Kura Oncology Receives Breakthrough Therapy Designation for Ziftomenib in NPM1-Mutant AML

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– Ziftomenib is the first investigational treatment to be granted Breakthrough Therapy Designation for NPM1-mutant AML –

– Registration-directed trial of ziftomenib in NPM1-mutant AML on track to complete enrollment by mid-2024 –

SAN DIEGO, April 22, 2024 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA), a clinical-stage biopharmaceutical company committed to realizing the promise of precision medicines for the treatment of cancer, today announced that its investigational drug, ziftomenib, has been granted Breakthrough Therapy Designation (BTD) by the U.S. Food and Drug Administration (FDA) for the treatment of patients with relapsed/refractory (R/R) NPM1-mutant acute myeloid leukemia (AML).

FDA granted BTD for ziftomenib based on data from Kura's ongoing KOMET-001 clinical trial in patients with R/R NPM1-mutant AML. BTD is for a drug that treats a serious or life-threatening condition and for which preliminary clinical evidence indicates the drug may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. The designation is intended to expedite development and review of drugs, including an organizational commitment by FDA senior managers and experienced review staff as well as eligibility for rolling review and priority review.[1]

"We are highly encouraged by FDA's decision to grant Breakthrough Therapy Designation to ziftomenib, recognizing its potential as an innovative medicine for patients with relapsed/refractory NPM1-mutant AML," said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. "NPM1-mutant AML represents approximately 30% of new AML cases annually, and this designation reflects that NPM1-mutant AML is a disease of significant unmet need for which there is no approved targeted therapy as well as the fact that ziftomenib offers potential to demonstrate substantial improvement over available therapies. We remain committed to bringing ziftomenib to the market as quickly as possible and look forward to working more closely with FDA to bring our ziftomenib program to patients in urgent need of effective treatments."

Kura is on track to complete the registration-directed trial of ziftomenib in R/R NPM1-mutant AML by mid-2024. Ziftomenib is also being evaluated in combination with current standards of care, including venetoclax/azacitidine or cytarabine plus daunorubicin (7+3) in NPM1-mutant and KMT2A-rearranged AML (KOMET-007) and with gilteritinib, FLAG-IDA or LDAC in NPM1-mutant and KMT2A-rearranged AML (KOMET-008).

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 occurrence of 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[2]. There are currently no FDA-approved therapies targeting NPM1-m AML.

About Ziftomenib

Ziftomenib is a novel, once-daily, oral investigational drug candidate targeting the menin-KMT2A/MLL protein-protein interaction for treatment of genetically defined AML patients with high unmet need. In the KOMET-001 Phase 1 study, ziftomenib demonstrated an encouraging safety profile and tolerability with reported events most often consistent with features and manifestations of underlying disease. Clinical activity of ziftomenib as a monotherapy was optimal at the 600 mg daily dose and a 35% complete remission rate was observed in 20 patients with NPM1-mutant AML treated at the recommended Phase 2 dose (600 mg). Ziftomenib has received Orphan Drug Designation from the U.S. Food and Drug Administration for the treatment of AML. Additional information about clinical trials for ziftomenib can be found at 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 is a once-daily, oral drug candidate targeting the menin-KMT2A protein-protein interaction for the treatment of genetically defined AML patients with high unmet need. Kura is currently enrolling patients in a Phase 2 registration-directed trial of ziftomenib in NPM1-mutant R/R AML (KOMET-001). The Company is also conducting a series of studies to evaluate ziftomenib in combination with current standards of care, including venetoclax/azacitidine and 7+3 in NPM1-mutant and KMT2A-rearranged newly diagnosed and R/R AML (KOMET-007) and with gilteritinib, FLAG-IDA or LDAC in NPM1-mutant and KMT2A-rearranged R/R AML (KOMET-008). Tipifarnib, a potent and selective farnesyl transferase inhibitor (FTI), is currently in a Phase 1/2 trial in combination with alpelisib for patients with PIK3CA-dependent head and neck squamous cell carcinoma (KURRENT-HN). Kura is also evaluating KO-2806, a next-generation FTI, in a Phase 1 dose-escalation trial as a monotherapy and in combination with cabozantinib in clear cell renal cell carcinoma and with adagrasib in $KRAS^{G12C}$-mutated non-small cell lung cancer (FIT-001). For additional information, please visit Kura's website at www.kuraoncology.com and follow us on X and LinkedIn.

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, potential benefits of combining ziftomenib with appropriate standards of care, and progress and expected timing of the ziftomenib program and clinical trials. 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 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] U.S. Food and Drug Administration. Breakthrough Therapy Designation. Accessed April 23, 2024.

[2] Issa G, et al. Blood Adv 2023;7(6):933-42.

Source: Kura Oncology, Inc.