Syndax Highlights Leadership in Menin Inhibition at ASH 2025 with Multiple Revuforj® (revumenib) Presentations Spanning the Acute Leukemia Treatment Continuum

December 8, 2025

– First real-world evidence for a menin inhibitor shows 77% ORR (10/13), 31% (4/13) CR/CRh, 75% (9/12) MRD negativity, and favorable tolerability among primarily R/R NPM1m, KMT2Ar, and NUP98r acute leukemia pts –

– Retrospective review shows revumenib was well tolerated as post-HSCT maintenance in children with KMT2Ar and NUP98r; all pts were alive and 90% (9/10) were relapse free at median follow-up of 19 months –

– Ph 2 SAVE trial of revumenib with venetoclax/HMA in newly diagnosed NPM1m and KMT2Ar AML shows 86% ORR (18/21), 76% CR (16/21), and 100% (18/18) MRD negativity among responders –

– Ph 1 trial of revumenib with intensive chemotherapy in newly diagnosed NPM1m and KMT2Ar AML pts shows favorable tolerability and robust activity, including 92% (24/26) CRc –

NEW YORK, Dec. 08, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today highlighted key Revuforj® (revumenib) presentations spanning the acute leukemia treatment continuum that have been presented at the 67th American Society of Hematology (ASH) Annual Meeting being held in Orlando, Florida, December 6-9, 2025. In total, Syndax and its collaborators will present 12 Revuforj abstracts at the 2025 ASH Annual Meeting. Revuforj is the Company's oral, first-in-class, FDA-approved menin inhibitor.

“We are thrilled to share new data, including the first real-world evidence for the class, showing that revumenib is highly efficacious with a favorable safety profile in multiple acute leukemia subtypes and settings, including in combination with standard of care therapies. Notably, the datasets show deep responses in R/R and frontline NPM1m and KMT2Ar acute leukemia, as well as encouraging safety and early efficacy in post-HSCT maintenance,” said Nick Botwood, MBBS, Head of Research & Development and Chief Medical Officer at Syndax. “With the initiation earlier this year of the pivotal frontline trial in patients unfit for intensive chemotherapy, and the recent initiation of our pivotal frontline trial in fit patients, Syndax is well positioned to further expand the clinical data supporting revumenib and lead in the frontline setting with this exciting new class of therapy.”

Overview of Key Revumenib Data Presented at the 67th ASH Annual Meeting

Early real-world experience with revumenib outside of a clinical trial setting: A single center retrospective review of efficacy and tolerability (Abstract #3448)

This presentation reported efficacy and tolerability data from a single-center retrospective review of 17 patients treated with Revuforj outside of a clinical trial. The median age was 54 years (range: 23-79). 47% (8/17) of patients had KMT2Ar, 29% (5/17) had NPM1m, and 18% (3/17) had NUP98r acute leukemia. Patients had a median of four prior lines of therapy (range: 0-6), including 71% (12/17) with prior venetoclax and 35% (6/17) with prior hematopoietic stem cell transplant (HSCT).

Revuforj was used as part of combination therapy in 82% (14/17) of patients, with venetoclax and a hypomethylating agent being the most common partner. Of the 17 patients treated with Revuforj, 13 patients were included in the efficacy analysis (four patients were excluded: one with false positive KMT2Ar test result, one who received Revuforj only as post-HSCT maintenance, and two who received Revuforj as a treatment for measurable residual disease (MRD) positive disease). The overall response rate (ORR) was 77% (10/13), the composite complete remission (CRc) rate was 62% (8/13), and the CR/CRh rate was 31% (4/13). 75% (9/12) of patients were MRD negative at best response. Four patients proceeded to receive a HSCT after revumenib therapy. Among patients who received a HSCT, 75% (3/4) received revumenib as post-HSCT maintenance.

The early real-world evidence supports the safety and tolerability of Revuforj in clinical practice. The rate of Grade 3 or 4 non-hematological adverse events was 24% (4/17). There was no differentiation syndrome (DS) or QTc prolongation above Grade 3. DS and QTc did not lead to treatment discontinuation in any patient. The rate of revumenib dose reductions and discontinuations was low at 6% (1/17) and 6% (1/17), respectively.

“Among a real-world group of heavily pre-treated NPM1m, KMT2Ar, and NUP98r patients who received Revuforj as a monotherapy or in combination, it is very encouraging to observe the vast majority of patients achieve MRD negative responses and to see a meaningful number of patients proceed to a stem cell transplant with durable ongoing remissions. Along with excellent clinical activity, we observed that Revuforj was well-tolerated, including in combination with other therapies,” said David Sallman, M.D., Associate Member in the Department of Malignant Hematology at Moffitt Cancer Center. “Our results underscore the potential for Revuforj to transform the standard of care for patients with menin-dependent acute leukemias.”

