# Kura Oncology and Kyowa Kirin Announce FDA Approval of KOMZIFTI™ (ziftomenib), the First and Only Once-Daily Targeted Therapy for Adults with Relapsed or Refractory NPM1-Mutated Acute Myeloid Leukemia

November 13, 2025

– *NPM1* mutations, one of the most common genetic drivers of AML, are now actionable for patients –

– Acute unmet need in R/R *NPM1*-mutated AML defined by historically poor outcomes and low survival rates at relapse –

– FDA grants full approval of KOMZIFTI ahead of PDUFA target action date –

– Approval is based on the KOMET-001 trial, in which KOMZIFTI demonstrated deep responses, a potentially best-in-class safety profile, once-daily administration, and ease of co-administration with common supportive medications in adult patients with R/R *NPM1*-mutated AML –

– KOMZIFTI approval granted with no Boxed Warning related to QTc prolongation or Torsades de Pointes –

– Kura Oncology will host a conference call on November 13, 2025, at 12:30 pm ET / 9:30 am PT –

SAN DIEGO and TOYKO, Nov. 13, 2025 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA) and Kyowa Kirin Co., Ltd. (TSE: 4151) today announced the U.S. Food and Drug Administration (FDA) has granted full approval of KOMZIFTI™ (ziftomenib) for adult patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) with a susceptible *NPM1* mutation who have no satisfactory alternative treatment options. KOMZIFTI is the first and only once-daily, oral menin inhibitor approved for R/R *NPM1*-mutated (*NPM1*-m) AML, a devastating blood cancer with limited treatment options.

“KOMZIFTI combines compelling efficacy, a favorable safety profile, compatibility with concomitant medications, and convenient once-daily oral administration in a population with few effective treatment options. These features highlight KOMZIFTI’s potential to serve as the menin inhibitor of choice in its approved indication,” said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. “Together with our partner, Kyowa Kirin, we remain committed to advancing development of KOMZIFTI across the treatment continuum for AML, where its best-in-class profile offers potential for even greater impact in combination regimens and earlier lines of therapy. We are fully prepared to launch KOMZIFTI today and deliver this new medicine to patients in need.”


*NPM1* mutations are among the most common founder mutations in AML, occurring in approximately 30% of cases. Historically, approximately 20% of patients with *NPM1*-m AML do not respond to front-line therapy. Of those who do respond, 70% will relapse within 3 years, most within 12 months. Early relapse and declining survival with each recurrence underscore the urgent need for treatment approaches that deliver lasting remission.

Approval is supported by the pivotal KOMET-001 trial (NCT04067336), which evaluated KOMZIFTI's safety and efficacy in 112 R/R *NPM1*-m AML patients. The rate of complete remission (CR) plus CR with partial hematologic recovery (CRh) was 21.4% (95% CI: 14.2, 30.2). The median duration of CR+CRh was 5.0 months (95% CI: 1.9, 8.1) and the median time to first response in patients who achieved a CR or CRh was 2.7 months (range: 0.9 to 15 months). 88% of patients who achieved CR or CRh did so within 6 months of initiating KOMZIFTI. These data from the Prescribing Information are generally consistent with findings recently published in the *Journal of Clinical Oncology.*[1]

"KOMZIFTI addresses a critical need for adult patients with R/R *NPM1*-m AML, many of whom are older and unable to tolerate intensive chemotherapy or transplant," said Eunice Wang, M.D., Chief of the Leukemia Service and Professor of Oncology at Roswell Park Comprehensive Cancer Center. "The clinical data demonstrate deep and durable responses with a manageable safety profile, including no drug-drug interactions and no Boxed Warnings for QTc prolongation or Torsades de Pointes – key advantages for patients on multiple concurrent medications. This approval equips physicians with a new oral therapy to integrate into care and improve outcomes for this vulnerable patient population."

The most common adverse reactions (≥20%), including laboratory abnormalities, were aspartate aminotransferase increased, infection without an identified pathogen, potassium decreased, albumin decreased, alanine aminotransferase increased, sodium decreased, creatinine increased, alkaline

phosphatase increased, hemorrhage, diarrhea, nausea, fatigue, edema, bacterial infection, musculoskeletal pain, bilirubin increased, potassium increased, differentiation syndrome, pruritus, febrile neutropenia, and transaminases increased. KOMZIFTI includes a Boxed Warning for differentiation syndrome, a well-studied mechanism-based risk in drugs that restore differentiation. Absence of clinically meaningful drug-drug interactions can ease the use of KOMZIFTI with concomitant therapies, including those that cause QTc interval prolongation. QTc interval prolongation was ≤ Grade 3 in 12% of patients and no Grade 4 or Grade 5 QTc interval prolongation was reported. QTc interval prolongation of any cause occurred in 10% of the 70 patients 65 years of age or older.

