# Kura Oncology Presents First Clinical Data for Menin Inhibitor KO-539 at American Society of Hematology Annual Meeting

December 5, 2020

– Evidence of biologic activity observed in each dose-escalation cohort treated to date –

– Clinical activity includes one CR in a patient with a NPM1 mutation and one CR in a patient with a SETD2/RUNX1 mutation –

– Continuous daily dosing well tolerated and with manageable safety profile to date –

– Enrollment continues in dose escalation of Phase 1/2A clinical trial in patients with relapsed/refractory AML –

– Anticipate recommending Phase 2 dose and advancing into expansion cohorts in first quarter of 2021 –

– Management to host virtual investor event today at 2:00 p.m. ET / 11:00 a.m. PT –

SAN DIEGO, Dec. 05, 2020 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq: KURA), a clinical-stage biopharmaceutical company focused on the development of precision medicines for the treatment of cancer, today announced preliminary clinical data from KOMET-001, an ongoing Phase 1/2A clinical trial of the Company's oral, potent and selective menin inhibitor, KO-539, including single-agent activity in genetically defined subgroups of patients with relapsed or refractory acute myeloid leukemia (AML).

These data are being presented during an oral session at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition. A copy of the presentation is available on Kura's website at www.kuraoncology.com/pipeline/publications.

"The preliminary first-in-human data generated by KO-539 for the treatment of patients with relapsed or refractory AML is encouraging," said Eunice Wang, M.D., Chief of the Leukemia Service at Roswell Park Comprehensive Cancer Center and principal investigator of the trial. "In addition to a favorable safety and tolerability profile, we have observed evidence of biologic activity in each dose-escalation cohort treated to date. I am delighted to observe evidence of clinical activity in patients with diverse genetic backgrounds, including patients with NPM1 mutations. The preliminary clinical data for KO-539 suggest it has the potential to be effective for multiple genetically defined AML subgroups of high unmet need."

As of the data cutoff on November 2, 2020, the first-in-human, open-label, multicenter trial enrolled 12 patients with relapsed or refractory AML, of whom eight were evaluable for efficacy. Patients were enrolled into four dose cohorts: 50 mg, 100 mg, 200 mg and 400 mg. KO-539 was administered orally, on a once-daily schedule in continuous 28-day cycles. These patients were heavily pretreated and received a median of three prior lines of therapy (range 2-7). Clinical or biological activity was reported in six of the eight efficacy-evaluable patients, including:

- An NPM1 mutant patient with DNMT3A and KMT2D co-mutations achieved a complete remission (CR) with no measurable residual disease. The patient entered the trial following seven prior lines of therapy and remains on KO-539 after three cycles.
- A second NPM1 mutant patient with FLT3-ITD, TET2 and CUX1 co-mutations achieved a morphological leukemia-free state (MLFS) following four prior lines of therapy. Both NPM1 mutant patients were dosed at 200 mg.
- A patient with SETD2 and RUNX1 co-mutations achieved a CR after two cycles and was dose-escalated from 100 mg to 200 mg on cycle seven after blast counts were observed to increase. The patient experienced clinical benefit for more than six months prior to disease progression.
- A patient with a KMT2A/MLL rearrangement had a marked decrease in hydroxyurea requirements and attained peripheral blood count stabilization at the 50 mg starting dose.

Notably, the clinical activity observed across patients was not correlated with concomitant treatment with CYP3A4 inhibitors. This is supported by drug pharmacokinetic studies in patients, which showed that KO-539 metabolism appears to be unaffected by co-administration of CYP3A4 inhibitors.

Four patients were not evaluable for efficacy as of the data cutoff, including an NPM1 mutant patient and a DNMT3A mutant patient with CUX1, ASXL1, IDH2, CBL, U2AF1 and RUNX1 co-mutations in the 400 mg cohort and a KMT2A/MLL-r patient in the 200 mg cohort.

Continuous daily dosing of KO-539 has been well tolerated and with a manageable safety profile to date. There have been no drug discontinuations due to treatment-related adverse events (AEs) and no evidence of QTc prolongation or other clinically significant EKG changes. Treatment related AEs (grade ≥ 3) have included pancreatitis, increased lipase, decreased neutrophil count, tumor lysis syndrome and deep venous thrombosis.

