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

November 15, 2021

- Initiated pivotal Phase 2 portion of SNDX-5613 AUGMENT-101 trial in patients with NPM1 and MLLr acute leukemias -

- Entered global collaboration with Incyte to develop and commercialize axatilimab for cGVHD and other fibrotic diseases -

- Updated data from SNDX-5613 and axatilimab clinical programs to be presented during oral sessions at 63rd ASH Annual Meeting -

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

WALTHAM, Mass., Nov. 15, 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 third quarter ended September 30, 2021. In addition, the Company provided a clinical and business update.

"We are thrilled to announce that the pivotal Phase 2 portion of AUGMENT-101, examining SNDX-5613 across three distinct patient populations, is open and enrolling following a productive meeting with the U.S. Food and Drug Administration (FDA)," said Briggs W. Morrison, M.D., Chief Executive Officer of Syndax. "Supported by a growing body of data, we firmly believe that SNDX-5613 is ideally positioned to be first to market and serve as a meaningful intervention for patients with mutant nucleophosmin (NPM1c) and mixed lineage leukemia rearranged (MLLr) acute leukemias who are deeply in need of effective therapies. We look forward to presenting updated data from the Phase 1 portion of the AUGMENT-101 trial, including updated durability of CR/CRh responses, during an oral session at the upcoming 63rd American Society of Hematology (ASH) Annual Meeting."

"We were also pleased to recently announce our partnership with Incyte for the development and commercialization of axatilimab. This agreement represents a key step forward in our strategy to expand and maximize the program across multiple lines of treatment in chronic graft-versus-host disease (cGVHD) as well as additional fibrotic diseases, such as idiopathic pulmonary fibrosis (IPF), where the monocyte-macrophage lineage plays a vital role. As we continue to execute on advancing the program, we look forward to sharing updated data from our Phase 1/2 trial of axatilimab in cGVHD during an oral session at the ASH Annual Meeting next month."

Recent Pipeline Progress and Anticipated Milestones

SNDX-5613

- The Phase 2 portion of AUGMENT-101 is currently enrolling patients with NPM1c mutant and MLLr relapsed/refractory (R/R) acute leukemias. A total of 64 adult and up to 10 pediatric patients will be enrolled across each of the following three distinct trial populations: patients with NPM1 mutant acute myeloid leukemia (AML), patients with MLLr AML, and patients with MLLr acute lymphocytic leukemia. Discussions with the FDA have confirmed that AUGMENT-101 may potentially serve as the basis for regulatory filings in each of the three distinct trials. The primary endpoint for each of the three trials will be efficacy as measured by complete remission rate (complete response [CR] + CR with partial hematologic recovery rate [CRh]), with key secondary endpoints including duration of response and overall survival.
- In November 2021, the Company announced that updated data from the Phase 1 portion of the ongoing AUGMENT-101 trial will be featured during an oral session at the 63rd ASH Annual Meeting being held December 11-14, 2021. Data included in the abstract demonstrated robust clinical activity with durable responses and no discontinuations due to treatment-related adverse events. The oral presentation will include updated Phase 1 data from additional patients as of a more recent cutoff date, as well as further details on durability and CR/CRh rate by mutational status.
- The Company today announced plans to initiate a new trial to assess the anti-leukemic efficacy of SNDX-5613 in NPM1 or MLLr patients with measurable residual disease (MRD) progression following initial treatment. The trial will be conducted as part of the Australian Leukemia and Lymphoma Group (ALLG) INTERCEPT Master Clinical Trial, a collaborative clinical trial investigating novel therapies to target early relapse and clonal evolution as pre-emptive therapy in AML. SNDX-5613 is the first menin inhibitor to be included in the INTERCEPT AML Master Clinical Trial. The Company expects ALLG to initiate the trial in the first half of 2022.
  In August 2021, the Company announced plans to initiate two additional trials to assess the safety, tolerability, and preliminary anti-leukemic efficacy of SNDX-5613 in combination with venetoclax and azacitidine as part of the Leukemia & Lymphoma Society's Beat® AML Master Clinical Trial, and in combination with chemotherapy in patients with R/R NPM1 or MLLr acute leukemias in the AUGMENT-102 trial. The Company expects both trials to initiate in the first half of 2022.

Axatilimab

- In November 2021, the Company announced that updated data from its Phase 1/2 trial of axatilimab in patients with cGVHD will be featured during an oral session at the 63rd ASH Annual Meeting. Data included in the abstract demonstrated broad efficacy and tolerability for axatilimab in patients with relapsed or refractory cGVHD. The oral presentation will include additional follow up on all patients enrolled.

