



Kura Oncology and Kyowa Kirin Announce Publication of Pivotal Ziftomenib Data in Relapsed/Refractory *NPM1*-Mutated AML in the Journal of Clinical Oncology

September 25, 2025

- KOMET-001 in R/R *NPM1*-m AML met its primary endpoint with ziftomenib monotherapy demonstrating significant clinical benefit and deep responses –
- Consistent activity across pre-specified subgroups, regardless of prior HSCT, prior venetoclax, lines of therapy, or *FLT3/IDH* co-mutations –
- Favorable safety and tolerability profile, including a lack of clinically significant QTc prolongation or myelosuppression –
- Prescription Drug User Fee Act (PDUFA) target action date for FDA Priority Review of ziftomenib in adults with R/R *NPM1*-m AML set for November 30, 2025 –

SAN DIEGO and TOKYO, Sept. 25, 2025 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA) and Kyowa Kirin Co., Ltd. (TSE: 4151, "Kyowa Kirin") today announced the *Journal of Clinical Oncology* published the full results from the pivotal KOMET-001 clinical trial ([NCT04067336](#)) evaluating ziftomenib, an investigational, once-daily, oral menin inhibitor, in adult patients with relapsed/refractory (R/R) *NPM1*-mutated (*NPM1*-m) acute myeloid leukemia (AML). Although newly diagnosed patients with *NPM1*-m AML have high response rates to approved standard of care, relapses are common and survival outcomes are poor. There is currently no approved therapy to specifically target *NPM1*-m AML. Ziftomenib is currently under priority review by the Food and Drug Administration (FDA) for treatment of R/R *NPM1*-m AML.

"Relapsed or refractory *NPM1*-mutated AML remains very challenging to treat, particularly after venetoclax-based therapy or transplant," said Eunice Wang, M.D., Chief of Leukemia Service, Professor of Oncology, Roswell Park Comprehensive Cancer Center, Buffalo, NY. "The manuscript describes deep responses, signals of clinical activity across relevant subgroups and a generally manageable tolerability profile, which is important in treatment of late line AML patients where accumulated toxicity can limit treatment options. The benefit-risk profile of ziftomenib is highly encouraging and, if replicated in additional treatment settings, has potential to be transformative for a large population of patients with menin pathway-driven AML."

"Publication in ASCO's *Journal of Clinical Oncology* is an important advancement for adult patients with *NPM1*-m AML," said Mollie Leoni, M.D., Chief Medical Officer of Kura Oncology. "In addition to evidence of monotherapy activity, the safety and tolerability profile of ziftomenib from this trial is encouraging, marked by the absence of clinically meaningful QTc prolongation as well as low rates of both myelosuppression and treatment discontinuation. No clinically meaningful drug-drug interactions were observed, including with commonly used supportive-care medications, which may simplify co-administration in a polypharmacy setting. We continue to conduct studies in earlier line settings and in combination with multiple therapeutic agents in close collaboration with investigators, study teams and our partner Kyowa Kirin."

Summary of the published data

The publication, entitled "Ziftomenib in Relapsed or Refractory *NPM1*-Mutated AML", includes positive data from 92 adult patients with R/R *NPM1*-m AML in the phase 2 portion of the clinical trial as of the primary data cutoff date of October 28, 2024.

The KOMET-001 phase 2 trial met its primary endpoint with a complete remission with full or partial hematologic recovery (CR/CRh) rate of 22% (95% CI, 14 to 32; P=0.0058), which was significantly higher than the 12% historical standard-of-care response rate for patients with R/R *NPM1*-m AML. One additional response of CRh occurred after the primary analysis data cutoff resulting in a cumulative CR/CRh rate of 23% (95% CI, 15 to 33). 61% of evaluable CR/CRh responders were negative for measurable residual disease (MRD). Overall response rate (ORR) was 33% (95% CI, 23 to 43), with a median duration of overall response of 4.6 months (95% CI, 2.8 to 7.4).

Median overall survival (OS) was 6.6 months (95% CI, 3.6 to 8.6). Among ORR responders, median OS was 18.4 months (95% CI, 8.6 to not estimable) vs. 3.5 months (95% CI, 2.7 to 4.2) among non-responders. Two responders received subsequent allogeneic stem cell transplant and both resumed ziftomenib maintenance after transplant. At the time of data cutoff, nine patients (two after transplantation) remained on ziftomenib treatment. Prespecified subgroup analyses showed comparable CR/CRh rates regardless of lines of therapy, prior venetoclax exposure, or presence of co-mutations, including *FLT3m* or *IDH1/2m*.

Ziftomenib was well tolerated with a safety profile consistent with previously disclosed data. The most common grade ≥ 3 treatment-emergent adverse events were febrile neutropenia (26%), anemia (20%), and thrombocytopenia (20%). Differentiation syndrome occurred in 25% of patients (15% grade 3; no grade 4-5) and was manageable with protocol-defined mitigation. Three patients (3%) discontinued treatment because of ziftomenib-related adverse events.

These findings formed part of the data set used for the New Drug Application for ziftomenib as a potential treatment for patients with R/R *NPM1*-m AML. The FDA target action date is November 30, 2025. There is currently no FDA-approved treatment for patients with R/R *NPM1*-m AML.

"The publication of the investigational ziftomenib data adds important scientific context for clinicians and patients," said Takeyoshi Yamashita, Ph. D., Executive Vice President and Chief Medical Officer, Kyowa Kirin. "Together with Kura Oncology, we are committed to rigorous, globally coordinated evidence generation to support the benefit-risk profile of menin inhibition across the treatment landscape. Our shared goal is to advance development rapidly and generate the evidence needed to deliver ziftomenib to appropriate patients in need."

The publication is now available on the [Journal of Clinical Oncology](#) website and in the [Scientific Manuscripts](#) section on Kura's website.

Ziftomenib is currently under clinical development, and its safety and efficacy have not been evaluated by any regulatory authority.

About Kura Oncology

Kura Oncology is a clinical-stage biopharmaceutical company committed to realizing the promise of precision medicines for the treatment of cancer. The Company's pipeline of small molecule drug candidates is designed to target cancer signaling pathways and address high-need hematologic malignancies and solid tumors. Kura is developing ziftomenib, a menin inhibitor targeting certain genetic drivers of acute myeloid leukemias, and continues to pioneer advancements in both menin inhibition and farnesyl transferase inhibition to address mechanisms of adaptive and innate resistance in the treatment of solid tumors. 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, among other things, statements regarding the efficacy, safety and therapeutic potential of ziftomenib. Factors that may cause actual results to differ materially include the risk that compounds that appeared promising in early research or clinical trials do not demonstrate safety and/or efficacy in later preclinical studies or clinical trials, the risk that Kura may not obtain approval to market its product candidates, uncertainties associated with performing clinical trials, regulatory filings, and other interactions with regulatory bodies, 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.

Kura Contact

Investors and Media:

Greg Mann

858-987-4046

gmann@kuraoncology.com

Kyowa Kirin Contacts

Investors:

Ryohei Kawai

ir@kyowakirin.com

Media, Global:

Nobuyuki Manita

media@kyowakirin.com



Source: Kura Oncology, Inc.