



## **Kura Oncology and Kyowa Kirin Announce Positive Ziftomenib Monotherapy Registrational Trial and Positive FDA Feedback for Upcoming Frontline Combination Trial Designs**

February 5, 2025

- KOMET-001 registrational trial in R/R NPM1-m AML achieved primary CR/CRh endpoint; topline data submitted for presentation at an upcoming medical meeting –
  - NDA submission for ziftomenib on track for 2Q 2025 –
- Registrational KOMET-017-IC trial of intensive chemotherapy combination will assess MRD negative CR and EFS as dual-primary endpoints to support potential U.S. accelerated approval and full approval –
- Registrational KOMET-017-NIC trial of venetoclax / azacitidine combination will assess CR and OS as dual-primary endpoints to support potential U.S. accelerated approval and full approval –
  - KOMET-017-IC and NIC Phase 3 trials expected to initiate in 2H 2025 –
  - Multiple clinical data presentations for ziftomenib and pipeline programs anticipated throughout 2025 –
  - Kura management to host virtual investor event today at 4:30 p.m. ET –

SAN DIEGO, Feb. 05, 2025 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA, "Kura") and Kyowa Kirin Co., Ltd. (TSE: 4151, "Kyowa Kirin") today announced positive topline results from KOMET-001, the Phase 2 registration-directed trial of ziftomenib, a highly selective, once-daily, oral investigational menin inhibitor, in patients with relapsed/refractory (R/R) NPM1-mutant (NPM1-m) acute myeloid leukemia (AML). Topline data for KOMET-001 has been submitted for presentation at an upcoming medical conference in the second quarter of 2025, and Kura is on track to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for ziftomenib in the second quarter of 2025.

The companies, which announced their joint collaboration to commercialize ziftomenib in 2024, also announced they plan to initiate a single protocol containing two independently powered, randomized, double-blind, placebo-controlled, registrational Phase 3 trials to evaluate ziftomenib in combination with both intensive and non-intensive combination regimens in patients with newly diagnosed NPM1-m and KMT2A-rearranged (KMT2A-r) AML, following successful interactions with the FDA. Each frontline trial design includes dual-primary endpoints to support potential U.S. accelerated approval and full approval. The companies plan to initiate the two frontline Phase 3 trials in the second half of 2025 and anticipate multiple clinical data presentations for the ziftomenib AML program as well as Kura's pipeline programs in 2025.

"We are excited to report positive topline results in R/R NPM1-m AML patients, underscoring the strong foundation for our ziftomenib program to potentially transform the treatment landscape for these patients. We appreciate the commitment and dedication from our team as well as our partners at Kyowa Kirin," said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. "We believe this achievement for our KOMET-001 trial positions Kura and Kyowa Kirin to deliver on its path to commercialization of ziftomenib, beginning with our potential first NDA submission in R/R NPM1-m AML next quarter. Furthermore, we believe the positive FDA interactions for the KOMET-017 protocol, including the opportunity for accelerated approval in both trials, pave the way for us to position ziftomenib as a potential frontline therapy to address up to 50% of patients with AML."

### **Positive KOMET-001 Ziftomenib Monotherapy Trial in R/R NPM1-m AML**

Kura and Kyowa Kirin today announced positive topline results from KOMET-001, a Phase 2 registration-directed trial of ziftomenib, in patients with R/R NPM1-m AML. The KOMET-001 trial achieved its primary endpoint of CR plus CR with partial hematological recovery (CRh) and the primary endpoint was statistically significant. The benefit-risk profile for ziftomenib is highly encouraging, and safety and tolerability were consistent with previous reports.

The KOMET-001 registration-directed trial is designed to assess evidence of clinical activity, safety and tolerability of ziftomenib, the only investigational therapy to receive Breakthrough Therapy Designation (BTD) from the FDA for treatment of R/R NPM1-mutant AML. Full results from the KOMET-001 trial will be presented at a future medical meeting in the second quarter of 2025. After successful FDA interactions in part facilitated by BTD, Kura announced that it is on track to submit an NDA to the FDA for ziftomenib for the treatment of patients with R/R NPM1-mutant AML in the second quarter of 2025.

