Syndax Pharmaceuticals Reports Fourth Quarter and Full Year 2022 Financial Results and Provides Clinical and Business Update

February 28, 2023

– Topline data from AUGMENT-101 KMT2Ar patients expected in the third quarter of 2023 –

–Topline data from the pivotal AGAVE-201 trial on track for mid-2023 –

– Two U.S. registrational filings expected by the end of 2023 –

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

WALTHAM, Mass., Feb. 28, 2023 /PRNewswire/ -- Syndax Pharmaceuticals, Inc. (Nasdaq: SNDX), a clinical-stage biopharmaceutical company developing an innovative pipeline of cancer therapies, today reported its financial results for the fourth quarter and full year ended December 31, 2022 and provided a business update.

"With two pivotal data readouts expected later this year and potential regulatory filings to follow shortly thereafter, we expect 2023 will be an extraordinary year for Syndax," said Michael A. Metzger, Chief Executive Officer. "The fourth quarter was marked by significant progress on the clinical, regulatory and operational fronts and we expect this positive momentum to continue in 2023. We had a strong presence at the American Society of Hematology (ASH) Annual Meeting where we presented positive revumenib results from the Phase 1 portion of the AUGMENT-101 trial and the axatilimab Phase 1/2 trial data were published in the *Journal of Clinical Oncology.* These data further support the potential for both our pipeline agents to be first- and best-in-class therapies that could meaningfully change treatment paradigms."

"For revumenib, we are on track to begin reporting topline data from the AUGMENT-101 pivotal trial in the third quarter of this year, with the first data expected to be in patients with KMT2A rearranged (KMT2Ar) acute leukemia and expect to file a New Drug Application (NDA) by year-end 2023. For axatilimab, we also remain on track to report topline results from our pivotal AGAVE-201 trial in chronic graft versus host disease (cGVHD) in mid-2023, with a Biologics License Application (BLA) filing expected to follow by year-end 2023. We look forward to providing updates on all of our progress as we continue to strive toward our mission of realizing a future in which people with cancer live longer and better than ever before."

Recent Pipeline Progress and Anticipated Milestones

Revumenib

- The pivotal Phase 2 portion of AUGMENT-101 is enrolling relapsed/refractory (R/R) patients across distinct trial populations: patients with nucleophosmin mutant (mNPM1) acute myeloid leukemia (AML), patients with KMT2Ar AML, and patients with KMT2Ar acute lymphocytic leukemia (ALL), each of which may serve as the basis for regulatory filings. Following the receipt of Breakthrough Therapy designation from the FDA for revumenib for the treatment of R/R acute leukemia harboring a KMT2A rearrangement, regardless of age or tumor type, and based on discussions with the FDA, the Company will pool data from the AUGMENT-101 cohorts enrolling R/R KMT2Ar AML and R/R KMT2Ar ALL to file a single NDA for the treatment of adult and pediatric KMT2Ar acute leukemia. The Company has completed enrollment of a sufficient number of KMT2Ar acute leukemia patients to support this filing strategy and expects to share topline data from the KMT2Ar cohort in the third quarter of 2023 and submit an NDA filing by the end of 2023. The Company also expects to complete enrollment of the NPM1 AML cohort in the second half of 2023.

- During two oral presentations at the 64th ASH Annual Meeting in December 2022, the Company reported updated positive data from the Phase 1 portion of the ongoing Phase 1/2 AUGMENT-101 trial. As of the March 2022 data cutoff date, 60 patients with R/R mutant NPM1 or KMT2Ar acute leukemia were efficacy evaluable. In the efficacy evaluable population, the overall response rate was 53% (32/60) with a CR/CRh rate of 30% (18/60), and 78% (14/18) of patients with CR/CRh attaining minimal residual disease (MRD) negativity. Additional analyses from the trial indicate that at doses which met the protocol defined criteria for a recommended Phase 2 dose, the CR/CRh rate was 27% in both the KMT2Ar (10/37) and the mutant NPM1 (3/11) patient populations. A total of 38% (12/32) of responders proceeded to transplant. The median time to response in the trial was 1.9 months, and the median duration of CR/CRh response was 9.1 months in the efficacy evaluable population as of data cutoff. Revumenib was well-tolerated, and there were no discontinuations due to treatment related adverse events.

- 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 KMT2Ar acute leukemias.

- The Australasian Leukaemia and Lymphoma Group (ALLG) has initiated the INTERCEPT trial of revumenib as

monotherapy in patients with AML who are minimal residual disease-positive following initial treatment. 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.

- A proof-of-concept clinical trial of revumenib in patients with unresectable metastatic microsatellite stable colorectal cancer has initiated and the Company expects to report initial topline data from the trial by year-end 2023.

Axatilimab

- The Company and its partner, Incyte, expect to report topline data from the pivotal AGAVE-201 trial evaluating axatilimab in patients with cGVHD following two or more prior lines of therapy in mid-2023, with the expectation for a BLA filing by year-end 2023.

