Syndax Pharmaceuticals Reports Third Quarter 2020 Financial Results and Provides Clinical and Business Update

November 2, 2020

- Presentation of Phase 1 data from AUGMENT-101 trial of SNDX-5613 and initiation of Phase 2 on track for early 2021 -

- Company provides update on axatilimab development plan in cGVHD following recent FDA interactions; expects to commence pivotal trial by year-end -

- Data from Phase 1 trial of axatilimab in patients with cGVHD to be highlighted during oral presentation at ASH Annual Meeting in December -

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

WALTHAM, Mass., Nov. 2, 2020 /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 third quarter ended September 30, 2020. In addition, the Company provided a clinical and business update.

"We continue to make exciting progress in the Phase 1 portion of the AUGMENT-101 trial of SNDX-5613, our highly selective, potent, oral menin inhibitor, in adult and pediatric patients with acute leukemias that harbor MLL-r and NPM1 genetic alterations," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "We are highly encouraged by initial data from this study, which demonstrated clear clinical activity in this difficult to treat population of patients with genetically defined acute leukemias. We remain on track to present Phase 1 data from AUGMENT-101 and commence the Phase 2 portion in early 2021."

Dr. Morrison added, "In addition, following recent interactions with the FDA, we are excited to take the next step of initiating a pivotal trial for axatilimab, our anti-CSF-1R monoclonal antibody, in patients with cGVHD which we expect by the end of this year. We are encouraged by the clinical activity and overall safety we've seen in the ongoing trial, and firmly believe axatilimab has the potential to serve as an effective intervention for patients with cGVHD. We look forward to sharing updated results from the Phase 1 trial during an oral presentation at the ASH Annual Meeting in December."

Pipeline Updates

SNDX-5613

- In August 2020, the Company enacted the following enhancements to the Phase 1 portion of the AUGMENT-101 trial in patients with MLL-r and NPM1 mutant acute leukemias: focusing enrollment exclusively on patients with mixed lineage leukemia rearranged (MLL-r) and nucleophosmin (NPM1) mutant acute leukemias; backfilling any dose escalation cohort up to a total of 12 patients if efficacy has been observed at that dose level; and expansion of enrollment to include pediatric patients over 30 days old. These enhancements were supported by initial clinical data, as well as insights from emerging data in the pediatric compassionate use setting. Enrollment in the amended Phase 1 portion remains ongoing, with a data presentation expected in early 2021. In early 2021, the Company also anticipates commencing the Phase 2 portion of AUGMENT-101, which it believes could serve as the basis for registration. SNDX-5613 was previously granted Orphan Drug Designation for the treatment of adult and pediatric acute myeloid leukemia by the U.S. Food and Drug Administration (FDA).

Axatilimab

- The Company today announced that following its End-of-Phase 1 meeting with the FDA, it has aligned on a regulatory path for axatilimab, its anti-CSF-1R monoclonal antibody, for the treatment of chronic graft versus host disease (cGVHD). The Company plans to commence a pivotal Phase 2 trial, AGAVE-201, to assess the safety and efficacy of different doses and schedules of axatilimab for the treatment of patients with cGVHD. The primary endpoint will assess objective response rate based on the 2014 NIH consensus criteria for GVHD with key secondary endpoints including duration of response and improvement in modified Lee Symptom Scale score. The Company expects to begin enrollment by year-end, with topline data anticipated in 2023.
- Enrollment remains ongoing in the Phase 2 portion of the Phase 1/2 trial evaluating axatilimab for the treatment of patients with cGVHD. In previously announced preliminary data from the Phase 1 portion of the trial, axatilimab demonstrated compelling clinical activity and a well-tolerated safety profile. The Company will present updated data from the Phase 1 portion in an oral presentation during the American Society of Hematology (ASH) Virtual Annual Meeting in December. Abstracts for the meeting, which will be held December 5-8, 2020, will be available on Thursday, November 5, 2020 at 9:00 a.m. ET.

Financial Update and Guidance

As of September 30, 2020, Syndax had cash, cash equivalents and short-term investments of $170.2 million and 44.4 million shares and share equivalents issued and outstanding which included 38.8 million shares of common stock and pre-funded warrants to purchase 5.6 million shares of common stock.

Third quarter 2020 research and development expenses increased to $14.4 million from $9.9 million for the prior year period. The increase was primarily due to increased clinical activity for SNDX-5613 and axatilimab and a $2.0 million milestone payable to UCB upon the achievement of a certain milestone.

General and administrative expenses for the third quarter 2020 increased to $5.8 million from $3.6 million for the prior year period. This increase was primarily due to employee related expenses including a one-time non-cash stock compensation expense.

For the three months ended September 30, 2020, Syndax reported a net loss attributable to common stockholders of $20.4 million or $0.46 per share compared to $12.8 million or $0.41 per share for the prior year period.

Financial Guidance

For the fourth quarter of 2020, research and development expenses are expected to be $15 to $20 million, and total operating expenses are expected to be $20 to $25 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, Monday, November 2, 2020.

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: 7974533
Domestic Dial-in Number: (855) 251-6663
International Dial-in Number: (281) 542-4259
Live webcast: https://edge.media-server.com/mmc/p/62ujymuo

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, 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, axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor, and entinostat, a class I HDAC inhibitor. For more information, please visit www.syndax.com or follow the Company on Twitter and LinkedIn.

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 progress, timing, clinical development and scope of clinical trials and the reporting of clinical data for Syndax's product candidates, the potential use of our product candidates to treat various cancer indications, Syndax's expected fourth quarter 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.

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands)	September 30, 2020	December 31, 2019
Cash, cash equivalents and short-term investments	$ 170,159	$ 59,775
Total assets	$ 179,309	$ 63,525
Total liabilities	$ 47,332	$ 31,925
Total stockholders' equity (deficit)	$ 131,977	$ 31,600
Common stock outstanding	38,834,381	27,140,484
Common stock and common stock equivalents*	51,852,235	42,292,534

*Common stock and common stock equivalents:		
Common stock	38,834,381	27,140,484
Common stock warrants (pre-funded)	5,557,952	4,500,000
Common stock and pre-funded stock warrants	44,392,333	31,640,484
Options to purchase common stock	6,770,660	6,057,011
Series 1 and 2 warrants	689,242	4,595,039
Total common stock and common stock equivalents	51,852,235	42,292,534

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)	**2020**	**2019**	**2020**	**2019**
License fee revenue	$ 379	$ 379	$ 1,138	$ 1,138
Operating expenses:				
Research and development	14,408	9,923	34,913	33,492
General and administrative	5,824	3,605	17,787	10,980
Total operating expenses	20,232	13,528	52,700	44,472
Loss from operations	(19,853)	(13,149)	(51,562)	(43,334)
Other (expense) income, net	(584)	320	(1,173)	1,287
Net loss	$ (20,437)	$ (12,829)	$ (52,735)	$ (42,047)
Net loss attributable to common stockholders	$ (20,437)	$ (12,829)	$ (56,641)	$ (42,047)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.46)	$ (0.41)	$ (1.43)	$ (1.40)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	44,156,808	31,630,639	39,714,490	30,103,338

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