Syndax Announces Data Presentations at EHA 2025 Showcasing Revuforj® (revumenib) and Niktimvo™ (axatilimab-csfr)

May 14, 2025

– Revumenib abstracts highlight compelling results in acute leukemia across the frontline and R/R setting and multiple genetic populations, including mNPM1, KMT2Ar, and NUP98r –

– Axatilimab abstracts highlight the robust responses observed in different organs and subgroups of patients with chronic GVHD in the AGAVE-201 trial –

NEW YORK, May 14, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today announced that multiple abstracts showcasing clinical data for Revuforj® (revumenib) and Niktimvo™ (axatilimab-csfr) were accepted for presentation at the 30th European Hematology Association (EHA) Annual Congress Meeting being held June 12-15, 2025, in Milan, Italy.

"The data being presented at EHA showcase the potential for revumenib and axatilimab to transform the treatment paradigm for patients with acute leukemia and chronic GVHD, respectively," said Nick Botwood, MBBS, Head of Research & Development and Chief Medical Officer at Syndax. "We and our clinical collaborators are particularly pleased to share updated data from the BEAT AML frontline trial of revumenib in combination with venetoclax and azacitidine in mNPM1 and KMT2Ar AML. We anticipate that the oral presentation of these encouraging results will drive even greater physician interest in the ongoing EVOLVE-2 frontline global registration trial of this same combination."

Key data being presented at EHA 2025 include:

- An oral presentation reporting updated results from the Phase 1 BEAT AML trial evaluating the combination of revumenib with venetoclax and azacitidine in newly diagnosed mutant NPM1 (mNPM1) or KMT2A-rearranged (KMT2Ar) acute myeloid leukemia (AML) patients aged 60 years or older.
- A poster presentation reporting data from the 77 patients with relapsed or refractory (R/R) mNPM1 AML who met the efficacy evaluable criteria in the Phase 2 cohort of the AUGMENT-101 trial of revumenib. The poster will expand upon the topline data that was reported from this population in December 2024.
- A poster presentation reporting outcomes in patients with R/R NUP98r acute leukemias in the Phase 1 portion of the AUGMENT-101 trial.
- A poster presentation reporting the dynamics of overall and organ-specific responses in the AGAVE-201 trial of axatilimab in chronic graft-versus-host disease (GVHD).

The accepted abstracts listed below are now available online at the EHA conference website. Copies of the oral and poster presentations will be made available in the 'Publications & Meetings Presentations' section of the Syndax website after the presentations occur.

Presentations During EHA 2025 (all times in CEST):

Revumenib

Abstract Titles	Presentation Details
Azacitidine, Venetoclax, and Revumenib for Newly Diagnosed Older Adults with Acute Myeloid Leukemia (AML) and NPM1 mutation or KMT2A rearrangement: Updated Results from the Beat AML Consortium	Oral presentation Abstract #: S138 Thursday, June 12 Session 5:00-6:15 pm
Patients With Relapsed or Refractory (R/R) Nucleophosmin 1–Mutated (NPM1m) Acute Myeloid Leukemia (AML): Updated Results From the Phase 2 AUGMENT-101 Study	Poster presentation (Session 2) Abstract #: PS1467 Saturday, June 14 at 6:30-7:30 pm
Updated Results and Longer Follow-Up From the AUGMENT-101 Phase 2 Study of Revumenib in All Patients With Relapsed or Refractory (R/R) KMT2Ar Acute Leukemia	Poster presentation (Session 2) Abstract #: PS1473 Saturday, June 14 at 6:30-7:30 pm *Encore presentation*
Revumenib Activity in Patients with NUP98r Acute Leukemia: Results from the AUGMENT-101 Phase 1 Study	Poster presentation (Session 2) Abstract #: PS1501 Saturday, June 14 at 6:30-7:30 pm
Trial in Progress: A Phase 1 Study of Revumenib + Intensive Chemotherapy in Patients With Newly Diagnosed Acute Myeloid Leukemia Harboring Genetic Alterations in KMT2A, NPM1, or NUP98 (SNDX-5613-0708)	Publication only* Abstract: PB2576
Treatment Patterns in Patients with Relapsed or Refractory NPM1 Mutated (NPM1m) Acute Myeloid Leukemia (AML) in the United States (US): Real-world Data Analysis	Publication only* Abstract: PB2513

** Abstracts will be published in the online Abstract Book, a supplement of HemaSphere (EHA's official journal), EHA Library and Congress platform.*

