapsed or refractory (R/R) mutant nucleophosmin (mNPM1) acute myeloid leukemia (AML) who met the recommended Phase 2 dose (RP2D) criteria. The final dataset includes three additional patients that were enrolled in the Phase 1 trial to complete the pharmacokinetic characterization of revumenib. In this analysis, the overall response rate (ORR)[1] was 50% (7/14) with a complete remission (CR) or a CR with partial hematological recovery (CRh) rate of 36% (5/14); 100% (5/5) of CR/CRh patients were minimal residual disease (MRD) negative. 43% (3/7) of responders proceeded to transplant, all after achieving a CR or CRh. 60% (3/5) of CR/CRh patients maintained a response beyond six months. At the time of the analysis, four patients remained in response, with two patients in response over twenty-two months. Revumenib was well tolerated, and the safety profile was consistent with what was previously reported in the AUGMENT-101 trial. There were no Grade 4 or 5 QTc prolongation or greater than Grade 2 differentiation syndrome events, and no patients discontinued due to treatment-related adverse events (TREAs).

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- In October 2023, the Company announced positive topline data from the pivotal AUGMENT-101 trial of revumenib, Syndax's first-in-class menin inhibitor, in patients with R/R KMT2A-rearranged (KMT2Ar) acute leukemia. The trial met its primary endpoint at the protocol-defined interim analysis stage with a CR/CRh rate of 23% (13/57; 95% confidence interval [CI]: [12.7, 35.8, one-sided p-value = 0.0036]) in the pooled KMT2Ar acute leukemia cohort. The CR/CRh rate in patients with KMT2Ar AML was 24.5% (12/49). 39% (14/36) of patients who achieved a CR/CRh underwent hematopoietic stem cell transplant (HSCT); eight of whom went to transplant prior to achieving CR/CRh and therefore, were not included in the reported CR/CRh rate. The CR/CRh responses in both the overall population and the AML subset were durable with a 6.4-month (95% CI: 3.4, NR) median duration as of the July 2023 data cut-off, with 46% (6/13) remaining in response. MRD status was assessed in 10 of the 13 patients who achieved a CR/CRh, 70% (7/10) of whom were MRD negative. Revumenib was well tolerated, and the overall safety profile was consistent with the Company's previously reported data. TRAEs leading to dose reductions and treatment discontinuation were low. Based on the Independent Data Monitoring Committee (IDMC) recommendation, the Company stopped the trial to further accrual in the KMT2Ar cohorts.

  The AUGMENT-101 pivotal data will be presented at the upcoming 65th American Society of Hematology (ASH) Annual Meeting on Sunday, December 10, 2023. Of note, the abstract for this session only includes data from the Phase 1 portion of the AUGMENT-101 trial, however the presentation will describe the pivotal dataset.

- The Company announced today that it has initiated a New Drug Application (NDA) for revumenib for the treatment of R/R KMT2Ar acute leukemia under the FDA's Real-time Oncology Review (RTOR) program. 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. The Company expects to complete the NDA submission by year-end 2023.

- The AUGMENT-101 pivotal trial is enrolling patients with mNPM1 AML which could support a second indication in acute leukemia for revumenib. The Company expects to complete enrollment of the cohort in late 1Q24 or early 2Q24 and report topline data in 4Q24.

- The Company also announced data from patients in the AUGMENT-101 trial who received revumenib as post-transplant maintenance, including patients who have been treated with revumenib maintenance for over a year, resulting in long-term responses and conversion to MRD negative status. The data will be presented at the upcoming ASH Annual Meeting on Monday, December 11, 2023. A copy of the abstract is available on the ASH website at www.hematology.org.

- The Company has several trials of revumenib ongoing across the treatment landscape in mNPM1 and KMT2Ar acute leukemias that include the following:

    - BEAT-AML: Evaluating the combination of revumenib with venetoclax and azacitidine in front-line AML patients, being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. The Company expects to present preliminary safety and efficacy data in 4Q23.

    - 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. Early results from the trial will be featured in an oral presentation at the ASH Annual Meeting on Saturday, December 9, 2023. A copy of the abstract is available on the ASH website at www.hematology.org.

    - AUGMENT-102: Evaluating the combination of revumenib with chemotherapy in patients with R/R acute leukemias. The Company expects to provide an update on initial safety data along with the RP2D from the trial in 4Q23.

    - INTERCEPT: Evaluating revumenib as a monotherapy in patients with AML who are minimal residual disease-positive following initial treatment as part of the INTERCEPT AML Master Clinical Trial.

- The Company plans to initiate a trial of revumenib with 7+3 cytarabine and daunorubicin chemotherapy followed by maintenance treatment in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias in late 4Q23 or early 1Q24.

