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CORNERSTONE PHARMACEUTICALS' CANCER METABOLISM INHIBITOR, CPI-613, SHOWING PROMISE IN PHASE I TRIAL FOR ACUTE MYELOID LEUKEMIA

Data to be presented at 53rd Annual American Society of Hematology Meeting (ASH)

San Diego, CA, December 12, 2011 –[Cornerstone Pharmaceuticals](#) today announced its first –in-class cancer metabolism inhibitor drug will be featured in a poster session at the [53rd Annual Meeting and Exposition of the American Society of Hematology \(ASH\)](#). [Findings from the study](#) will be presented along with preclinical findings at ASH from a Phase I clinical trial of CPI-613 in refractory acute leukemias. In August 2011, CPI-613 received FDA orphan drug designation for the treatment of acute myeloid leukemia (AML).

CPI-613 is the subject of a Phase I clinical single agent trial to evaluate safety, tolerability, maximum tolerated dose, efficacy, and pharmacokinetics for patients with relapsed and refractory hematologic malignancies. To date, eleven patients have been treated with at least one cycle of CPI-613. Interim clinical data suggests that altered lipid and mitochondrial metabolism are viable targets in acute leukemias. CPI-613 is a novel agent that has activity against several acute leukemia cell lines *in vitro* and *in vivo* and demonstrates activity in patients with relapsed AML and Myelodysplastic Syndrome (MDS). Several patients experienced significant clinical benefit, including a sustained complete remission when treated with CPI-613 as a single agent. Only minor toxicities were observed.

According to the National Cancer Institute, one in 77 men and women will be diagnosed with leukemia during their lifetime. In adults these diseases disproportionately affect the elderly with a median age of onset of 72 for acute myeloid leukemia. Patients over the age of 60 have less than 10 percent survival at five years. It is estimated that 12,950 Americans (6,830 men and 6,120 women) will be diagnosed with acute myeloid leukemia in 2011. Chemotherapy is the standard of treatment for untreated adult. Cornerstone's CPI-613 may represent a potential new alternative treatment for this disease.

[“This study demonstrates early proof of concept for this class of agents](#), which is very important because relatively few treatment options exist for patients with myeloid leukemia, especially with relapsed/refractory disease,” said the CEO of Cornerstone Pharmaceuticals, Robert Shorr, Ph.D. “Preliminary results from the ongoing Phase I study demonstrate the safety of this first-in-class agent, CPI-613, with very few adverse events, and support further exploration of CPI-613 as a potential treatment for AML and other hematologic malignancies.”



The CPI-613 poster session, "[Altered Lipid and Mitochondrial Metabolism Are Viable Targets in Acute Leukemia](#)" is scheduled for December 12 from 6 p.m. to 8 p.m. at Hall GH (San Diego Convention Center).

About [CPI-613](#)

CPI-613 is the first drug in a new chemical class that, through a novel mechanism, targets metabolic changes considered to be common to many, if not all, cancer types and not present in normal cells. Patients with solid tumors and hematologic cancers are currently being treated in multiple Phase I and Phase I/II human clinical trials evaluating CPI-613. These trials include a Phase I/II single agent trial for patients with solid tumors who have failed all other therapy options, a Phase I/II combination trial with gemcitabine in newly diagnosed or relapsed patients, and a single agent trial in hematologic malignancies. CPI-613 was granted orphan drug status by the US FDA for pancreatic cancer and for AML.

About [Cornerstone Pharmaceuticals](#)

Cornerstone Pharmaceuticals, Inc. is a privately held company that is committed to changing the way cancer is treated through the discovery and development of innovative therapies capitalizing on the unique metabolic processes of cancer cells. The company's founding members, management and scientific advisory team include pre-eminent scientists focused on cancer cell metabolism, cancer research and drug development. The company's unique approach to targeting cancer metabolism has led to the discovery of first-in-class drugs with the potential to transform the way cancer is treated.

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This release contains forward-looking statements. These statements relate to future events or each company's future financial performance. In some cases, you can identify forward-looking statements by terminology such as "may", "will", "should", "expect", "plan", "anticipate", "believe", "estimate", "predict", "potential" or "continue", the negative of such terms, or other comparable terminology. These statements are only predictions. Actual events or results may differ materially from those in the forward-looking statements as a result of various important factors. Although we believe that the expectations reflected in the forward-looking statements are reasonable, such statements should not be regarded as a representation by the company, or any other person, that such forward looking statements will be achieved. The business and operations of the company are subject to substantial risks which increase the uncertainty inherent in forward-looking statements. We undertake no duty to update any of the forward-looking statements, whether as a result of new information, future events or otherwise. In light of the foregoing, readers are cautioned not to place undue reliance on such forward-looking statements.