Axatilimab

Abstract Titles	Presentation Details

The Effects of Prior Lines of Therapy on Clinical Outcomes for Patients With Chronic Graft-Versus-Host Disease Receiving Axatilimab: A Post Hoc Analysis of AGAVE-201	Poster presentation (Session 2) Abstract #: PS2029 Saturday, June 14 at 6:30-7:30 pm *Encore presentation*
Dynamics of Overall and Organ-Specific Responses to Axatilimab in Chronic Graft-Versus-Host Disease: Analysis From the AGAVE-201 Study	Poster presentation (Session 1) Abstract #: PF1035 Friday, June 13 at 6:30-7:30 pm *Encore presentation*
Trial in Progress: A Randomized, Open-Label, Phase 3 Study of Axatilimab Versus Best Available Therapy in Patients With Chronic Graft-Versus-Host Disease After ≥2 Prior Lines of Systemic Therapy	Poster presentation (Session 1) Abstract #: PF1090 Friday, June 13 at 6:30-7:30 pm
Correlations of Clinician-Reported Responses With Other Response Measures in Patients With Chronic Graft-Versus-Host Disease: An Analysis From the AGAVE-201 Trial	Poster presentation (Session 1) Abstract #: PF1041 Friday, June 13 at 6:30-7:30 pm *Encore presentation*

About Revuforj® (revumenib)

Revuforj (revumenib) is an oral, first-in-class, selective menin inhibitor that is FDA approved for the treatment of relapsed or refractory (R/R) acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in adult and pediatric patients one year and older.

Revumenib is in development for the treatment of R/R acute myeloid leukemia (AML) with a nucleophosmin 1 mutation (mNPM1). Positive pivotal data from the AUGMENT-101 trial in this population with revumenib as a monotherapy were recently published and the Company submitted a supplemental NDA for revumenib in R/R mNPM1 AML in April 2025. Additionally, multiple trials of revumenib in combination with standard-of-care agents in mNPM1 AML or KMT2A-rearranged acute leukemia are ongoing or planned across the treatment landscape, including in newly diagnosed patients.

Revumenib was previously granted Orphan Drug Designation for the treatment of AML, ALL and acute leukemias of ambiguous lineage (ALAL) by the U.S. FDA and for the treatment of AML by the European Commission. The U.S. FDA also granted Fast Track designation to revumenib for the treatment of adult and pediatric patients with R/R acute leukemias harboring a KMT2A rearrangement or NPM1 mutation and Breakthrough Therapy Designation for the treatment of adult and pediatric patients with R/R acute leukemia harboring a KMT2A rearrangement.

About Niktimvo™ (axatilimab-csfr)

Niktimvo (axatilimab-csfr) is a first-in-class colony stimulating factor-1 receptor (CSF-1R)-blocking antibody approved for use in the U.S. for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs).

In 2016, Syndax licensed exclusive worldwide rights to develop and commercialize axatilimab from UCB. In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab in chronic GVHD and any future indications.

Axatilimab is being studied in frontline combination trials in chronic GVHD – a Phase 2 combination trial with ruxolitinib (NCT06388564) and a Phase 3 combination trial with steroids (NCT06585774) are underway. Axatilimab is also being studied in an ongoing Phase 2 trial in patients with idiopathic pulmonary fibrosis (NCT06132256).

Niktimvo is a trademark of Incyte.

Revuforj (revumenib)

IMPORTANT SAFETY INFORMATION

WARNING: DIFFERENTIATION SYNDROME

Differentiation syndrome, which can be fatal, has occurred with Revuforj. Signs and symptoms may include fever, dyspnea, hypoxia, pulmonary infiltrates, pleural or pericardial effusions, rapid weight gain or peripheral edema, hypotension, and renal dysfunction. If differentiation syndrome is suspected, immediately initiate corticosteroid therapy and hemodynamic monitoring until symptom resolution.

WARNINGS AND PRECAUTIONS

Differentiation syndrome: Revuforj can cause fatal or life-threatening differentiation syndrome (DS). Symptoms of DS, including those seen in patients treated with Revuforj, include fever, dyspnea, hypoxia, peripheral edema, pleuropericardial effusion, acute renal failure, and/or hypotension. In clinical trials, DS occurred in 39 (29%) of 135 patients treated with Revuforj. DS was Grade 3 or 4 in 13% of patients and fatal in one. The median time to onset was 10 days (range 3-41 days). Some patients experienced more than 1 DS event. Treatment interruption was required for 7% of patients, and treatment was withdrawn for 1%.

Reduce the white blood cell count to less than 25 Gi/L prior to starting Revuforj. If DS is suspected, immediately initiate treatment with systemic corticosteroids (e.g., dexamethasone 10-mg IV every 12 hours in adults or dexamethasone 0.25-mg/kg/dose IV every 12 hours in pediatric patients weighing less than 40 kg) for a minimum of 3 days and until resolution of signs and symptoms. Institute supportive measures and hemodynamic monitoring until improvement. Interrupt Revuforj if severe signs and/or symptoms persist for more than 48 hours after initiation of systemic corticosteroids, or earlier if life-threatening symptoms occur such as pulmonary symptoms requiring ventilator support. Restart steroids promptly if DS recurs after tapering corticosteroids.