Retrospective review of revumenib as post-HSCT maintenance in children with HOX-driven AML (Abstract #3461)

This study retrospectively analyzed ten pediatric patients with HOX-driven AML who received revumenib maintenance after HSCT at a single-center. The median age was 10 years (range: 1-18). 80% (8/10) had KMT2Ar and 20% (2/10) had NUP98r AML. 50% (5/10) of patients had ≥2 prior HSCTs.

Patients completed a median of 2 cycles (range: 1-4) of revumenib pre-HSCT. Patients began revumenib following HSCT, with planned continuation for up to 12 months. Patients resumed revumenib a median of 111 days (range: 58-175) post-HSCT. Patients completed a median of 11 revumenib cycles (range: 3-25) post-HSCT. At a median follow-up of 19 months (range: 4-41), all ten patients were alive, with one relapse, yielding a 90% relapse-free survival.

Revumenib was well tolerated in the post-HSCT maintenance setting, with most adverse events being low grade and manageable. No patients discontinued therapy due to drug-related toxicity.

Results from Phase 2 SAVE trial of revumenib with venetoclax and decitabine/cedazuridine in patients with newly diagnosed AML (Abstract #47)

The Phase 2 SAVE trial is an investigator-sponsored trial evaluating the all-oral combination of revumenib with venetoclax and decitabine/cedazuridine in pediatric and adult patients with R/R or newly diagnosed AML or MPAL harboring either NPM1m, KMT2Ar, or NUP98r alterations. Patients ≥12

years of age with these molecular subtypes who were not candidates for high-intensive chemotherapy were eligible to enroll in the newly diagnosed cohort.

At data cutoff (November 10, 2025), 21 newly diagnosed patients had been enrolled. The median age was 70 years (range: 60-83). 67% (14/21) had NPM1m and 33% (7/21) had KMT2Ar AML.

High rates of response, MRD negativity, and HSCT were observed among NPM1m and KMT2Ar AML patients who received the all-oral combination. The ORR was 86% (18/21) and the complete remission (CR) rate was 76% (16/21). The MRD negativity rate by flow cytometry was 100% (18/18) among responders. 33% (7/21) of patients proceeded to HSCT after receiving the combination, with 57% (4/7) having resumed revumenib as post-HSCT maintenance at the data cutoff.

With a median follow-up of 9 months, the median overall survival (OS) and event-free survival (EFS) were not reached.

The combination was generally well tolerated. The most common (>20%) Grade ≥3 treatment-emergent adverse events (TEAEs) were febrile neutropenia (48%), thrombocytopenia (33%), and neutropenia (24%). There was no treatment-emergent QTc prolongation above Grade 2. Two (10%) patients had Grade 3 treatment-emergent differentiation syndrome (DS) which promptly resolved with steroids. There was no treatment-emergent DS above Grade 3.

Results from Phase 1 trial of revumenib with intensive chemotherapy in patients with newly diagnosed KMT2Ar or NPM1m AML (SNDX-5613-0708) (Abstract #3425)

Preliminary data were reported from the Phase 1, multi-center, open-label, dose-escalation and dose-expansion trial of revumenib in combination with intensive chemotherapy (SNDX-5613-0708). Adults with newly diagnosed KMT2Ar, NPM1m, or NUP98r AML who were candidates for intensive chemotherapy were eligible to enroll. The primary endpoints were the occurrence of dose-limiting toxicities (DLTs) and safety. The secondary endpoints were PK parameters. Exploratory endpoints included the rate of CR, CRc, ORR, and MRD negative CR.

At the data cutoff (September 30, 2025), 30 patients had been enrolled and treated across two revumenib dose levels (DL1: revumenib 110 or 220 mg q12hr with/without strong CYP3A4i; DL2: 160 or 270 mg q12hr with/without strong CYP3A4i). The median age was 49 years (range: 19-71). 63% (19/30) had KMT2Ar and 37% (11/30) had NPM1m.

The safety profile of the combination was consistent with the profile for intensive chemotherapy alone. The most common treatment-related adverse events (TRAEs) of any Grade were decreases in neutrophil count (31%), anemia (23%), nausea (23%), and vomiting (23%) in DL1, and anemia (18%) in DL2. No cases of DS were reported. No events of QTc prolongation above Grade 3 were reported. The rates of TEAEs leading to revumenib reductions or discontinuations were low at 7% and 13%, respectively. One DLT of Grade 3 QTc prolongation was reported in DL1 in a patient taking several concomitant medications that can also prolong the QTc interval. This patient discontinued revumenib during Cycle 1; of note, at the end of Cycle 1, the patient had achieved MRD-negative CR and proceeded to HSCT.

The early data show encouraging clinical activity with the combination. Across both dose levels combined, the ORR was 96% (25/26), CRc rate was 92% (24/26), CR rate was 69% (18/26), and MRD negative CR rate was 86% (12/14) among patients with MRD results available. Data will continue to mature, and responses could deepen further with longer follow-up, particularly in DL2.

