Syndax Pharmaceuticals Reports First Quarter 2021 Financial Results and Provides Clinical and Business Update

May 11, 2021

- Today announced two of the prior responders from ongoing Phase 1 portion of AUGMENT-101 trial of SNDX-5613 achieved full count recovery bringing CR/CRh rate up to 23% -

- Company on track to initiate pivotal Phase 2 portion of AUGMENT-101 trial in 2Q21 -

- Enrollment of 23 cGVHD patients in Phase 2 expansion cohort testing axatilimab at 1 mg/kg complete; enrollment ongoing in pivotal AGAVE-201 trial of axatilimab in cGVHD -

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

WALTHAM, Mass., May 11, 2021 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. ("Syndax," the "Company" or "we") (Nasdaq: SNDX), a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results for the first quarter ended March 31, 2021. In addition, the Company provided a clinical and business update.

"We are excited to provide an update on the positive interim Phase 1 data from the ongoing AUGMENT-101 trial of SNDX-5613 in patients with mixed lineage leukemia rearranged (MLLr) and nucleophosmin (NPM1c) mutant acute leukemias," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "As Dr. Eytan Stein indicated in his presentation, complete eradication of leukemia need not occur simultaneously with complete recovery of blood cell counts, and today we are able to share that two prior responders have advanced from complete response with incomplete platelet recovery (CRp) to a complete response (CR). The benefit we observe in AUGMENT-101 support our strong conviction that SNDX-5613 has the potential to meaningfully shape the treatment paradigm for these patients who are in desperate need of improved therapeutic options. As previously announced, we have identified a candidate recommended Phase 2 dose (RP2D) and look forward to commencing the pivotal Phase 2 portion of the trial."

"In addition, we continue to make progress with axatilimab in patients with chronic graft versus host disease (cGVHD). We are pleased to announce today that we have completed enrollment of 23 patients in the Phase 2 expansion portion of the Phase 1/2 trial. We look forward to sharing full updated results from the Phase 1/2 trial later this year, with top-line data from the ongoing pivotal AGAVE-201 trial expected in 2023. Supported by emerging data, we firmly believe that axatilimab has the potential to benefit many of the more than 10,000 patients diagnosed with cGVHD in the U.S. each year."

Recent Progress and Anticipated Milestones

SNDX-5613

The Company today announced new data from the ongoing Phase 1 dose escalation portion of the Phase 1/2 AUGMENT-101 trial of SNDX-5613, a highly selective oral menin inhibitor, in patients with MLLr and NPM1c mutant relapsed/refractory (R/R) acute leukemias. The new data reported today, showed two prior responders have improved from CRp to CR with no evidence of minimal residual disease (MRD-). With the addition of these two patients, a total of 7/31 patients (23%) have achieved CR/CRh.

Syndax previously announced positive data from the Phase 1 portion of the AUGMENT-101 trial in April 2021 and hosted a conference call featuring Eytan M. Stein, M.D., Assistant Attending Physician and Director, Program for Drug Development in Leukemia, Department of Medicine at Memorial Sloan Kettering Cancer Center. As of the data cutoff date, the overall response rate (ORR) among evaluable patients was 48% (n=15), with 67% (n=10) of these responders achieving MRD- status, and four proceeding to receive stem cell transplant. The ORR in evaluable patients harboring an MLL-rearrangement (n=24), was 54% (n=13), and in evaluable patients harboring an NPM1c mutation (n=7), was 29% (n=2). Across all patients enrolled in the trial as of the data cutoff date (n=43), SNDX-5613 was generally well-tolerated, with no discontinuations due to treatment-related adverse events observed in heavily pretreated patients.

Syndax remains on track to initiate the pivotal Phase 2 portion of the trial this quarter.

Axatilimab

Syndax today announced that enrollment is now complete in the Phase 2 expansion portion of the Phase 1/2 trial of axatilimab, its anti-CSF-1R monoclonal antibody, in patients with cGVHD. The Company anticipates reporting updated results later this year for 40 patients, including the 17 in the Phase 1 portion and 23 from the Phase 2 expansion portion, which evaluated 1 mg/kg of axatilimab every two weeks. Syndax reported preliminary data from the Phase 1 portion of the trial during an oral presentation at the American Society of Hematology Annual Meeting in December 2020 which highlighted the tolerability and high response rate of axatilimab in cGVHD patients refractory to multiple therapeutic agents.

