Kura Oncology’s Menin-MLL Inhibitor KO-539 Receives Orphan Drug Designation from FDA for Treatment of Acute Myeloid Leukemia

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SAN DIEGO, July 24, 2019 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA), a clinical-stage biopharmaceutical company focused on the development of precision medicines for oncology, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to the Company’s menin-mixed lineage leukemia (menin-MLL) inhibitor KO-539 for the treatment of acute myeloid leukemia (AML).

“Orphan Drug Designation for AML represents a significant milestone in the development of KO-539,” said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. “This decision by the FDA follows the clearance of our investigational new drug (IND) application in March 2019 and recognizes the potential for KO-539 to address a high unmet need for patients suffering from AML. We are very encouraged by our preclinical data in genetically defined subsets, such as tumors with MLL fusions and rearrangements and NPM1 mutations, and we are in the final stages of study startup for our Phase 1 clinical trial in relapsed or refractory AML.”

The FDA's Orphan Drug Designation program provides orphan status to drugs defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan Drug Designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemptions and seven-year marketing exclusivity upon FDA approval.

About KO-539

KO-539 is a potent and selective small molecule inhibitor of the menin-MLL protein-protein interaction. MLL-rearranged leukemias are characterized by chromosomal translocations of the MLL gene that are primarily found in patients with AML and acute lymphoblastic leukemia (ALL). These translocations form oncogenes encoding MLL fusion proteins, which play a causative role in the onset, development and progression of MLL-rearranged leukemias. The target genes of the MLL fusion proteins are also found to be overexpressed in a broader subset of AMLs characterized by oncogenic driver mutations in genes such as NPM1. These mutations also appear to be dependent on the interaction between menin and MLL, suggesting that the menin-MLL complex is a central node in epigenetic dysregulation driven by distinct oncogenic driver mutations known to be important in AML and other hematologic malignancies. In preclinical studies, KO-539 has demonstrated potent and selective inhibition of the proliferation of MLL-rearranged leukemia cell lines. Kura has also generated preclinical data showing robust and durable efficacy in multiple *in vivo* models of AML characterized by MLL-rearrangements or oncogenic driver mutations in genes such as NPM1.

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 where there is a strong scientific and clinical rationale to improve outcomes by identifying those patients most likely to benefit from treatment. Kura’s lead drug candidate is tipifarnib, a farnesyl transferase inhibitor, for which the Company is conducting a registration-directed trial of tipifarnib in recurrent or metastatic patients with HRAS mutant HNSCC. In addition, tipifarnib is being evaluated in multiple other Phase 2 clinical trials in solid tumor and hematologic indications. Kura’s pipeline also includes KO-947, an ERK inhibitor, currently in a Phase 1 dose-escalation trial, and KO-539, a menin-MLL inhibitor, which is anticipated to enter into a Phase 1 clinical trial shortly. For additional information about Kura, please visit the Company’s website at www.kuraoncology.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 Kura’s product candidate KO-539, the progress and expected timing of Kura’s drug development programs and clinical trials and the potential benefits of Orphan Drug Designation. 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 applications, risks associated with reliance on third parties to successfully conduct clinical trials, the risks associated with reliance on outside financing to meet capital requirements, risks that the actual benefits of Orphan Drug Designation will not be as expected 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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