Time to count recovery was rapid and similar at both dose levels. The median time to neutrophil (≥1,000 cells/μL) recovery among CRc responders was 29 days in both DL1 and DL2 in Cycle 1. The median time to platelet (≥100,000 cells/μL) recovery among CRc responders was 28 days and 29 days in DL1 and DL2, respectively, in Cycle 1.

Syndax Investor Event

The Company will host an investor event on Monday, December 8, 2025, at 7:00 a.m. ET to discuss key Revuforj and Niktimvo data presented at the 67th ASH Annual Meeting. The live audio webcast and accompanying slides for the event may be accessed through the Events & Presentations page of the Company's website or directly through the meeting link here.

For those unable to join the live webcast, a replay will be available on the Investors section of the Company's website at www.syndax.com for a limited time.

About Revuforj® (revumenib)

Revuforj (revumenib) is an oral, first-in-class 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 as determined by an FDA-authorized test in adult and pediatric patients one year and older. Revuforj is also indicated for the treatment of R/R acute myeloid leukemia (AML) with a susceptible nucleophosmin 1 (NPM1) mutation in adult and pediatric patients one year and older who have no satisfactory alternative treatment options.

Multiple trials of revumenib are ongoing or planned across the treatment landscape, including in combination with standard of care therapies in newly diagnosed patients with NPM1m or KMT2Ar AML.

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.

IMPORTANT SAFETY INFORMATION

WARNING: DIFFERENTIATION SYNDROME, QTc PROLONGATION, and TORSADES DE POINTES

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.

QTc prolongation and Torsades de Pointes have occurred in patients receiving Revuforj. Correct hypokalemia and hypomagnesemia prior to and during treatment. Do not initiate Revuforj in patients with QTcF > 450 msec. If QTc interval prolongation occurs, interrupt, reduce, or permanently discontinue Revuforj.

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, rash, and/or hypotension.

In clinical trials, DS occurred in 60 (25%) of 241 patients treated with Revuforj at the recommended dosage for relapsed or refractory acute leukemia. Among those with a KMT2A translocation, DS occurred in 33% of patients with acute myeloid leukemia (AML), 33% of patients with mixed-phenotype acute leukemia (MPAL), and 9% of patients with acute lymphoblastic leukemia (ALL); DS occurred in 18% of patients with NPM1m AML. DS was Grade 3 or 4 in 12% of patients and fatal in 2 patients. The median time to initial onset was 9 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 and Torsades de Pointes: Revuforj can cause QT (QTc) interval prolongation and Torsades de Pointes.

Of the 241 patients treated with Revuforj at the recommended dosage for relapsed or refractory acute leukemia in clinical trials, QTc interval prolongation was reported as an adverse reaction in 86 (36%) patients. QTc interval prolongation was Grade 3 in 15% and Grade 4 in 2%. The heart-rate corrected QT interval (using Fridericia's method) (QTcF) was greater than 500 msec in 10%, and the increase from baseline QTcF was greater than 60 msec in 24%. Revuforj dose reduction was required for 7% due to QTc interval prolongation. QTc prolongation occurred in 21% of the 34 patients less than 17 years old, 35% of the 146 patients 17 years to less than 65 years old, and 46% of the 61 patients 65 years or older. One patient had a fatal outcome of cardiac arrest, and one patient had non-sustained Torsades de Pointes.

Correct electrolyte abnormalities, including hypokalemia and hypomagnesemia, prior to and throughout 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 9 (4%) patients who received Revuforj, including 4 with sudden death, 2 with differentiation syndrome, 2 with hemorrhage, and 1 with cardiac arrest.

Serious adverse reactions were reported in 184 (76%) patients. The most frequent serious adverse reactions (≥10%) were infection (29%), febrile neutropenia (20%), bacterial infection (15%), differentiation syndrome (13%), and hemorrhage (11%).

The **most common adverse reactions** (≥20%) including laboratory abnormalities, were phosphate increased (51%), hemorrhage (48%), nausea (48%), infection without identified pathogen (46%), aspartate aminotransferase increased (44%), alanine aminotransferase increased (40%), creatinine increased (38%), musculoskeletal pain (37%), febrile neutropenia (37%), electrocardiogram QT prolonged (36%), potassium decreased (34%), parathyroid hormone intact increased (34%), alkaline phosphatase increased (33%), diarrhea (29%), bacterial infection (27%), triglycerides increased (27%), phosphate decreased (25%), differentiation syndrome (25%), fatigue (24%), edema (24%), viral infection (23%), decreased appetite (20%), and constipation (20%).

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.

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

Pediatric: monitor bone growth and development in pediatric patients**.**

Geriatric: no overall differences were observed in the effectiveness of Revuforj between patients who were 65 years and older, and younger patients. Compared to younger patients, the incidences of QTc prolongation and edema were higher in patients 65 years and older.

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 WARNINGS.

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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Source: Syndax Pharmaceuticals, Inc.