QTc interval prolongation: In the clinical trials, QTc interval prolongation was reported as an adverse reaction in 39 (29%) of 135 patients treated with Revuforj. QTc interval prolongation was Grade 3 in 12% of patients. The heart-rate corrected QT interval (using Fridericia's method) (QTcF) was greater than 500 msec in 8%, and the increase from baseline QTcF was greater than 60 msec in 18%. Revuforj dose reduction was required for 5% of patients due to QTc interval prolongation. QTc prolongation occurred in 16% of the 31 patients less than 17 years old, 33% of the 88 patients 17 years to less than 65 years old, and in 50% of the 16 patients 65 years or older.

Correct electrolyte abnormalities, including hypokalemia and hypomagnesemia, prior to treatment with Revuforj. Perform an electrocardiogram (ECG) prior to initiation of Revuforj, and do not initiate Revuforj in patients with QTcF >450 msec. Perform an ECG at least once weekly for the first 4 weeks and at least monthly thereafter. In patients with congenital long QTc syndrome, congestive heart failure, electrolyte abnormalities, or those who are taking medications known to prolong the QTc interval, more frequent ECG monitoring may be necessary. Concomitant use with drugs known to prolong the QTc interval may increase the risk of QTc interval prolongation.

- Interrupt Revuforj if QTcF increases >480 msec and <500 msec, and restart Revuforj at the same dose twice daily after the QTcF interval returns to ≤480 msec
- Interrupt Revuforj if QTcF increases >500 msec or by >60 msec from baseline, and restart Revuforj twice daily at the lower-dose level after the QTcF interval returns to ≤480 msec
- Permanently discontinue Revuforj in patients with ventricular arrhythmias and in those who develop QTc interval prolongation with signs or symptoms of life-threatening arrhythmia.

Embryo-fetal toxicity: Revuforj can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Advise females of reproductive potential and males with female partners of reproductive potential to use effective contraception during treatment with Revuforj and for 4 months after the last dose of Revuforj.

ADVERSE REACTIONS

Fatal adverse reactions occurred in 4 (3%) patients who received Revuforj, including 2 with differentiation syndrome, 1 with hemorrhage, and 1 with sudden death.

Serious adverse reactions were reported in 99 (73%) patients. The most frequent **serious adverse reactions** (≥5%) were infection (24%), febrile neutropenia (19%), bacterial infection (17%), differentiation syndrome (12%), hemorrhage (9%), and thrombosis (5%).

The most **common adverse reactions** (≥20%) including laboratory abnormalities, were hemorrhage (53%), nausea (51%), phosphate increased (50%), musculoskeletal pain (42%), infection (41%), aspartate aminotransferase increased (37%), febrile neutropenia (35%), alanine aminotransferase increased (33%), parathyroid hormone intact increased (33%), bacterial infection (31%), diarrhea (30%), differentiation syndrome (29%), electrocardiogram QT prolonged (29%), phosphate decreased (25%), triglycerides increased (25%), potassium decreased (24%), decreased appetite (24%), constipation (23%), edema (23%), viral infection (23%), fatigue (22%), and alkaline phosphatase increased (21%).

DRUG INTERACTIONS

Drug interactions can occur when Revuforj is concomitantly used with:

- Strong CYP3A4 inhibitors: reduce Revuforj dose
- Strong or moderate CYP3A4 inducers: avoid concomitant use with Revuforj
- QTc-prolonging drugs: avoid concomitant use with Revuforj. If concomitant use is unavoidable, obtain ECGs when initiating, during concomitant use, and as clinically indicated. Withhold Revuforj if the QTc interval is >480 msec. Restart Revuforj after the QTc interval returns to ≤480 msec.

SPECIFIC POPULATIONS

Lactation: advise lactating women not to breastfeed during treatment with Revuforj and for 1 week after the last dose.

Pregnancy and testing: Revuforj can cause fetal harm when administered to a pregnant woman. Verify pregnancy status in females of reproductive potential within 7 days prior to initiating Revuforj.

Pediatric: monitor bone growth and development in pediatric patients.

Geriatric: compared to younger patients, the incidences of QTc prolongation and edema were higher in patients 65 years and older.

Infertility: based on findings in animals, Revuforj may impair fertility. The effects on fertility were reversible.

