Syndax Announces Publication of SAVE Data on Revuforj® (revumenib) in Combination with Decitabine/Cedazuridine and Venetoclax in Relapsed/Refractory NPM1m, KMT2Ar, and NUP98r AML in the Journal of Clinical Oncology

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– High response rates observed with the all-oral combination in a heavily pretreated population, including 88% (37/42) ORR, 71% (30/42) CRc, and 60% CR/CRh (25/42) –

– Strong activity across subgroups, including 70% (14/20) CR/CRh in venetoclax-naïve and 50% (11/22) CR/CRh in venetoclax-exposed patients –

– Deep responses with 80% (24/30) MRD negativity among evaluable CRc responders –

– Robust transplant rate with 45% (19/42) of patients proceeding to transplant and 63% (12/19) resuming revumenib post-transplant –

– Encouraging durability with median overall survival after transplant not reached –

– Combination was generally well-tolerated –

NEW YORK, June 11, 2026 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today announced that data from the Phase 1/2 SAVE trial of an all-oral regimen of Revuforj® (revumenib), decitabine/cedazuridine, and venetoclax in relapsed or refractory (R/R) NPM1 mutated (NPM1m), KMT2A-rearranged (KMT2Ar), or NUP98-rearranged (NUP98r) acute myeloid leukemia (AML) were published in the Journal of Clinical Oncology and simultaneously presented at the European Hematology Association (EHA) 2026 Congress in Stockholm, Sweden.

Revuforj is the first and only menin inhibitor that is FDA approved for patients one year and older with R/R AML with a susceptible NPM1 mutation who have no satisfactory alternative treatment options or R/R acute leukemia with a KMT2A translocation as determined by an FDA-authorized test.

"The deep and durable remissions observed among patients with R/R NPM1m, KMT2Ar, or NUP98r AML who received an all-oral combination of revumenib, decitabine/cedazuridine, and venetoclax, highlight the potential for revumenib combinations to advance the standard of care treatment for menin-dependent acute leukemias," said Nick Botwood, MBBS, Head of Research & Development and Chief Medical Officer at Syndax. "The SAVE data provide strong support for further studying revumenib with venetoclax and a hypomethylating agent in multiple settings, including among newly diagnosed patients who are unfit for intensive chemotherapy in the ongoing pivotal EVOLVE-2 trial and among fit patients in the RAVEN trial."

"The results observed with the all-oral SAVE regimen in heavily pretreated patients with R/R NPM1m, KMT2Ar, or NUP98r AML are very encouraging," said Ghayas C. Issa, M.D., Associate Professor of Leukemia at The University of Texas MD Anderson Cancer Center and Principal Investigator of the SAVE trial. "Notably, 88% of patients achieved a response with the majority achieving MRD negativity, 45% proceeded to a potentially curative stem cell transplant, and we observed a 14-month median overall survival. We also saw an impressive 50% CR/CRh rate and approximately 12-month median overall survival in patients with prior venetoclax exposure, a population that has historically experienced poor outcomes with a median survival of less than three months."

Summary of Key Results from the SAVE Trial in R/R NPM1m, KMT2Ar, and NUP98r AML

The publication entitled, "All-Oral Combination of Revumenib, Decitabine, and Venetoclax for Relapsed or Refractory AML (SAVE)" reports results from the R/R cohort of patients in the Phase 1/2, single-center, open-label SAVE trial. The primary endpoint of Phase 1 was the recommended Phase 2 dose of revumenib in combination therapy, which was identified as dose level 1 (the FDA-approved monotherapy dose of revumenib). The primary endpoint of Phase 2 was the composite complete remission[1] (CRc) rate.

As of January 2026, 42 patients with R/R AML were enrolled in the SAVE trial, including five adolescents. 38% (16/42) had NPM1m, 40% (17/42) had KMT2Ar, and 21% (9/42) had NUP98r. The median age was 40 years (range: 12-82). Patients were heavily pretreated, with a median of two prior lines of therapy (range: 1-5); 52% (22/42) had received prior venetoclax and 33% (14/42) a prior hematopoietic stem cell transplant (HSCT).

