Syndax Announces FDA Approval of Revuforj® (revumenib) in Adult and Pediatric Patients with Relapsed or Refractory NPM1 Mutated Acute Myeloid Leukemia

October 24, 2025

– First and only therapy FDA approved in both R/R acute myeloid leukemia (AML) with an NPM1 mutation and R/R acute leukemia with a KMT2A translocation –

– Second approved indication for Revuforj in less than one year further solidifies Syndax's leadership in menin inhibition –

– Included in NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for R/R NPM1 mutated AML –

– Syndax to host conference call today at 2:30 p.m. ET –

NEW YORK, Oct. 24, 2025 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today announced that the U.S. Food and Drug Administration (FDA) has approved Revuforj® (revumenib) for the treatment of relapsed or refractory (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. Revuforj previously received FDA approval in 2024 for the treatment of R/R acute leukemia with a KMT2A translocation in adult and pediatric patients one year and older. Revuforj is the first and only FDA-approved therapy for both R/R AML with an NPM1 mutation and R/R acute leukemia with a KMT2A translocation.

"We are thrilled to have secured a second indication for Revuforj, making it the first and only menin inhibitor that is FDA-approved for multiple acute leukemia subtypes in both adults and children. The breadth of the indicated patient population highlights the compelling and consistent efficacy and tolerability of Revuforj in multiple different types of patients," said Michael A. Metzger, Chief Executive Officer. "Our launch into this second population will greatly benefit from physicians' already strong familiarity with Revuforj and positive experience treating well over 1,000 patients in clinical trials and nearly one year of commercial use."

Mr. Metzger continued, "I would like to thank everyone who made this approval possible, especially the patients and clinicians who participated in our trial and our dedicated Syndax team. We will continue to innovate for patients with menin-dependent acute leukemias and look forward to leading the development of this exciting new therapeutic class into the frontline."

The expansion of the Revuforj label is based on efficacy data from patients with R/R NPM1 mutated AML in the Phase 2 portion of the pivotal AUGMENT-101 trial. The rate of complete remission (CR) plus CR with partial hematological recovery (CRh) was 23% (15/65 pts; 95% CI: 14%, 35%). The median time to CR or CRh response was 2.8 months and the median duration of CR or CRh was 4.5 months. Results from the AUGMENT-101 trial were published in the journal *Blood* and presented at the 2025 European Hematology Association (EHA) Annual Congress Meeting.[1-2]

"The expanded FDA approval of Revuforj marks a major advancement in the management of acute leukemia patients. For the first time, a targeted, oral therapy that is well tolerated and efficacious is approved for R/R NPM1 mutated AML and R/R KMT2A translocated acute leukemia," said Joshua F. Zeidner, M.D., Chief, Leukemia Research at the University of North Carolina, Lineberger Comprehensive Cancer Center. "The compelling clinical activity observed with Revuforj in clinical trials and clinical practice paves the way for a new standard of care for these two aggressive and difficult-to-treat blood cancers."

The safety evaluation of Revuforj was based on the FDA's analysis of 241 patients (207 adult and 34 pediatric patients) with R/R acute leukemia with an NPM1 mutation or a KMT2A translocation who were treated with Revuforj in clinical trials. The most common adverse reactions are consistent with the known safety profile of Revuforj.

"New treatment options are vitally needed for patients with NPM1 mutated AML whose disease has returned or not improved after previous treatment," said Lore Gruenbaum, Ph.D., Chief Scientific Officer of Blood Cancer United® (formerly The Leukemia & Lymphoma Society). "The FDA approval of a precision treatment that selectively targets the pathway driving this form of AML offers new hope to patients and their loved ones."

On September 18, 2025, revumenib was added to the National Comprehensive Cancer Network® (NCCN®) Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for AML as a category 2A recommended treatment option for R/R NPM1m AML based on published data from the AUGMENT-101 trial.[3] Revumenib is also included in the NCCN Guidelines for AML and acute lymphoblastic leukemia (ALL) as a category 2A recommended treatment option for R/R acute leukemia with a KMT2A rearrangement.

AML is a cancer of the bone marrow and blood marked by rapid disease progression. Mutations in the NPM1 gene are the most common genetic alteration observed in AML, occurring in approximately 30% of adults with AML. Mutations in this gene play a critical role in the development of NPM1 mutated AML, an aggressive blood cancer associated with high rates of relapse. Outcomes are poor for patients with NPM1 mutated AML who relapse or are refractory to treatment.

Revuforj is available for order in the United States through Syndax's existing network of specialty distributors and specialty pharmacies. Syndax is committed to supporting patients and removing barriers to access. As part of that commitment, Syndax has established SyndAccess®, a comprehensive program that offers personalized support and resources to U.S. patients who are prescribed Revuforj, including financial assistance for eligible patients. For more information, visit SyndAccess.com or call 1-888-567-SYND (7963), Monday-Friday, 8:00 AM to 8:00 PM Eastern Time (ET).

Conference Call and Webcast

Syndax will host a conference call and webcast to discuss the FDA approval of Revuforj today, October 24, 2025, at 2:30 p.m. ET.

The live webcast 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: SyndaxConf
U.S. and Canada: (800) 590-8290
International: (240) 690-8800
Webcast URL: https://sndx-conf.open-exchange.net

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 NPM1 Mutated (NPM1m) Acute Myeloid Leukemia (AML)

Mutations in the NPM1 gene are the most common genetic alteration observed in AML, occurring in approximately 30% of adults with AML. Mutations in this gene play a critical role in the development of NPM1 mutated (NPM1m) AML, an aggressive blood cancer associated with high rates of relapse. Patients with relapsed or refractory NPM1m AML have a poor prognosis and critical unmet need. Similar to KMT2A-rearranged acute leukemia, NPM1m AML is highly dependent on the menin-KMT2A interaction and disruption of this interaction has been shown to lead to downregulation of certain leukemogenic genes. The diagnosis of NPM1m AML is facilitated by currently available screening techniques, enabling identification of eligible patients for targeted therapies.

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.

References

1. Martha L. Arellano, et al. Menin inhibition with revumenib for NPM1-mutated relapsed or refractory acute myeloid leukemia: the AUGMENT-101 study. Blood 2025. https://doi.org/10.1182/blood.2025028357

2. Martha L. Arellano, et al. 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 at the European Hematology Association (EHA) 2025 Annual Congress Meeting.

3. NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Acute Myeloid Leukemia (Version 1.2026 – September 18, 2025); NCCN makes no warranties of any kind whatsoever regarding their content, use or application and disclaims any responsibility for their application or use in any way.

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Source: Syndax Pharmaceuticals, Inc.