- A proof-of-concept clinical trial of revumenib in patients with unresectable metastatic microsatellite stable colorectal cancer is enrolling patients, and the Company expects to provide an update on the Phase 1 trial in 1Q24.

Axatilimab

- In July 2023, the Company and its partner, Incyte, announced positive topline data from the pivotal AGAVE-201 trial of axatilimab, Syndax's anti-CSF-1R antibody, in patients with chronic graft-versus-host disease (cGVHD) following two or more prior lines of therapy. All three dose cohorts, 0.3 mg/kg every two weeks, 1.0 mg/kg every two weeks and 3.0 mg/kg every four weeks, met the primary endpoint. The ORR within the first six months of treatment at the 0.3 mg/kg dose was 74%, and 60% of these patients were still responding at one year. Furthermore, axatilimab was generally well tolerated, and the most common adverse events were consistent with on-target effects and prior trials. Syndax and Incyte expect to submit a Biologics License Application (BLA) filing by year-end 2023.

  The pivotal AGAVE-201 trial results will be featured at the plenary session at the ASH Annual Meeting on Sunday, December 10, 2023. A copy of the abstract is available on the ASH website at www.hematology.org.

- The Company expects to initiate a randomized, double-blind and placebo-controlled Phase 2 trial that assesses the efficacy, safety and tolerability of axatilimab in patients with idiopathic pulmonary fibrosis (IPF) by year-end 2023.

- Incyte and Syndax expect to initiate a trial assessing axatilimab in combination with ruxolitinib in cGVHD in mid-2024.

Third Quarter 2023 Financial Results

As of September 30, 2023, Syndax had cash, cash equivalents, short and long-term investments of $379.3 million and 69.9 million common shares and prefunded warrants outstanding.

Third quarter 2023 research and development expenses increased to $39.1 million from $26.9 million for the comparable prior year period. The 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.

Third quarter 2023 general and administrative expenses increased to $17.3 million from $8.2 million for the comparable prior year period. The increase

is primarily due to employee-related expenses and professional fees.

For the three months ended September 30, 2023, Syndax reported a net loss attributable to common stockholders of $51.1 million, or $0.73 per share, compared to a net loss attributable to common stockholders of $35.4 million, or $0.58 per share, for the comparable prior year period.

Financial Update and Guidance

For the full year of 2023, the Company expects research and development expenses to be $160 to $165 million and total operating expenses to be $225 to $230 million. This is a reduction from prior guidance of $160 to $175 million for research and development expenses and $225 to $240 million for total operating expenses.

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, November 2, 2023.

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: SNDX3Q23
Domestic Dial-in Number: 800-590-8290
International Dial-in Number: 240-690-8800
Live webcast: https://www.veracast.com/webcasts/syndax/events/SNDX3Q23.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 Syndax

Syndax 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 "may," "will," "expect," "plan," "anticipate," "estimate," "intend," "could," "believe" 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 submission of an NDA and BLA by year-end, the potential use of our product candidates to treat various cancer indications and fibrotic diseases, and Syndax's expected full year research and development expenses as well as 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.

References

[1] Overall response rate includes CR, CRh, CRp, CRi, MLFS, and PR
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

	September 30,	December 31,

(In thousands)	2023	2022
Cash, cash equivalents, short and long-term investments	$ 379,261	$ 481,271
Total assets	$ 399,224	$ 497,236
Total liabilities	$ 40,038	$ 29,787
Total stockholders' equity	$ 359,186	$ 467,449
Common stock outstanding	69,638,168	68,111,385
Common stock and common stock equivalents*	83,992,083	77,460,706
*Common stock and common stock equivalents:		
Common stock	69,638,168	68,111,385
Common stock warrants (pre-funded)	285,714	1,142,856
Common stock and pre-funded stock warrants	69,923,882	69,254,241
Options to purchase common stock	13,543,965	7,981,677
Restricted Stock Units	524,236	224,788
Total common stock and common stock equivalents	83,992,083	77,460,706

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(In thousands, except share and per share data)	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 39,087	$ 26,901	$ 107,906	$ 86,658
General and administrative	17,268	8,240	44,143	23,066
Total operating expenses	56,355	35,141	152,049	109,724
Loss from operations	(56,355)	(35,141)	(152,049)	(109,724)
Other income (expense), net	5,209	(262)	15,162	(420)
Net loss	$ (51,146)	$ (35,403)	$ (136,887)	$ (110,144)
Net loss attributable to common stockholders	$ (51,146)	$ (35,403)	$ (136,887)	$ (110,144)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.73)	$ (0.58)	$ (1.97)	$ (1.84)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	69,855,766	60,670,294	69,645,888	59,941,384

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