Syndax Pharmaceuticals Reports Third Quarter 2022 Financial Results and Provides Clinical and Business Update

November 3, 2022

– Updated positive data from Phase 1 portion of AUGMENT-101 trial demonstrates CR/CRh rate of 30% and a median durability of response of 9.1 months –

– Enrollment complete in pivotal Phase 2 AGAVE-201 trial of axatilimab in patients with cGVHD –

– Company remains on track for two U.S. registrational filings by the end of 2023 –

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

WALTHAM, Mass., Nov. 3, 2022 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. (Nasdaq: SNDX), a clinical-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results and provided a business update for the third quarter ended September 30, 2022.

"As we near the end of this transformational year, we are particularly excited to share that updated data from the Phase 1 portion of the ongoing AUGMENT-101 trial of revumenib will be featured during two oral presentations at the American Society of Hematology (ASH) Annual Meeting in December. The rate of patients achieving a complete response (CR/CRh) and the nine-month median duration of CR/CRh response as of the data cutoff, further support the robust clinical profile of revumenib," said Michael A. Metzger, Chief Executive Officer. "The data continue to underscore the potential for revumenib to serve as a first- and best-in-class treatment option for patients with relapsed/refractory (R/R) nucleophosmin 1 (NPM1) mutations and mixed lineage leukemia rearrangements (MLLr). Enrollment continues in AUGMENT-101 and we anticipate reporting topline data from at least one of the pivotal cohorts starting in the third quarter of 2023, with a potential New Drug Application (NDA) filing expected by the end of 2023."

Mr. Metzger added, "We also continue to make significant progress on the development of axatilimab and are pleased to announce that we have completed enrollment in our pivotal AGAVE-201 trial in chronic graft versus host disease (cGVHD). We look forward to announcing topline results from the trial in mid-2023, with a potential biologics license application (BLA) filing expected to follow by the end of 2023."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- Earlier today, the Company announced updated positive data from the Phase 1 portion of the ongoing AUGMENT-101 trial of revumenib in patients with R/R NPM1 mutant or MLLr (also referred to as KMT2Ar) acute leukemia which highlighted a 30% (18/60) CR/CRh rate and a median duration of CR/CRh response of 9.1 months. Additionally, of the 12 patients who achieved a complete response on revumenib treatment and then went on to receive a stem cell transplant, nine (75%) remained in remission as of the data cutoff date, with a median follow-up of 12.3 months. Three patients were treated with revumenib maintenance in the compassionate use setting following stem cell transplant or non-myeloablative stem cell boost, two of whom (67%) remained in remission for over one year. To date, there have been no discontinuations due to treatment-related adverse events. Data reported today will be featured in two oral sessions at the ASH Annual Meeting on Saturday, December 10, 2022. Copies of both abstracts are available on the ASH website at www.hematology.org.

  The Company also announced today that it will host a conference call and webcast to discuss the ASH data presentations on Sunday, December 11, 2022 at 8 a.m. CT / 9 a.m. ET. Joining the call will be members of the Syndax management team as well as Principal Investigators from the AUGMENT-101 trial. For additional information on how to access the event, please visit the Investor section of the Company's website at www.syndax.com.

- The pivotal Phase 2 portion of AUGMENT-101 is ongoing, enrolling patients across each of three distinct trial populations: patients with NPM1 mutant acute myeloid leukemia (AML), patients with MLLr AML, and patients with MLLr acute lymphocytic leukemia (ALL). Based on discussions with the U.S. Food and Drug Administration, AUGMENT-101 may serve as the basis for regulatory filings in each of the three distinct populations. The Company expects completion of enrollment in the first trial to extend into the first quarter of 2023 and to report topline data from at least one of the trials starting in the third quarter of 2023. The Company continues to expect to submit its first NDA filing for revumenib by the end of 2023.

- Two trials, BEAT-AML and AUGMENT-102, are ongoing and will assess the safety, tolerability, and preliminary anti-leukemic efficacy of revumenib, and establish an appropriate Phase 2 dose when used in combination with other approved agents. BEAT-AML is a front-line combination trial of revumenib with venetoclax and azacitidine being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. AUGMENT-102 is a trial assessing revumenib in combination with chemotherapy in patients with R/R mNPM1 or MLLr acute leukemias.

- The Company expects the Australasian Leukaemia and Lymphoma Group (ALLG) to initiate the INTERCEPT trial of

revumenib as monotherapy in patients with AML who are minimal residual disease-positive following initial treatment, in the fourth quarter of 2022. The trial is a part of the INTERCEPT AML Master Clinical Trial, a collaborative clinical trial investigating novel therapies to target early relapse and clonal evolution as pre-emptive therapy in AML. Revumenib is the first menin inhibitor to be included in the INTERCEPT AML Master Clinical Trial.

- The Company remains on track to initiate a proof-of-concept clinical trial of revumenib in patients with unresectable metastatic microsatellite stable colorectal cancer in the fourth quarter of 2022.