Enrollment is ongoing in the Company's global pivotal Phase 2 AGAVE-201 trial of axatilimab in patients with cGVHD, 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.

- In September 2021, Syndax and Incyte announced that they entered into an exclusive worldwide collaboration and license agreement to develop and commercialize axatilimab. Syndax and Incyte are seeking to develop axatilimab as a backbone therapy for patients with cGVHD as well as in additional immune-mediated diseases where CSF-1R-dependent monocytes and macrophages are believed to contribute to organ fibrosis. In addition to the ongoing global pivotal Phase 2 AGAVE-201 trial of axatilimab monotherapy in patients with cGVHD, the companies also plan to initiate additional trials of axatilimab in patients with cGVHD in 2022, including a Phase 2 trial in combination with a JAK inhibitor in patients with steroid-refractory cGVHD. Beyond cGVHD, Syndax plans to commence a Phase 2 proof of concept trial of axatilimab mid next year in patients with IPF, a serious, life-limiting orphan disease for which axatilimab may represent a much-needed treatment option with a novel mechanism of action.

Corporate Updates

- In September 2021, Syndax announced the expansion of its Board of Directors to nine members with the appointment of Martin H. Huber, M.D. Dr. Huber has over 20 years of academic, biotechnology, and pharmaceutical drug development experience, currently serving as the President of Research and Development (R&D) and Chief Medical Officer of Xilio Therapeutics, Inc.

Third Quarter 2021 Financial Results

As of September 30, 2021, Syndax had cash, cash equivalents and short-term investments of $229.7 million and 52.2 million shares and share equivalents issued and outstanding. This includes 3.3 million pre-funded warrants.

Third quarter 2021 R&D expenses increased to $25.6 million from $14.4 million for the prior year period. The increase was primarily due to increased clinical trial activities and CMC activities for both SNDX-5613 and axatilimab.

General and administrative expenses for the third quarter 2021 increased to $6.8 million from $5.8 million for the prior year period. The increase is primarily due to increased professional fees.

For the three months ended September 30, 2021, Syndax reported a net loss attributable to common stockholders of $20.6 million or $0.40 per share compared to $20.4 million or $0.46 per share for the prior year period.

Financial Update and Guidance

For the full year of 2021, R&D 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, Monday, November 15, 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: 9558836
Domestic Dial-in Number: (855) 251-6663
International Dial-in Number: (281) 542-4259
Live webcast: https://edge.media-server.com/mmc/p/85k2svad

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 and fibrotic diseases, and Syndax's expected 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)	September 30, 2021	December 31, 2020
Cash, cash equivalents and short-term investments	$ 229,714	$ 293,065
Total assets	$ 239,503	$ 300,613
Total liabilities	$ 40,499	$ 48,425
Total stockholders' equity (deficit)	$ 199,004	$ 252,188
Common stock outstanding	48,850,539	47,881,223
Common stock and common stock equivalents*	59,631,478	57,836,910
*Common stock and common stock equivalents:		
Common stock	48,850,539	47,881,223
Common stock warrants (pre-funded)	3,307,952	3,557,952
Common stock and pre-funded stock warrants	52,158,491	51,439,175
Options to purchase common stock	7,340,654	6,379,235
Restricted Stock Units	132,333	18,500
Total common stock and common stock equivalents	59,631,478	57,836,910

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

(In thousands, except share and per share data)	Three Months Ended September 30, 2021	Three Months Ended September 30, 2020	Nine Months Ended September 30, 2021	Nine Months Ended September 30, 2020
License fee revenue	$ 12,375	$ 379	$ 13,133	$ 1,138
Operating expenses:				
Research and development	25,606	14,408	64,348	34,913
General and administrative	6,801	5,824	18,314	17,787
Total operating expenses	32,407	20,232	82,662	52,700
Loss from operations	(20,032)	(19,853)	(69,529)	(51,562)
Other (expense) income, net	(607)	(584)	(1,743)	(1,173)
Net loss	$ (20,639)	$ (20,437)	$ (71,272)	$ (52,735)
Net loss attributable to common stockholders	$ (20,639)	$ (20,437)	$ (71,272)	$ (56,641)
Net loss per share attributable to common stockholders--basic and diluted	$ (0.40)	$ (0.46)	$ (1.38)	$ (1.43)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	51,962,320	44,156,808	51,690,173	39,714,490

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