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

August 6, 2020

-- Emerging data for SNDX-5613 support several protocol enhancements to AUGMENT-101 to expand enrollment to pediatric patients and focus exclusively on patients with MLL-r and NPM1 mutant acute leukemias --

-- Recommended Phase 2 dose for AUGMENT-101 anticipated by year end; full Phase 1 data presentation expected in early 2021 --

-- Results from Phase 1 trial of axatilimab in patients with cGVHD expected in 4Q20 --

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

WALTHAM, Mass., Aug. 6, 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 second quarter ended June 30, 2020. In addition, the Company provided a clinical and business update.

"We are very pleased that the FDA has agreed to several proposed changes to the Phase 1 portion of AUGMENT-101 which build on emerging clinical data and help us maximize SNDX-5613's potential in as many appropriate patients as possible," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "This includes focusing enrollment exclusively on patients with MLL-r and NPM1 mutant acute leukemias, the ability to expand dose cohorts that demonstrate efficacy, and the inclusion of pediatric patients, which has long been a key component of our overall strategy for SNDX-5613. We remain on track to identify a recommended Phase 2 dose by the end of this year, with full Phase 1 data anticipated in early 2021."

Dr. Morrison added, "Progress also continues with the Phase 1/2 trial of axatilimab, our anti-CSF-1R monoclonal antibody, in patients with cGVHD, with Phase 1 results expected in the fourth quarter. We are actively committed to helping people with cancer live longer and better than ever before and look forward to further advancing this mission throughout the balance of the year."

Pipeline Updates

SNDX-5613

- Syndax today announced that the U.S. Food and Drug Administration (FDA) has agreed to several enhancements to the Phase 1 portion of the AUGMENT-101 protocol. AUGMENT-101 is the Company's Phase 1/2 open-label trial designed to evaluate the safety, tolerability, pharmacokinetics and efficacy of orally administered SNDX-5613, its potent, highly selective oral menin inhibitor, in patients with acute leukemias. As recently reported, the Phase 1 dose escalation portion of AUGMENT-101 was separated into two cohorts based on concomitant treatment with a strong CYP3A4 inhibitor. Arm A will enroll patients not receiving a strong CYP3A4 inhibitor, while Arm B will enroll patients receiving a strong CYP3A4 inhibitor.

  Supported by initial clinical data, as well as new insights from emerging data in the pediatric compassionate use setting, the Company will enact the following enhancements to the Phase 1 portion of the trial: 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 in either Arm A or Arm B if efficacy has been observed at that dose level; and expansion of enrollment to include pediatric patients over 30 days old. The Company continues to anticipate identifying a recommended Phase 2 dose by the end of 2020, with full data from the amended Phase 1 portion expected in early 2021. SNDX-5613 was recently granted Orphan Drug Designation for the treatment of adult and pediatric AML by the FDA.

- The Company recently participated in the U.S. FDA's June 2020 meeting of the Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee (pedsODAC) to discuss the clinical development plan for SNDX-5613 in pediatric patients. A replay of the pedsODAC meeting, which was intended to improve and encourage the development of oncology and hematology drugs for pediatric use, as well as a copy of the Company's briefing package, can be found here.

- At the 2020 American Association for Cancer Research (AACR) Virtual Annual Meeting I in April, Syndax announced initial clinical data from the AUGMENT-101 trial. Data presented serve as the first clinical evidence that inhibition of the menin-MLL1 interaction can induce response in patients with MLL-r acute leukemias. The presentation also highlighted preclinical findings, including data published in Cancer Cell and Science magazine, supporting the potential of single-agent menin-MLL inhibition to serve as an effective intervention for both MLL-r acute leukemias and NPM1 mutant AML. A copy of the presentation is available on Syndax's website under Publications, Menin-MLL-r Inhibitors.

Axatilimab

- Enrollment continues across the Company's Phase 1/2 trial evaluating axatilimab, its anti-CSF-1R monoclonal antibody, for the treatment of chronic graft versus host disease (cGVHD). The Phase 1 portion continues to explore alternate dose and

schedules, while the Phase 2 expansion is evaluating the benefit of treatment at 1 mg/kg every two weeks. The Company expects to present additional results from the Phase 1 trial in the fourth quarter of 2020.

- Data from the Phase 1 trials exploring axatilimab, both as a monotherapy and in combination with IMFINZI® (durvalumab) in patients with locally-advanced or metastatic solid tumors, were summarized in two oral presentations at the AACR Virtual Annual Meeting I. The data indicate that axatilimab is tolerated well in solid tumor patients and provide evidence of its ability to deplete circulating pro-inflammatory monocytes. A recommended Phase 2 dose of axatilimab for the treatment of patients with solid tumors was determined as monotherapy and in combination with IMFINZI® (durvalumab). A copy of each presentation is available on Syndax's website under Publications, Axatilimab.

