Data suggesting that omacetaxine can eradicate leukemic stem cells may offer a breakthrough for CML

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Data showing the ability of omacetaxine to kill leukemic stem cells in mouse models with drug-resistant chronic myelogenous leukemia (CML) are the subject of an advance online publication in the journal *Leukemia,* ChemGenex Pharmaceuticals Limited (ASX:CXS and NASDAQ:CXSP) announced today. The findings of this study provide new insights into the problem of minimal residual disease and may open the door to the development of a curative treatment strategy for some patients with CML.

Commenting on the publication, Dr. Shaoguang Li, the study leader at the University of Massachusetts Medical School, said, "Omacetaxine killed 90% of the leukemic stem cells in vitro. In contrast, imatinib and dasatinib, the two leading CML drugs, only controlled 9% and 25% of these cancer stem cells, respectively. We further demonstrated that omacetaxine prolonged survival in test animals carrying the T315I mutation, which would normally render them resistant to all currently available drugs."

ChemGenex is currently developing omacetaxine as a potential treatment for a range of blood malignancies, including CML, and is completing a pivotal study in CML patients who harbor the T315I mutation. The company expects to complete its filing of a New Drug Application for use of omacetaxine in that patient population to the U.S. Food and Drug Administration by mid-year.

In the USA, almost 5,000 new cases of CML are diagnosed each year and recent estimates suggest that by 2025, prevalence will be 300,000. While there are a number of licensed drugs, collectively known as tyrosine kinase inhibitors (TKIs), which are very effective in treating CML, they must be administered daily for the rest of the patient's life; very few patients remain disease free when these drugs are discontinued.

Hagop M. Kantarjian, M.D., Chairman and Professor, Department of Leukemia, at M.D. Anderson Cancer Center in Houston, Texas, described the study results as "very promising". He added, "While the currently licensed drugs target and disable the diseased cells in the blood stream and bone marrow, they have little, if any, affect on the primitive leukemic stem cells that are at the "root" of this blood cancer. I look forward to working with ChemGenex in future trials to evaluate the clinical application of these recent findings. "

Greg Collier, Ph.D., Managing Director and Chief Executive Officer of ChemGenex added, "The results of Dr. Li's animal study are very encouraging and we are currently collaborating with Professor Tessa Holyoake from the United Kingdom, to carry out similar investigations in primary human stem and progenitor cells. In the meantime, ChemGenex remains focused on our primary objective of developing omacetaxine as a therapeutic option for CML patients who have developed the T315I mutation and who are resistant to all first and second line TKIs. This is the most pressing unmet medical need in the field of CML management."

About CML

CML is a form of leukemia characterized by the increased and unregulated growth of undifferentiated myeloid cells in the bone marrow and the accumulation of these non-functional cells in the blood. Healthy myeloid cells should give rise to some of the most important components

of blood:

red cells (to carry oxygen around the body), platelets (to help with blood clotting) and certain white cells (to help defend against infection).

CML patients therefore show symptoms of anemia and are prone to bleeding and are susceptible to illnesses caused by bacterial and fungal infections.

Minimal residual disease (MRD) is the name given, to small numbers of leukemic cells that remain in the patient during treatment, or after treatment when the patient is in remission (no symptoms or signs of disease). It is the major cause of relapse in cancer and leukemia.

Source: Kureczka/Martin Associates