To report SUSPECTED ADVERSE REACTIONS, contact Syndax Pharmaceuticals at 1-888-539-3REV or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see Full Prescribing Information, including BOXED WARNING.

Niktimvo (axatilimab-csfr)

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS
Infusion-Related Reactions
Niktimvo™ (axatilimab-csfr) can cause infusion-related reactions. Infusion-related reactions, including hypersensitivity reactions, occurred in 18% of patients who received Niktimvo in the clinical trial (AGAVE-201), with Grade 3 or 4 reactions in 1.3%.

Premedicate with an antihistamine and an antipyretic for patients who have previously experienced an infusion-related reaction to Niktimvo. Monitor patients for signs and symptoms of infusion-related reactions, including fever, chills, rash, flushing, dyspnea, and hypertension. Interrupt or slow the rate of infusion or permanently discontinue Niktimvo based on severity of the reaction.

Embryo-Fetal Toxicity
Based on its mechanism of action, Niktimvo may cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to the fetus. Advise females of reproductive potential to use effective contraception during treatment with Niktimvo and for 30 days after the last dose.

ADVERSE REACTIONS
Serious adverse reactions occurred in 44% of patients who received Niktimvo (N=79). Serious adverse reactions in >2 patients included infection (pathogen unspecified) (14%), viral infection (14%) and respiratory failure (5.1%). Permanent discontinuation of Niktimvo due to an adverse reaction occurred in 10% of patients and dose reduction due to adverse reaction occurred in 8% of patients. Dose interruptions due to an adverse reaction occurred in 44% of patients. The adverse reactions leading to dose interruption in >2 patients were viral infection, infection (pathogen unspecified), bacterial infection, musculoskeletal pain, and pyrexia.

The most common (≥15%) adverse reactions, including laboratory abnormalities, were increased aspartate aminotransferase (AST), infection

(pathogen unspecified), increased alanine aminotransferase (ALT), decreased phosphate, decreased hemoglobin, viral infection, increased gamma glutamyl transferase (GGT), musculoskeletal pain, increased lipase, fatigue, increased amylase, increased calcium, increased creatine phosphokinase (CPK), increased alkaline phosphatase (ALP), nausea, headache, diarrhea, cough, bacterial infection, pyrexia, and dyspnea.

Clinically relevant adverse reactions in <10% of patients who received Niktimvo included:

- *Eye disorders:* periorbital edema
- *Skin and subcutaneous skin disorders:* pruritus
- *Vascular disorders:* hypertension

Immunogenicity: Anti-Drug Antibody–Associated Adverse Reactions
Across treatment arms in patients with cGVHD who received Niktimvo in clinical trials, among the patients who developed anti-drug antibodies (ADAs), hypersensitivity reactions occurred in 26% (13/50) of patients with neutralizing antibodies (NAb) and in 4% (2/45) of those without NAb.

USE IN SPECIFIC POPULATIONS
Lactation
Because of the potential for serious adverse reactions in a breastfed child, advise women not to breastfeed during treatment and for 30 days after the last dose of Niktimvo.

Females and Males of Reproductive Potential
Pregnancy Testing
Verify pregnancy status in females of reproductive potential prior to initiating Niktimvo.

Contraception
Females
Advise females of reproductive potential to use effective contraception during treatment with Niktimvo and for 30 days after the last dose of Niktimvo.

DOSAGE AND ADMINISTRATION
Dosage Modifications for Adverse Reactions
Monitor aspartate aminotransferase (AST), alanine aminotransferase (ALT), alkaline phosphatase (ALP), creatine phosphokinase (CPK), amylase, and lipase prior to the start of Niktimvo therapy, every 2 weeks for the first month, and every 1 to 2 months thereafter until abnormalities are resolved. See Table 1 in the Prescribing Information for more recommendations.

Please see the full Prescribing Information for Niktimvo.

About Syndax

Syndax Pharmaceuticals is a commercial-stage biopharmaceutical company advancing innovative cancer therapies. Highlights of the Company's pipeline include Revuforj® (revumenib), an FDA-approved menin inhibitor, and Niktimvo™ (axatilimab-csfr), an FDA-approved monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor. Fueled by our commitment to reimagining cancer care, Syndax is working to unlock the full potential of its pipeline and is conducting several clinical trials across the continuum of treatment. For more information, please visit www.syndax.com/ or follow the Company on X 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 "anticipate," "believe," "could," "estimate," "expects," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative or plural of those terms, 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 acceptance of Syndax and its partners' products in the marketplace, sales, marketing, manufacturing and distribution requirements, and the potential use of its product candidates to treat various cancer indications and fibrotic diseases. 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 to Revuforj's or Niktimvo's commercial availability; changes in expected or existing competition; changes in the regulatory environment; 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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