Kura Oncology and Kyowa Kirin Announce Submission of New Drug Application for Ziftomenib to FDA

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– Submission seeks approval for the treatment of adult patients with relapsed or refractory AML with a NPM1 mutation –

SAN DIEGO and TOKYO, April 08, 2025 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA, "Kura"), and Kyowa Kirin Co., Ltd. (TSE: 4151, "Kyowa Kirin"), today announced Kura submitted a New Drug Application (NDA) for ziftomenib, a highly selective, once-daily, oral, investigational menin inhibitor, for the treatment of adult patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) with a nucleophosmin 1 (NPM 1) mutation to the U.S. Food and Drug Administration (FDA) on March 31, 2025.

Ziftomenib has received Breakthrough Therapy, Fast Track, and Orphan Drug Designations. The FDA has a 60-day filing review period to determine whether the NDA is complete and accepted for review; Kura expects to receive notification from the FDA on this preliminary evaluation in the second quarter of 2025. Priority Review was requested, which, if granted, would provide a target FDA review period of six months after NDA acceptance.

"This NDA submission brings us one step closer to our goal of advancing ziftomenib to market as a new therapeutic option for adult patients with R/R *NPM1*-m AML, a devastating disease for which there are currently no FDA-approved targeted therapy options," said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. "We look forward to working closely with the FDA throughout the review process and are optimistic about the potential of ziftomenib to impact patients with *NPM1*-mutant AML. We extend our gratitude to the team at Kura, our dedicated investigators, study site teams, and most importantly, to the patients who participated in our clinical trials, and their families and caregivers, who all helped make this possible. We appreciate the support and cooperation we enjoy with our partner Kyowa Kirin, and we look forward with confidence to the continued progress of this program and our collaboration."

About *NPM1*-Mutant AML

AML is the most common acute leukemia in adults and begins when the bone marrow makes abnormal myeloblasts (white blood cells), red blood cells, or platelets. Despite the many available treatments for AML, prognosis for patients remains poor and a high unmet need remains. The menin pathway is considered a driver for multiple genetic alterations of the disease, of which *NPM1* mutations are among the most common, representing approximately 30% of AML cases. While patients with *NPM1*-m AML have high response rates to frontline therapy, relapse rates are high and survival outcomes are poor, with only 30% overall survival at 12 months in the R/R setting. Additionally, *NPM1* mutations frequently occur with co-mutations in other disease-associated genes, including *FLT3*, *DNMT3A*, and *IDH1/2*, with prognosis heavily influenced by the presence of such co-occurring mutations. Adult patients with *NPM1*-m AML and select co-mutations and/or R/R disease have a poor prognosis, with median overall survival of only approximately 7.8 months in 2nd line, 5.3 months in 3rd line, and 3.5 months following the 4th line[1]. There are currently no FDA-approved therapies targeting *NPM1*-m AML.

About Ziftomenib

Ziftomenib is a potent and selective, oral, investigational menin inhibitor currently in development for the treatment of genetically defined AML patients with high unmet need. In April 2024, ziftomenib received Breakthrough Therapy Designation (BTD) from the FDA for the treatment of adult patients with R/R AML with a NPM1 mutation based on data from Kura's KOMET-001 clinical trial. Additional information about clinical trials for ziftomenib can be found at www.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, designed to target cancer signaling pathways. Ziftomenib, a once-daily, oral menin inhibitor, is the first and only investigational therapy to receive BTD from the FDA for the treatment of R/R *NPM1*-m AML. In November 2024, Kura entered into a global strategic collaboration agreement with Kyowa Kirin 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*-m AML has been completed, and in the second quarter of 2025, the companies announced submission of an NDA for ziftomenib for the treatment of adult patients with R/R *NPM1*-m AML. Kura and Kyowa Kirin are conducting a series of clinical trials to evaluate ziftomenib in combination with current standards of care in newly diagnosed and R/R *NPM1*-m and *KMT2A*-rearranged AML. KO-2806, a next-generation farnesyl transferase inhibitor (FTI), is being evaluated in a Phase 1 dose-escalation trial (FIT-001) as a monotherapy and in combination with targeted therapies for patients with various solid tumors. Tipifarnib, a potent and selective FTI, is currently in a Phase 1/2 trial (KURRENT-HN) 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, we have invested in drug discovery and biotechnology innovation for more than 70 years and are 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 our values, to sustainable growth, and to making people smile unites us across the globe. You can learn more about the business of Kyowa Kirin at: https://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 efficacy, safety and therapeutic potential of ziftomenib; the anticipated timing of the FDA's notification on its preliminary evaluation of the NDA for ziftomenib; future interactions with the FDA related to the NDA; and the continued progress of Kura's ziftomenib program and our collaboration with Kyowa Kirin. 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 the FDA, risks associated with reliance on third parties to successfully conduct clinical trials, the risks associated with reliance on outside financing to meet capital requirements, 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 the Company faces, please refer to the Company'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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[1] Issa G, et al. Blood Adv 2023;7(6):933-42.

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