



Syndax's Revuforj® (revumenib) Wins Prestigious 2026 Prix Galien USA Award for Best Product for Rare/Orphan Diseases

October 30, 2026

- Revuforj is the first and only menin inhibitor that is approved for adults and children one year and older with R/R NPM1m AML and KMT2A translocated acute leukemia -

NEW YORK, Oct. 30, 2026 (GLOBE NEWSWIRE) -- Syndax Pharmaceuticals (Nasdaq: SNDX), a commercial-stage biopharmaceutical company advancing innovative cancer therapies, today announced that Revuforj® (revumenib) has been recognized as the Best Product for Rare/Orphan Diseases of 2026 by The Galien Foundation. The Galien Foundation fosters, recognizes, and rewards excellence in scientific innovation to improve human health.

Revuforj is the first and only menin inhibitor that is approved for adults and children one year and older with multiple types of relapsed or refractory (R/R) acute leukemia. In November 2024, Revuforj was FDA [approved](#) for the treatment of R/R acute leukemia with a KMT2A translocation. In October 2025, the FDA approval was [expanded](#) to include patients with R/R acute myeloid leukemia (AML) with a susceptible NPM1 mutation who have no satisfactory alternative treatment options.

"We are honored to receive this prestigious award from The Galien Foundation that recognizes the groundbreaking science behind Revuforj and its transformational impact on the treatment paradigm for relapsed or refractory KMT2A-translocated and NPM1-mutated acute leukemias," said Peter Ordentlich, Ph.D., Chief Scientific Officer and Founder of Syndax. "We share this recognition with the many patients, scientists, clinicians, and Syndax team members whose tireless dedication made it possible for us to bring a new type of targeted treatment to adults and children battling some of the most aggressive forms of acute leukemia."

The Galien Foundation's Prix Galien Award is recognized as one of the world's most prestigious awards for biomedical research and innovation. The Prix Galien is widely regarded as the equivalent of the Nobel Prize in biopharmaceutical research.

About The Galien Foundation

The Galien Foundation fosters, recognizes, and rewards excellence in scientific innovation to improve human health. Its vision is to serve as a catalyst for the development of the next generation of treatments and technologies that will transform medical practice and save lives. Established in Paris in 1970 by Roland Mehl in honor of Galen, the father of medical science and modern pharmacology, the Prix Galien is widely regarded as the equivalent of the Nobel Prize in biopharmaceutical research. For more information, visit www.galienfoundation.org.

About NPM1-Mutated 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.

About KMT2A-Rearranged Acute Leukemia

Rearrangements of the KMT2A gene (KMT2Ar) give rise to an aggressive form of acute leukemia associated with a very poor prognosis and high relapse rates. It is estimated that more than 95% of patients with KMT2Ar acute leukemia have a KMT2A translocation, a type of rearrangement that occurs when part of one chromosome breaks and fuses to a different chromosome. KMT2A translocations are found in about 10% of acute leukemias and are especially prevalent among infants and children with acute leukemia.

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 NPM1-mutated or KMT2A-rearranged AML.

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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