- The Company and Incyte announced that results from the Phase 1/2 trial of axatilimab in patients with recurrent or refractory cGVHD following two or more prior lines of therapy were published in the *Journal of Clinical Oncology*. The article, titled "*Axatilimab for chronic graft-versus-host disease after failure of at least two prior systemic therapies: results of a Phase 1/2 study*," is available online.

- The Company plans to initiate a 52-week, randomized, double-blind and placebo-controlled Phase 2b trial to assess the efficacy, safety and tolerability of axatilimab in patients with idiopathic pulmonary fibrosis (IPF) in the first half of 2023. 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 Incyte to initiate a trial testing axatilimab in combination with ruxolitinib in steroid naive cGVHD. The Phase 1 trial is expected to begin later this year.

Corporate Updates

- In December 2022, the Company announced the appointment of Steve Sabus as Chief Commercial Officer. Mr. Sabus brings to Syndax nearly thirty years of commercial experience launching drugs and building sales and marketing organizations within the biopharmaceutical industry.

Fourth Quarter and Full Year 2022 Financial Results

As of December 31, 2022, Syndax had cash, cash equivalents, short-term and long-term investments of $481.3 million and 69.3 million common shares and prefunded warrants outstanding.

Fourth quarter 2022 research and development expenses increased to $31.8 million from $23.9 million, and for the full year increased to $118.5 million compared to $88.2 million for 2021. The increase was primarily due to increased clinical activities as well as 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 fourth quarter 2022 increased to $10.2 million from $6.9 million and for the full year increased to $33.3 million compared to $25.2 million for 2021. The increase is primarily due to increased pre-commercialization activities as well as employee related expenses and professional fees.

For the three months ended December 31, 2022, Syndax reported a net loss attributable to common stockholders of $39.2 million, or $0.62 per share, compared to a net gain attributable to common stockholders of $96.2 million, or $1.81 per share, for the prior year period. For the year ended December 31, 2022, Syndax reported a net loss attributed to common stockholders of $149.3 million or $2.46 per share, compared to a net gain attributable to common stockholders of $24.9 million or $0.48 per share for the prior year.

Financial Update and Guidance

In December 2022, Syndax issued 7,840,909 shares of its common stock at a price to the public of $22.00 per share. This includes the exercise in full by the underwriters of their option to purchase up to 1,022,727 additional shares of common stock. As a result, Syndax received aggregate net proceeds of $162.0 million after deducting underwriting discounts and commissions and estimated offering expenses payable by Syndax.

For the first quarter of 2023, the Company expects research and development expenses to be $30 to $35 million and total operating expenses to be $40 to $45 million. For the full year of 2023, the Company expects research and development expenses to be $160 to $175 million and total operating expenses to be $225 to $240 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, February 28, 2023.

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: SNDXQ422

Domestic Dial-in Number: 800-245-3047
International Dial-in Number: 203-518-9765
Live webcast: https://www.veracast.com/webcasts/OpenEx/General/SNDXQ4.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, a highly selective inhibitor of the Menin–KMT2A 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 first quarter and full year research and development expenses, and expected first quarter and full year 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)	December 31, 2022	December 31, 2021
Cash, cash equivalents, short and long-term investments	$ 481,271	$ 439,936
Total assets	$ 497,236	$ 449,657
Total liabilities	$ 29,787	$ 41,289
Total stockholders' equity (deficit)	$ 467,449	$ 408,368
Common stock outstanding	68,111,385	54,983,105
Common stock and common stock equivalents*	77,460,706	66,011,976
*Common stock and common stock equivalents:		
Common stock	68,111,385	54,983,105
Common stock warrants (pre-funded)	1,142,856	3,975,024
Common stock and pre-funded stock warrants	69,254,241	58,958,129
Options to purchase common stock	7,981,677	6,921,514
Restricted Stock Units	224,788	132,333
Total common stock and common stock equivalents	77,460,706	66,011,976

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

(In thousands, except share and per share data)	Three Months Ended December 31, 2022	Three Months Ended December 31, 2021	Twelve Months Ended December 31, 2022	Twelve Months Ended December 31, 2021
License fee revenue	$ -	$ 126,576	$ -	$ 139,709
Operating expenses:				
Research and development	31,841	23,900	118,499	88,248
General and administrative	10,192	6,927	33,258	25,241
Total operating expenses	42,033	30,827	151,757	113,489
Loss from operations	(42,033)	95,749	(151,757)	26,220
Other income (expense), net	2,839	449	2,419	(1,294)
Net loss	$ (39,194)	$ 96,198	$ (149,338)	$ 24,926
Net loss attributable to common stockholders	$ (39,194)	$ 96,198	$ (149,338)	$ 24,926
Net loss per share attributable to common stockholders--basic and diluted	$ (0.62)	$ 1.81	$ (2.46)	$ 0.48
Weighted-average number of common stock used to compute net loss per share attributable to common stockholders--basic and diluted	63,192,750	53,176,335	60,760,906	52,064,809

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