Axatilimab

- The Company and its partner, Incyte, today announced completion of enrollment in the pivotal AGAVE-201 trial evaluating axatilimab in patients with cGVHD following two or more prior lines of therapy. The trial is evaluating the safety and efficacy of three dosing regimens 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. The Company expects to report topline data in mid-2023, with the expectation for a BLA filing later in 2023.

- The Company plans to initiate a Phase 2b trial to assess the efficacy, safety and tolerability of axatilimab in patients with idiopathic pulmonary fibrosis (IPF) in the fourth quarter of 2022. This 52-week, randomized, double-blind and placebo-controlled trial is expected to enroll approximately 170 patients. The primary endpoint will assess the change from baseline in forced vital capacity, which is the current registrational endpoint in IPF.

- The Company is working with its partner, Incyte, to initiate a trial testing axatilimab in combination with ruxolitinib in steroid naive cGVHD. The Phase 1 trial is in preparation and is expected to begin in the first quarter of 2023.

Third Quarter 2022 Financial Results

As of September 30, 2022, Syndax had cash, cash equivalents and short-term investments of $337.8 million and 61.3 million common shares and pre-funded warrants outstanding.

Third quarter 2022 research and development expenses increased to $26.9 million from $25.6 million for the prior year period. The increase was primarily due to increased employee related expenses and professional fees partially offset by decreased clinical and manufacturing expenses, in large part the result of axatilimab cost sharing benefits.

General and administrative expenses for the third quarter 2022 increased to $8.2 million from $6.8 million for the prior year period. The increase is primarily due to increased employee related expenses and professional fees.

For the three months ended September 30, 2022, Syndax reported a net loss attributable to common stockholders of $35.4 million, or $0.58 per share, compared to a net loss attributable to common stockholders of $20.6 million, or $0.40 per share, for the prior year period.

Financial Update and Guidance

For the full year of 2022, the Company is lowering its expectations of both research and development and total operating expenses. The Company now expects research and development expenses to be $115 to $125 million and total operating expenses to be $145 to $155 million. This is a reduction from the Company's prior guidance for the full year of 2022 of $130 to $140 million in research and development expenses and $160 to $170 million in total operating expenses. The updated guidance reflects the benefits derived from the shared expenses for our axatilimab cGVHD development program.

In September, Syndax made a debt repayment in connection with the termination of a Loan Agreement. This loan repayment has no adverse impact on Syndax's cash guidance and the Company continues to expect to have sufficient capital to fund operations into the second half of 2024.

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 3, 2022.

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: SYNDAXQ3
Domestic Dial-in Number: 800-225-9448
International Dial-in Number: 203-518-9708
Live webcast: https://www.veracast.com/webcasts/OpenEx/General/S03S7x.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 Pharmaceuticals, Inc.

Syndax Pharmaceuticals is a clinical stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include revumenib (SNDX-5613), a highly selective inhibitor of the Menin–MLL binding interaction, and axatilimab, a monoclonal

antibody that blocks the colony stimulating factor 1 (CSF-1) receptor, both currently in pivotal trials. 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," "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 use of our product candidates to treat various cancer indications and fibrotic diseases, and Syndax's expected fourth 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)	September 30, 2022	December 31, 2021
Cash, cash equivalents, short and long-term investments	$ 337,838	$ 439,936
Total assets	$ 359,687	$ 449,657
Total liabilities	$ 21,585	$ 41,289
Total stockholders' equity (deficit)	$ 338,102	$ 408,368
Common stock outstanding	60,122,661	54,983,105
Common stock and common stock equivalents*	68,877,618	66,011,976
*Common stock and common stock equivalents:		
Common stock	60,122,661	54,983,105
Common stock warrants (pre-funded)	1,142,856	3,975,024
Common stock and pre-funded stock warrants	61,265,517	58,958,129
Options to purchase common stock	7,386,063	6,921,514
Restricted Stock Units	226,038	132,333
Total common stock and common stock equivalents	68,877,618	66,011,976

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

(In thousands, except share and per share data)	Three Months Ended September 30, 2022	Three Months Ended September 30, 2021	Nine Months Ended September 30, 2022	Nine Months Ended September 30, 2021
License fee revenue	$ -	$ 12,375	$ -	$ 13,133
Operating expenses:				
Research and development	26,901	25,606	86,658	64,348
General and administrative	8,240	6,801	23,066	18,314
Total operating expenses	35,141	32,407	109,724	82,662
Loss from operations	(35,141)	(20,032)	(109,724)	(69,529)
Other (expense) income, net	(262)	(607)	(420)	(1,743)
Net loss	$ (35,403)	$ (20,639)	$ (110,144)	$ (71,272)
Net loss attributable to common stockholders	$ (35,403)	$ (20,639)	$ (110,144)	$ (71,272)

Net loss per share attributable to common stockholders--basic and diluted	$ (0.58)	$ (0.40)	$ (1.84)	$ (1.38)
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	60,670,294	51,962,320	59,941,384	51,690,173

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