Kura expects to determine a maximum tolerated dose and/or a recommended Phase 2 dose in the first quarter of 2021, at which point the Company intends to advance into expansion cohorts in NPM1-mutant AML and KMT2A/MLL-rearranged AML, selected patient populations where KO-539 has the potential to demonstrate pronounced clinical benefit.

"We are pleased to see encouraging evidence of biologic and clinical activity from KO-539 in the preliminary data, particularly in these heavily pretreated AML patients who were enrolled without genetic selection in the dose-escalation portion of this trial," said Stephen Dale, M.D., Chief Medical Officer of Kura Oncology. "We are also encouraged by the clinical activity observed in cytogenetically normal patients with various co-mutations whose activity may be implicated by the upstream menin-KMT2A/MLL interaction, and believe these patients may represent a potential third expansion cohort. Meanwhile, we continue to explore options to broaden the opportunity for KO-539 in the treatment of acute leukemias, as well as in combination with chemotherapy and targeted therapies to support advancement to earlier lines of therapy."

**Virtual Investor Event**

Kura's management will host a virtual investor event at 2:00 p.m. ET / 11:00 a.m. PT today, December 5, 2020, to provide a review of KO-539 following the oral presentation of preliminary clinical data at the ASH Annual Meeting. The event will feature members of the Kura management team along with two of the investigators from the KOMET-001 clinical trial, Dr. Eunice Wang and Dr. Ghayas Issa. A live video webcast of the event will be available in the Investors section of Kura's website at www.kuraoncology.com, with an archived replay available shortly after the conclusion of the event.

**About Acute Myeloid Leukemia**

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, especially in the relapsed/refractory setting. More than 50% of patients with AML who achieve a CR after induction therapy relapse within one to three years, and less than 10% of those with relapsed/refractory AML are alive at three years. Prognosis is poor in patients with KMT2A rearrangements and in those with co-mutations that may include NPM1.

**About KOMET-001**

KOMET-001 (Kura Oncology Menin Inhibitor Trial) is a Phase 1/2A clinical trial to determine the safety, tolerability and recommended Phase 2 dose of KO-539 in patients with refractory or relapsed AML. A planned expansion phase in specific genetic subgroups, including NPM1 mutant AML and KMT2A/MLL rearranged AML, is expected to further evaluate anti-leukemic activity and tolerability of KO-539. Additional information about KOMET-001 can be found at kuraoncology.com/clinical-trials-komet.

**About KO-539**

KO-539 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 preclinical models, KO-539 inhibits the KMT2A/MLL protein complex and has downstream effects on HOXA9/MEIS1 expression. KO-539 has received Orphan Drug Designation from the U.S. Food and Drug Administration for the treatment of AML.

**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 two wholly owned 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 most advanced drug candidate is tipifarnib, a potent, selective and orally bioavailable farnesyl transferase inhibitor currently in a registration-directed trial (AIM-HN) in patients with recurrent or metastatic HRAS mutant head and neck squamous cell carcinoma. The Company's pipeline is also highlighted by KO-539, a potent and selective inhibitor of the menin-KMT2A/MLL protein-protein interaction currently in a Phase 1/2A clinical trial (KOMET-001) in patients with relapsed or refractory AML. 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 drug candidate, KO-539, progress and expected timing of Kura's development program and clinical trials for KO-539, the presentation of KO-539 data, plans regarding future clinical trials and Kura's potential for growth. 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 Oncology 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, 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 Oncology assumes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.*

**Contacts**

Company:
Pete De Spain
Vice President, Investor Relations &
Corporate Communications
(858) 500-8803
pete@kuraoncology.com

Investors:
Robert H. Uhl
Managing Director
Westwicke Partners, LLC
(858) 356-5932
robert.uhl@westwicke.com

Media:
Jason Spark
Managing Director
Canale Communications
(619) 849-6005
jason@canalecomm.com

Source: Kura Oncology, Inc.