### **Positive FDA Feedback for Upcoming Frontline Combination Trial Designs**

Kura and Kyowa Kirin recently announced plans for KOMET-017, a global protocol evaluating ziftomenib in combination with standards of care for adults with newly diagnosed NPM1-m or KMT2A-r AML. Following successful End-of-Phase 1 meetings with the FDA, the companies announced they will proceed with plans to initiate the KOMET-017 trial, comprising of two independent, global, randomized, double-blind, placebo-controlled Phase 3 trials to evaluate ziftomenib in combination with both intensive and non-intensive combination regimens in patients with newly diagnosed NPM1-m and/or KMT2A-r AML. The positive feedback from the FDA, along with data from the KOMET-007 trial presented at the 2024 American Society of Hematology Annual Meeting, reinforces Kura's and Kyowa Kirin's commitment to evaluating ziftomenib in patients across the continuum of frontline treatment options.

The registrational KOMET-017-IC (Intensive Combination) trial will evaluate the combination of ziftomenib with induction chemotherapy (7+3) in newly

diagnosed NPM1-m and KMT2A-r AML patients. Patients will be randomized to receive ziftomenib or placebo, in combination with standard induction, consolidation chemotherapy and post consolidation maintenance. The KOMET-017-IC trial will assess minimum residual disease (MRD) negative complete response (CR) and event-free survival (EFS) as dual-primary endpoints to support potential U.S. accelerated approval and full approval, respectively and is anticipated to be initiated in the second half of 2025.

The registrational KOMET-017-NIC (Non-Intensive Combination) trial will evaluate the combination of ziftomenib with venetoclax plus azacitidine in newly diagnosed NPM1-m patients unfit to receive intensive chemotherapy. The KOMET-017-NIC trial will assess CR and overall survival (OS) as dual-primary endpoints to support potential U.S. accelerated approval and full approval, respectively. Patients will be randomized to receive ziftomenib or placebo, in combination with venetoclax and azacitidine. The KOMET-017-NIC trial is anticipated to be initiated in the second half of 2025.

“Even with approved therapies, up to 70% of patients who achieve a first CR will see their AML return within 3 years. The 5-year survival rate for AML is 31.9% and as low as 11.2% for patients aged older than 65 years,” said Mollie Leoni, M.D., Chief Medical Officer of Kura Oncology. “Given this urgent need, we are pleased with the outcome of these FDA interactions and look forward to initiating our Phase 3 trials to establish the benefit-risk profile of ziftomenib in both the intensive and non-intensive chemotherapy settings. We were particularly pleased by the FDA’s willingness to allow the trials to use MRD negative CR and CR as primary endpoints for accelerated approval in the two populations. In so doing, the KOMET-017 protocol is breaking new ground, which may help deliver ziftomenib more quickly to patients living with this devastating disease.”

“Starting patients on combination therapy early is essential to improving outcomes in AML,” said Takeyoshi Yamashita, Ph.D., Senior Managing Executive Officer and Chief Medical Officer of Kyowa Kirin. “The data from the completed KOMET-001 trial and FDA feedback on the planned KOMET-017 protocol strengthens our confidence these trials may offer valuable treatment options for patients throughout the continuum of treatment. We remain committed to working with our colleagues at Kura to bring ziftomenib as rapidly as possible to AML patients worldwide.”

MRD is a term describing small numbers of leukemic cells, which are still detectable during or after treatment, even when a patient has achieved CR by standard criteria. Remaining leukemia cells in the body can become active and start to multiply, resulting in a relapse of the disease, which may be fatal for patients. Achieving MRD negativity, which may be associated with longer remissions and improved survival, means that a treatment has reduced the number of leukemic cells to below the limit of detection by the most sensitive analytical methods.

“We carefully designed KOMET-017 to allow patients to go on the same protocol but on one of the two sub-studies based on whether they are fit or unfit for intensive chemotherapy, and this approach is intended to be very patient centric, facilitate rapid enrollment and offer operational advantages to the study sites,” said Amer Zeidan, MBBS, MHS, chief of the Division of Hematologic Malignancies, director of Hematology Early Therapeutics Research at Yale Cancer Center, and the lead investigator of the KOMET-017 trial. “Further, we designed KOMET-017 to allow for a potential accelerated approval based on endpoints that have been widely accepted as surrogates for meaningful clinical benefit in these patient populations,” Dr Zeidan added. “The association between MRD negativity and improved survival in patients with NPM1-mutated AML is well established in the literature. AML experts around the world recommend monitoring MRD in patients to guide treatment decisions. The best opportunity to achieve long-lasting remission and extend survival is to achieve MRD negativity with the first attempt at treatment. Therefore, using MRD negative CR as an approvable endpoint in AML is very innovative and could allow faster availability of therapies to our patients,” Dr Zeidan concluded.