Entinostat

- In May 2020, the Company reported final results of E2112, the Phase 3 clinical trial conducted by ECOG-ACRIN Cancer Research Group and sponsored by the National Cancer Institute, that evaluated the investigational compound entinostat, Syndax's class I HDAC inhibitor, plus exemestane in patients with advanced hormone receptor positive, human epidermal growth factor receptor 2 negative (HR+, HER2-) breast cancer. The trial did not achieve the primary endpoint of demonstrating a statistically significant overall survival benefit over hormone therapy alone. The Company has decided to deprioritize the entinostat program to focus resources on advancing the remainder of its pipeline.

Financial Update and Guidance

As of June 30, 2020, Syndax had cash, cash equivalents and short-term investments of $186.8 million and 44.1 million shares and share equivalents issued and outstanding which included 38.5 million shares of common stock and pre-funded warrants to purchase 5.6 million shares of common stock.

In May 2020, Syndax closed an underwritten public offering whereby the Company sold 6.4 million shares of common stock at a price of $18.00 per share. The aggregate net proceeds received by the Company were $107.9 million, net of underwriting discounts and commissions and estimated offering expenses payable by the Company.

Second quarter 2020 research and development expenses decreased to $10.9 million from $12.3 million for the prior year period. The second quarter decrease was primarily due to the impact of a $4.0 million expense for achievement of a clinical milestone associated with SNDX-5613 which was recognized and recorded in the second quarter of 2019. Excluding this milestone, research and development expenses in the second quarter of 2020 increased compared to the prior year period primarily due to an increase in clinical activities related to SNDX-5613 and axatilimab, and professional fees, partially offset by a decrease in clinical activities related to entinostat.

General and administrative expenses for the second quarter 2020 increased to $6.0 million from $3.5 million for the prior year period. The increase was primarily due to increased pre-commercialization activities in advance of the Phase 3 breast cancer results for entinostat and employee related expenses.

For the three months ended June 30, 2020, Syndax reported a net loss attributable to common stockholders of $17.1 million or $0.42 per share compared to $14.9 million or $0.47 per share for the prior year period.

Financial Guidance

Today the Company provided operating expense guidance for the third quarter and second half of 2020. For the third quarter and second half of 2020, research and development expenses are expected to be $14 to $16 million and $30 to $35 million, respectively, and total operating expenses are expected to be $19 to $21 million and $40 to $45 million, respectively.

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, August 6, 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: 8998873
Domestic Dial-in Number: (855) 251-6663
International Dial-in Number: (281) 542-4259
Live webcast: https://edge.media-server.com/mmc/p/sdfqemqh

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 third 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)	June 30, 2020	December 31, 2019
Cash, cash equivalents and short-term investments	$ 186,753	$ 59,775
Total assets	$ 192,628	$ 63,525
Total liabilities	$ 44,981	$ 31,925
Total stockholders' equity (deficit)	$ 147,647	$ 31,600
Common stock outstanding	38,512,744	27,140,484
Common stock and common stock equivalents*	52,034,345	42,292,534
*Common stock and common stock equivalents:		
Common stock	38,512,744	27,140,484
Common stock warrants (pre-funded)	5,557,952	4,500,000
Common stock and pre-funded stock warrants	44,070,696	31,640,484
Options to purchase common stock	6,857,741	6,057,011
Series 1 and 2 warrants	1,105,908	4,595,039
Total common stock and common stock equivalents	52,034,345	42,292,534

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

(In thousands, except share and per share data)	Three Months Ended June 30, 2020	Three Months Ended June 30, 2019	Six Months Ended June 30, 2020	Six Months Ended June 30, 2019
License fee revenue	$ 379	$ 379	$ 758	$ 758
Operating expenses:				
Research and development	10,943	12,290	20,505	23,569
General and administrative	6,046	3,463	11,963	7,374
Total operating expenses	16,989	15,753	32,468	30,943
Loss from operations	(16,610)	(15,374)	(31,710)	(30,185)
Other (expense) income, net	(452)	458	(588)	967
Net loss	$ (17,062)	$ (14,916)	$ (32,298)	$ (29,218)
Net loss attributable to common stockholders	$ (17,062)	$ (14,916)	$ (36,204)	$ (29,218)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.42)	$ (0.47)	$ (0.97)	$ (1.00)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	40,609,205	31,605,279	37,468,922	29,327,029

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