

# Kura Oncology Identifies Potential Biomarkers of Activity for Lead Candidate Tipifarnib in Bone Marrow Cancers

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CXCR4/CXCR2 expression ratio and bone marrow homing of myeloid cells identified as potential markers for tipifarnib in MDS, AML and CMML

CXCL12/CXCR4 pathway a potential therapeutic target of farnesyl transferase inhibitors

Findings support development of tipifarnib in certain bone marrow cancers

Results presented at American Society of Hematology Annual Meeting

SAN DIEGO, Dec. 11, 2017 (GLOBE NEWSWIRE) -- Kura Oncology, Inc. (Nasdaq:KURA), a clinical-stage biopharmaceutical company focused on the development of precision medicines for oncology, today reported new findings supporting the development of lead candidate tipifarnib, a potent and selective inhibitor of farnesyl transferase, in the treatment of certain bone marrow cancers. These results were featured in presentations at the American Society of Hematology (ASH) Annual Meeting and Exposition in Atlanta. Copies of the posters are now available on the company's website at <a href="https://www.kuraoncology.com">www.kuraoncology.com</a>.

Among the findings presented at ASH were the identification of CXCR4/CXCR2 expression ratio and bone marrow homing of myeloid cells as potential biomarkers of tipifarnib activity across the bone marrow cancers, myelodysplastic syndromes (MDS), acute myeloid leukemia (AML) and chronic myelomonocytic leukemia (CMML), further showing that the CXCL12/CXCR4 pathway is a potential therapeutic target of farnesyl transferase inhibitors.

"Although tipifarnib has previously demonstrated clinical responses in certain patients with AML and MDS, no molecular mechanism of action was identified that could explain the activity of the drug candidate in those patient populations," said Troy Wilson, Ph.D., J.D., President and Chief Executive Officer of Kura Oncology. "These new findings are exciting because they may define potential biomarkers for tipifarnib in bone marrow tumors and characterize a subgroup of patients that are most likely to derive clinical benefit from a targeted therapy such as tipifarnib."

Previously, Kura Oncology reported preliminary results from an ongoing Phase 2 clinical trial of tipifarnib in patients with peripheral T-cell lymphoma (PTCL) identifying the CXCL12/CXCR4 pathway as a potential target of tipifarnib. Specifically, high levels of CXCL12 gene expression and absence of single nucleotide gene variations in the 3'-untranslated region of the CXCL12 gene were associated with observed clinical activity of tipifarnib in these PTCL patients.

In the ASH presentation entitled, "The CXCL12/CXCR4 Pathway As a Potential Target of Tipifarnib: Preliminary Results from an Open-Label, Phase II Study in Relapsed or Refractory Peripheral T-Cell Lymphoma," Kura Oncology extends these observations and provides data supporting the observed tipifarnib-derived clinical benefit for the CXCL12-positive population.

CXCL12 is a chemokine that is secreted in large amounts by lymph nodes, bone marrow stroma, liver, and lung, and plays key roles in tumor invasion, bone marrow homing and site of metastasis. Among its multiple functions, CXCL12 is essential for homing of myeloid cells to the bone marrow and lymphoid cells to lymph nodes and other organs.

Based on its initial observations in PTCL, the company investigated a role for the CXCL12/CXCR4 pathway and bone marrow homing of myeloid cells as biomarkers of tipifarnib activity in AML and MDS studies.

In the ASH presentation entitled, "The CXCL12/CXCR4 Pathway As a Potential Target of Tipifarnib in Acute Myeloid Leukemia and Myelodysplastic Syndromes," Kura Oncology presented results that identify the ratio of CXCR4/CXCR2 gene expression and bone marrow homing of myeloid cells as potential biomarkers of the activity of tipifarnib in certain bone marrow tumors. The results were obtained by analyzing data from previous studies of tipifarnib in AML and MDS, as well as data from the ongoing Phase 2 clinical trial of tipifarnib in CMML.

"We were very encouraged to identify CXCR4/CXCR2 expression ratio and bone marrow homing as markers of tipifarnib's activity in MDS, AML and CMML," said Antonio Gualberto, M.D., Ph.D., Chief Medical Officer at Kura Oncology. "Although our analysis is retrospective, the fact that we observe a consistent clinical benefit across different endpoints, treatment settings and indications gives us increased confidence in the potential for these biomarkers. Based on our preliminary data, we believe CXCL12/CXCR4 may have the potential to unlock the therapeutic value of farnesyl transferase inhibition across multiple bone marrow neoplasias."

# **About Tipifarnib**

Kura Oncology's lead candidate, tipifarnib, is a potent and selective inhibitor of the enzyme farnesyl transferase, a key cell signaling process implicated in cancer initiation and development. Tipifarnib has previously been studied in more than 5,000 patients in more than 70 clinical trials has a well-established safety profile and has demonstrated compelling and durable anti-cancer activity in certain patient subsets.

Leveraging advances in next-generation sequencing as well as emerging information about cancer genetics and tumor biology, Kura Oncology is seeking to identify patients most likely to benefit from tipifarnib. The company is conducting clinical and preclinical studies in multiple disease indications where tipifarnib has previously shown signs of activity with the goal of identifying and validating biomarkers associated with the observed

clinical activity of tipifarnib.

In September 2017, Kura Oncology reported that its Phase 2 trial of tipifarnib in patients with HRAS mutant head and neck squamous cell carcinomas (HNSCC) achieved its primary efficacy endpoint prior to the completion of patient enrollment. The company is now planning to initiate a registration-enabling study of tipifarnib in HRAS mutant HNSCC in 2018.

#### **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 Oncology's lead drug candidate is tipifarnib, a farnesyl transferase inhibitor, which is currently being studied in multiple Phase 2 clinical trials. The company's pipeline also includes KO-947, an ERK inhibitor, currently in a Phase 1 trial, and KO-539, an inhibitor of the menin-MLL protein-protein interaction, currently in preclinical testing. For additional information about Kura Oncology, please visit the company's website at <a href="https://www.kuraoncology.com">www.kuraoncology.com</a>.

### **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 tipifarnib, progress and expected timing of Kura Oncology's drug development programs and clinical trials and plans regarding future clinical trials and development activities. 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 <a href="https://www.sec.gov.such forward-looking statements">www.sec.gov.such forward-looking statements

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