The overall response rate (ORR) was 88% (37/42), the CRc rate was 71% (30/42), and the complete remission plus complete remission with partial hematological recovery (CR/CRh) rate was 60% (25/42) for the entire population. Response rates were similar across genotypes, including patients with NPM1m, KMT2Ar, or NUP98r. Overall, 45% (19/42) of patients proceeded to HSCT following treatment with the regimen, including 38% (6/16) of NPM1m patients, 65% (11/17) of KMT2Ar patients, and 22% (2/9) of NUP98r patients. Of the 19 patients that proceeded to HSCT, 63% (12/19) resumed revumenib after HSCT.

The rates of measurable residual disease (MRD) negativity by flow cytometry were 68% (25/37) among evaluable responders, 80% (24/30) among those with CRc, and 80% (20/25) among those with CR/CRh. Among evaluable patients with CRc, 67% (8/12) were MRD negative by NPM1 NGS (<0.01% threshold), with the vast majority of those patients (7/8) below the limit of detection of the assay ($5x10^{-5}$), highlighting the depth of MRD clearance.

With a median follow-up of 22 months, the median duration of response among patients with CR/CRh was 10.5 months for the entire cohort, 10.7 months among NPM1m patients, not reached among KMT2Ar patients, and 5.9 months among NUP98r patients. The observed 1-year overall survival (OS) rate was 56% for the entire cohort, 63% among NPM1m patients, 47% among KMT2Ar patients, and 67% among NUP98r patients. Median OS after HSCT was not reached. Patients who were MRD negative by flow cytometry had a longer median duration of response (20 months vs. 2.9 months) and OS (not reached vs. 8.4 months) compared to those who were MRD positive.

Notably, clinical activity was observed among patients with prior exposure to venetoclax, a population in whom outcomes are typically poor, with a historical estimated median survival of 2.4 months. In this trial, the CR/CRh rate was 50% (11/22) in patients with venetoclax exposure versus 70% (14/20) in those without. The median OS observed was similar between the two groups (at least 12 months in both groups), based on a Kaplan-Meier estimate. This observation supports a potential biologic synergy between BCL2 inhibition and menin inhibition and the possibility that menin inhibition

may restore sensitivity to BCL2 inhibition after resistance has developed.

Revumenib was generally well-tolerated in combination with decitabine/cedazuridine and venetoclax. The most common treatment-emergent adverse events (TEAEs) included elevations in aspartate aminotransferase or alanine aminotransferase (71%), nausea (52%), and vomiting (48%). The most common Grade ≥3 TEAEs were febrile neutropenia (36%), lung infection (21%), thrombocytopenia (21%), and elevations in aspartate aminotransferase or alanine aminotransferase (21%). Rates of Grade ≥3 differentiation syndrome (5%) and QTc prolongation (5%) were both low.

About Revuforj® (revumenib)

Revuforj (revumenib) is the first and only menin inhibitor that is FDA approved for the treatment of adult and pediatric patients one year and older with relapsed or refractory (R/R) acute myeloid leukemia (AML) with a susceptible NPM1 mutation who have no satisfactory alternative treatment options or R/R acute leukemia with a KMT2A translocation as determined by an FDA-authorized test.

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.

Revuforj (revumenib)

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. Composite complete remission (CRc) includes CR, CRh, CRp, and CRi. Overall response rate (ORR) includes CR, CRh, CRp, CRi, MLFS, and PR.
   CR = Complete remission
   CRh = Complete remission with partial hematologic recovery
   CRp = Complete remission with incomplete platelet recovery
   CRi = Complete remission with incomplete count recovery
   MLFS = Morphologic leukemia-free state
   PR = Partial response

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