"The approval of KOMZIFTI underscores our commitment to advancing precision medicines to address the genetic drivers of disease in hematology and oncology," said Takeyoshi Yamashita, Ph.D., Executive Vice President and Chief Medical Officer of Kyowa Kirin. "In AML, where many patients face severe disease progression and limited treatment options, the evolution toward targeted therapies such as KOMZIFTI represents a major step forward and offers potential to transform existing standards of care. We are proud to partner with Kura Oncology in bringing this important therapy to patients and their families."

In November 2024, Kura Oncology and Kyowa Kirin entered into a global strategic collaboration to develop and commercialize KOMZIFTI. The collaboration builds on Kyowa Kirin's leadership and expertise in hematologic malignancies. Under the terms of the collaboration, Kura leads development, regulatory and commercial strategy in the U.S. and is responsible for manufacturing KOMZIFTI. The companies will jointly perform certain commercialization activities in accordance with a co-created U.S. territory commercialization plan. Outside the U.S., Kyowa Kirin leads development, regulatory and commercial strategy and is responsible for commercializing KOMZIFTI.

As part of its commitment to helping patients access KOMZIFTI, Kura has established a support program, Kura RxKonnect ™, to minimize barriers to access and reimbursement for appropriate patients prescribed KOMZIFTI. Kura RxKonnect is available 8:00 am – 8:00 pm ET by phone at (844) KuraPSP (844-587-2777) or online at www.KuraRxKonnect.com.

**Conference Call and Webcast**
Kura will host a conference call and webcast featuring Company management and guest speaker Eunice Wang, M.D., Chief of the Leukemia Service and Professor of Oncology at Roswell Park Comprehensive Cancer Center, at 12:30 pm ET / 9:30 am PT on November 13, 2025. The live webcast and replay will be available on the Company's website at www.kuraoncology.com under the Investors tab in the Events and Presentations section.

**About KOMZIFTI™**
KOMZIFTI (ziftomenib) is an oral menin inhibitor approved for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (AML) with a susceptible *NPM1* mutation who have no satisfactory alternative treatment options.

KOMZIFTI is in development for the front-line treatment of AML harboring *NPM1* mutations, *KMT2A* translocations and *FLT3* mutations, with the potential to be combined with approved therapies and benefit a broad spectrum of patients earlier in their disease course.

**IMPORTANT SAFETY INFORMATION FOR KOMZIFTI FROM THE U.S. PRESCRIBING INFORMATION**

**Boxed WARNING: DIFFERENTIATION SYNDROME**

**Differentiation syndrome, which can be fatal, has occurred with KOMZIFTI. Signs and symptoms may include fever, joint pain, hypotension, hypoxia, dyspnea, rapid weight gain or peripheral edema, pleural or pericardial effusions, pulmonary infiltrates, acute kidney injury, and rashes. If differentiation syndrome is suspected, interrupt KOMZIFTI, and initiate oral or intravenous corticosteroids with hemodynamic and laboratory monitoring until symptom resolution; resume KOMZIFTI upon symptom improvement.**

**WARNINGS AND PRECAUTIONS**

**Differentiation Syndrome**

KOMZIFTI can cause fatal or life-threatening differentiation syndrome (DS). DS is associated with rapid proliferation and differentiation of myeloid cells. Symptoms of DS, including those seen in patients treated with KOMZIFTI, may include fever, hypoxia, joint pain, hypotension, dyspnea, rapid weight gain or peripheral edema, pleural or pericardial effusions, acute kidney injury, and rashes.

In the clinical trial, DS occurred in 29 (26%) of 112 patients with R/R AML with an *NPM1* mutation who were treated with KOMZIFTI at the recommended dosage. DS was Grade 3 in 13% and fatal in two patients. In broader evaluation of all patients with any genetic form of AML treated with KOMZIFTI monotherapy in clinical trials, DS occurred in 25% of patients. Four fatal cases of DS occurred out of 39 patients with *KMT2A*-rearranged AML treated with KOMZIFTI. KOMZIFTI is not approved for use in patients with *KMT2A*-rearranged AML.

In the 112 patients with an *NPM1* mutation, DS was observed with and without concomitant hyperleukocytosis, in as early as 3 days and up to 46 days after KOMZIFTI initiation. The median time to onset was 15 days. Two patients experienced more than one DS event. Treatment was interrupted and resumed in 15 (13%) patients, while it was permanently discontinued in 2 (2%) patients.