The Company's pivotal Phase 2 AGAVE-201 trial of axatilimab in patients with cGVHD is ongoing, with topline data expected in 2023. The trial will evaluate the safety and efficacy of three doses and schedules of axatilimab. The primary endpoint will assess objective response rate based on the 2014 NIH consensus criteria for cGVHD, with key secondary endpoints including duration of response and improvement in modified Lee Symptom Scale score.

Earlier this year, the Company announced that the U.S. Food and Drug Administration granted Orphan Drug Designation to axatilimab for the treatment of patients with cGVHD and idiopathic pulmonary fibrosis.

First Quarter 2021 Financial Results

As of March 31, 2021, Syndax had cash, cash equivalents and short-term investments of $271.3 million and 51.6 million shares and share equivalents issued and outstanding. This includes 3.3 million pre-funded warrants.

First quarter 2021 research and development expenses increased to $21.9 million from $9.6 million. The increase was primarily due to increased clinical trial activities and increased CMC activities.

General and administrative expenses for the first quarter 2021 decreased to $5.7 million from $5.9 million. The decrease is primarily due to decreased pre-commercialization expenses for entinostat.

For the three months ended March 31, 2021, Syndax reported a net loss attributable to common stockholders of $27.7 million or $0.54 per share compared to $19.1 million or $0.56 per share for the prior year period.

Financial Update and Guidance

For the second quarter of 2021, research and development expenses are expected to be $30 to $35 million, and total operating expenses are expected to be $35 to $40 million. For the full year of 2021, research and development expenses are expected to be $90 to $100 million, and total operating expenses are expected to be $110 to $120 million.

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, May 11, 2021.

The live audio webcast and accompanying slides may be accessed through the Events & Presentations page in the Investors section of the Company's website at www.syndax.com. Alternatively, the conference call may be accessed through the following:

Conference ID: 8178876
Domestic Dial-in Number: (855) 251-6663
International Dial-in Number: (281) 542-4259
Live webcast: https://edge.media-server.com/mmc/p/4gwngoxg

For those unable to participate in the conference call or webcast, a replay will be available for 30 days on the Investors section of the Company's website, www.syndax.com.

About Syndax Pharmaceuticals, Inc.

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. The Company's pipeline includes SNDX-5613, a highly selective inhibitor of the Menin–MLL binding interaction for the treatment of genetically-defined leukemias; axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor; and entinostat, a class I HDAC inhibitor.

Syndax's Cautionary Note on 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," "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 design, progress, timing, clinical development and scope of clinical trials, plans for initiating future clinical trials, reporting of clinical data for Syndax's product candidates, the association of data with treatment outcomes, the potential use of our product candidates to treat various cancer indications and fibrotic diseases, Syndax's expected second quarter and full year research and development expenses, and expected 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, the COVID-19 pandemic may disrupt our business and that of the third parties on which we depend, including delaying or otherwise disrupting our clinical trials and preclinical studies, manufacturing and supply chain, or impairing employee productivity, 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, 2021	December 31, 2020
Cash, cash equivalents and short-term investments	$ 271,335	$ 293,065
Total assets	$ 279,672	$ 300,613
Total liabilities	$ 51,467	$ 48,425
Total stockholders' equity (deficit)	$ 228,205	$ 252,188
Common stock outstanding	48,248,559	47,881,223
Common stock and common stock equivalents*	59,085,417	57,836,910
*Common stock and common stock equivalents:		
Common stock	48,248,559	47,881,223
Options to purchase common stock	7,404,823	6,379,235
Restricted Stock Units	124,083	18,500
Pre-funded warrants	3,307,952	3,557,952
	59,085,417	57,836,910

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

(In thousands, except share and per share data)	Three Months Ended March 31, 2021	2020
License fee revenue	$ 379	$ 379
Operating expenses:		
Research and development	21,870	9,562
General and administrative	5,672	5,917
Total operating expenses	27,542	15,479
Loss from operations	(27,163)	(15,100)
Other income (expense), net	(560)	(136)
Net loss	$ (27,723)	$ (15,236)
Net loss attributable to common stockholders	$ (27,723)	$ (19,142)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.54)	$ (0.56)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	51,499,831	34,328,640

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