## 2025 Anticipated Clinical Data Highlights

Kura and Kyowa Kirin expect to present multiple clinical data updates from their ziftomenib AML program, and Kura expects to present updates from its KO-2806 and tipifarnib programs, in 2025 as follows:

- Topline data from the KOMET-001 trial of ziftomenib monotherapy in R/R NPM1-m AML (2Q 2025)
- KOMET-007 Phase 1b data of ziftomenib in combination with 7+3 in newly diagnosed NPM1-m AML and KMT2A-r AML (2Q 2025)
- FIT-001 Phase 1 data of KO-2806 monotherapy in HRAS-mutant and KRAS-mutated solid tumors (2H 2025)
- FIT-001 Phase 1 data of KO-2806 in combination with cabozantinib in renal cell carcinoma (RCC) (2H 2025)
- KURRENT-HN Phase 1 data of tipifarnib in combination with alpelisib in PIK3CA-dependent head and neck squamous cell carcinoma (HNSCC) (2H 2025)
- KOMET-007 Phase 1b data of ziftomenib in combination with venetoclax / azacitidine in NPM1-m AML (2H 2025)

## Virtual Investor Event

Kura will host a webinar and conference call featuring company management today at 4:30 p.m. ET. Those who would like to participate may access the live webinar [here](#), or register in advance for the teleconference [here](#). The link to the live webinar will also be available on the [Investors](#) section of Kura’s website, with an archived replay available shortly after the event.

## About AML

AML primarily affects adults and is one of the most difficult-to-treat blood cancers. AML starts in the bone marrow and can quickly move to the blood and other parts of the body including the lymph nodes, spleen and central nervous system. Approximately 20,000 Americans are diagnosed with AML each year, with an NPM1 genetic mutation or KMT2A rearrangement found in approximately 35% of cases. Relapse in AML is common, and despite available treatments, nearly 11,000 Americans will die from the disease each year.

## About Ziftomenib

Ziftomenib is a selective and oral menin inhibitor currently in development for the treatment of genetically defined AML patients with high unmet need. In April 2024, ziftomenib received BTM by the FDA for the treatment of R/R NPM1-mutated AML based on data from Kura’s KOMET-001 clinical trial. Additional information about clinical trials for ziftomenib can be found at [kuraoncology.com/clinical-trials/#ziftomenib](https://kuraoncology.com/clinical-trials/#ziftomenib).

## 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 consists of small molecule drug candidates that target cancer signaling pathways. Ziftomenib, a once-daily, oral menin

inhibitor, is the first and only investigational therapy to receive BTM from the FDA for the treatment of relapsed/refractory (R/R) NPM1-m AML. In November 2024, Kura entered a global strategic collaboration agreement with Kyowa Kirin Co., Ltd. to develop and commercialize ziftomenib for AML and other hematologic malignancies. Enrollment in a Phase 2 registration-directed trial of ziftomenib in R/R NPM1-mutant AML has been completed, and the companies anticipate submission of a New Drug Application in the second quarter of 2025. Kura and Kyowa Kirin are also conducting a series of clinical trials to evaluate ziftomenib in combination with current standards of care in newly diagnosed and R/R NPM1-mutant and KMT2A-rearranged AML. KO-2806, a next-generation farnesyl transferase inhibitor, is being evaluated in a Phase 1 dose-escalation trial as a monotherapy and in combination with targeted therapies. Tipifarnib, a potent and selective FTI, is currently in a Phase 1/2 trial in combination with alpelisib for patients with PIK3CA-dependent head and neck squamous cell carcinoma. For additional information, please visit Kura's 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](http://www.kyowakirin.com).

### **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 therapeutic potential and potential success of ziftomenib, KO-2806 and tipifarnib; plans, trial designs and expected timing of clinical trials; the expected timing and presentation of data from clinical trials; the anticipated timing of submission of a New Drug Application for ziftomenib; the potential for U.S. accelerated approval and full approval of product candidates; and the success and impact of interactions with the FDA. 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, applications and other interactions with regulatory bodies, risks associated with reliance on third parties to successfully conduct clinical trials, the risks associated with reliance on outside financing to meet capital requirements, 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 that Kura faces, please refer to Kura's periodic and other filings with the Securities and Exchange Commission, which are available at [www.sec.gov](http://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.

*Amer Zeidan has consulted for and received honoraria from Kura. Opinions expressed are his own and do not necessarily represent those of his employer.*

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