Prior to starting treatment with KOMZIFTI, reduce the WBC counts to less than 25 x $10^9$/L. If DS is suspected, interrupt KOMZIFTI, initiate oral or intravenous corticosteroids (e.g., dexamethasone 10 mg every 12 hours) for a minimum of 3 days with hemodynamic and laboratory monitoring. Resume treatment with KOMZIFTI at the same dose level when signs and symptoms improve and are Grade 2 or lower. Taper corticosteroids over a minimum of 3 days after adequate control or resolution of symptoms. Symptoms of DS may recur with premature discontinuation of corticosteroid treatment.

**QTc Interval Prolongation**

KOMZIFTI can cause QTc interval prolongation. In the clinical trial, QTc interval prolongation was reported as an adverse reaction in 12% of 112 patients treated with KOMZIFTI at the recommended dosage for R/R AML with an *NPM1* mutation. QTc interval prolongation was Grade 3 in 8% of patients. The heart-rate corrected QT interval (using Fridericia's method) (QTcF) was greater than 500 msec in 9% of patients, and the increase from baseline QTcF was greater than 60 msec in 12% of patients. KOMZIFTI dose reduction was required for 1% of patients due to QTc interval prolongation. QTc prolongation occurred in 14% of the 42 patients less than 65 years of age and in 10% of the 70 patients 65 years of age or older.

Correct electrolyte abnormalities, including hypokalemia and hypomagnesemia, prior to treatment with KOMZIFTI. Perform an ECG prior to initiation of

treatment with KOMZIFTI, and do not initiate KOMZIFTI in patients with QTcF > 480 msec. Perform an ECG at least once weekly for the first four weeks on treatment, and at least monthly thereafter. Interrupt KOMZIFTI if the QTc interval is > 500 ms or the change from baseline is > 60 ms (Grade 3). 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 of KOMZIFTI with drugs known to prolong the QTc interval may increase the risk of QTc interval prolongation, result in a greater increase in the QTc interval and adverse reactions associated with QTc interval prolongation, including Torsades de Pointes, other serious arrhythmias, and sudden death.

**Embryo-Fetal Toxicity**

Based on findings in animals and its mechanism of action, KOMZIFTI can cause embryo-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 KOMZIFTI and for 6 months after the last dose. Advise males with female partners of reproductive potential to use effective contraception during treatment with KOMZIFTI and for 3 months after the last dose.

**ADVERSE REACTIONS**

Fatal adverse reactions occurred in 4 (4%) patients who received KOMZIFTI, including 2 with differentiation syndrome, 1 with infection, and 1 with sudden death. Serious adverse reactions were reported in 79% of patients who received KOMZIFTI. Serious adverse reactions occurring in ≥ 5% of patients included infection without an identified pathogen (29%), febrile neutropenia (18%), bacterial infection (16%), differentiation syndrome (16%), and dyspnea (6%).

Dosage interruption of KOMZIFTI due to an adverse reaction occurred in 54% of patients. Adverse reactions that required dose interruption in ≥ 2% of patients included infection without an identified pathogen (15%), differentiation syndrome (13%), febrile neutropenia (5%), pyrexia (4%), electrocardiogram QT prolonged (4%), leukocytosis (4%), bacterial infection (3%), cardiac failure (2%), cholecystitis (2%), diarrhea (2%), pruritus (2%), and thrombosis (2%). Dose reduction of KOMZIFTI due to an adverse reaction occurred in 4% of patients. Permanent discontinuation of KOMZIFTI due to an adverse reaction occurred in 21% of patients. Adverse reactions that required permanent discontinuation of KOMZIFTI in ≥ 2% of patients were infection without an identified pathogen (8%), bacterial infection (4%), cardiac arrest (2%), and differentiation syndrome (2%).

**Most common (≥ 20%) adverse reactions, including laboratory abnormalities**, were aspartate aminotransferase increased (53%), infection without an identified pathogen (52%), potassium decreased (52%), albumin decreased (51%), alanine aminotransferase increased (50%), sodium decreased (49%), creatinine increased (45%), alkaline phosphatase increased (41%), hemorrhage (38%), diarrhea (36%), nausea (35%), fatigue (34%), edema (30%), bacterial infection (28%), musculoskeletal pain (28%), bilirubin increased (27%), potassium increased (26%), differentiation syndrome (26%), pruritus (23%), febrile neutropenia (22%), and transaminases increased (21%).

**DRUG INTERACTIONS**

Drug interactions may occur when KOMZIFTI is concomitantly used with:

- Strong or Moderate CYP3A4 Inhibitors: Monitor patients more frequently for KOMZIFTI-associated adverse reactions.
- Strong or Moderate CYP3A4 Inducers: Avoid concomitant use of KOMZIFTI.
- Gastric Acid Reducing Agents: Avoid concomitant use of KOMZIFTI with proton pump inhibitors (PPIs), H2 receptor antagonists (H2RAs), or locally acting antacids. If concomitant use with H2RAs or locally acting antacids cannot be avoided, modify KOMZIFTI administration time.
    - Take KOMZIFTI 2 hours before or 10 hours after administration of an H2 receptor antagonist.
    - Take KOMZIFTI 2 hours before or 2 hours after administration of a locally acting antacid.
- Drugs that Prolong the QTc Interval: Avoid concomitant use of KOMZIFTI. If concomitant use cannot be avoided, obtain ECGs when initiating, during concomitant use, and as clinically indicated. Interrupt KOMZIFTI if the QTc interval is > 500 ms or the change from baseline is > 60 ms.

**USE IN SPECIFIC POPULATIONS**

**Pregnancy:** Based on findings in animals and its mechanism of action, KOMZIFTI can cause embryo-fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Verify pregnancy status in females of reproductive potential prior to starting KOMZIFTI.

**Lactation:** Because of the potential for adverse reactions in the breastfed child, advise women not to breastfeed during treatment with KOMZIFTI and for 2 weeks after the last dose.

**Infertility:** Based on findings in animals, KOMZIFTI may impair fertility in females and males of reproductive potential.

Please see full Prescribing Information, including **Boxed WARNING**.

*NPM1,* nucleophosmin 1, *KMT2A*, lysine methyltransferase 2A, *FLT3,* Fms-like tyrosine kinase 3

1. Wang *et al.* 2025 *J Clin Oncol*: JCO2501694, online ahead of print. Reprint available here.

**About Kura Oncology**
Kura Oncology is a biopharmaceutical company committed to realizing the promise of precision medicines for the treatment of cancer. Kura's pipeline of small molecule drug candidates is designed to target cancer signaling pathways and address high-need hematologic malignancies and solid tumors. Kura developed and is commercializing KOMZIFTI, the FDA-approved once-daily, oral menin inhibitor for the treatment of adults with relapsed or refractory *NPM1*-mutated acute myeloid leukemia, and continues to pioneer advancements in farnesyl transferase inhibition. For additional information, please visit the Kura website at https://kuraoncology.com/ and follow us on X and LinkedIn.

**About Kyowa Kirin**
Kyowa Kirin aims to discover and deliver novel medicines and treatments with life-changing value. As a Japan-based Global Specialty Pharmaceutical

Company, Kyowa Kirin has invested in drug discovery and biotechnology innovation for more than 70 years and is currently working to engineer the next generation of antibodies and cell and gene therapies with the potential to help patients with high unmet medical needs, such as bone & mineral, intractable hematological diseases/hemato-oncology and rare diseases. A shared commitment to Kyowa Kirin's values, to sustainable growth, and to making people smile unites Kyowa Kirin across the globe. You can learn more about the business of Kyowa Kirin at www.kyowakirin.com.

**Kura Forward-Looking Statements**

This news release contains certain forward-looking statements that involve risks and uncertainties that could cause actual results to be materially different from historical results or from any future results expressed or implied by such forward-looking statements. Such forward-looking statements include statements regarding, among other things, the potential of KOMZIFTI to have a best-in-class, favorable and differentiated safety profile; the potential of KOMZIFTI to offer a best-in-class benefit-risk profile for patients with R/R *NPM1*-m AML and to be the menin inhibitor of choice in its approved indication; the potential of KOMZIFTI to have a greater impact in combination regimens and earlier lines of therapy than as a monotherapy in the R/R setting; the potential of KOMZIFTI to clinically benefit and improve outcomes for patients with R/R *NPM1*-m AML; Kura's ability to launch KOMZIFTI; Kura's and Kyowa Kirin's plans to commercialize KOMZIFTI in the U.S. and to continue to develop KOMZIFTI across the AML treatment continuum; potential benefits to patients of the absence of drug-drug interactions and that the approval of KOMZIFTI did not require Boxed Warnings for QTc prolongation and Torsades de Pointes; and the potential of KOMZIFTI to be combined with approved therapies and benefit a broad spectrum of patients earlier in their disease course. Factors that may cause actual results to differ materially include the risk that KOMZIFTI may have unintended side effects; risks associated with market competition, market acceptance and commercialization of KOMZIFTI; the risk that the collaboration with Kyowa Kirin is unsuccessful; and other risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. You are urged to consider statements that include the words "may," "will," "would," "could," "should," "believes," "estimates," "projects," "promise," "potential," "expects," "plans," "anticipates," "intends," "continues," "designed," "goal," or the negative of those words or other comparable words to be uncertain and forward-looking. For a further list and description of the risks and uncertainties Kura faces, please refer to Kura's periodic and other filings with the Securities and Exchange Commission, which are available at www.sec.gov. Such forward-looking statements are current only as of the date they are made, and Kura assumes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/d9984035-330f-4cdb-ac17-aa7739fdadeb

Source: Kura Oncology, Inc.

**KOMZIFTI Packaging**


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