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Phase I Trial Data for Cornerstone Pharmaceuticals' CPI-613 in Combination with High Dose Ara-C (HDAC) and Mitoxantrone in Relapsed or Refractory Acute Myeloid Leukemia to Be Presented at 57th Annual ASH Meeting

Combination is Highly Active in Poor Risk Relapsed or Refractory Patients

CRANBURY, NEW JERSEY (November 23, 2015) – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced that data from the Phase I clinical trial evaluating CPI-613 in combination with high dose Ara-C (HDAC) and mitoxantrone in relapsed or refractory acute myeloid leukemia (AML) is to be presented at the 57th annual meeting of the American Society of Hematology (ASH) in Orlando, Florida on Sunday, December 6, 2015. CPI-613 is the company's lead Altered Energy Metabolism Directed (AEMD) drug candidate, a first-in-class anticancer compound designed to disrupt the altered energy production pathways in cancer cells by targeting mitochondrial metabolism.

The trial was performed by researchers at Wake Forest Baptist Medical Center. The abstract for the presentation was made available online on November 5, 2015 at <https://ash.confex.com/ash/2015/webprogram/Paper79012.html>.

“We are very pleased that the results have been accepted for publication by ASH, the world's largest professional society concerned with the causes and treatment of blood disorders,” said Steve Carchedi, Chief Executive Officer of Cornerstone Pharmaceuticals. “The patients on this study presented with an exceptionally difficult-to-treat hematologic malignancy. We look forward to working with Wake Forest on continuing clinical trials in this patient population to perfect this combination therapeutic approach.”

The open-label, dose-escalation Phase I trial was designed solely to determine the maximum tolerated dose (MTD), safety, and efficacy of CPI-613 given intravenously in combination with HDAC and mitoxantrone. A total of 67 patients (median age of 60, ages ranging between 21-79) with advanced relapsed or refractory AML were enrolled in the trial, with 65 patients ultimately being evaluable. The goal of the study was to get responders to stem cell transplant whenever possible.

The overall intention to treat response rate was 48% (26CR+5CRi) with a median survival of 6.4 months. In patients ≥ 60 years old, the CR/CRi rate was 42% (15/36). The response rate for patients with poor risk cytogenetics was 47% (11CR+3CRi) with a median survival of 5.2 months, whereas, in a historical cohort treated with HDAC, mitoxantrone, and asparaginase, only 19% (3/16) of patients with poor risk cytogenetics responded, with a median survival of 2.8 months. At the time of submission of the abstract, thirteen patients were able to receive allogeneic stem cell transplantation.



About CPI-613

CPI-613 is the lead drug candidate from Cornerstone's proprietary AEMD platform. Cornerstone's AEMD drug platform disrupts the essential "bioenergetic" differences that support the growth and development of many types of cancer cells. In the case of CPI-613, the compound has been shown to selectively induce inhibition of pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH), key mitochondrial enzymes involved in cancer cell metabolism *in vitro*. Disruption of PDH and KGDH function cuts off the tumor's energy supply, culminating in cell death. CPI-613 is currently being evaluated in Phase I, I/II, and II human clinical trials in solid tumors and hematological malignancies.

About Cornerstone Pharmaceuticals

Cornerstone Pharmaceuticals, Inc. is a clinical stage, oncology-focused pharmaceutical company committed to the development and commercialization of therapies that exploit the metabolic differences between normal cells and cancer cells. The company's primary objective is to develop highly selective and effective agents with minimal toxic effects on normal cells and tissues. The company's unique approach to targeting cancer metabolism has led to two distinct technology platforms: altered energy metabolism directed, or AEMD, compounds and an Emulsiphan lipid nanoemulsion based drug delivery system. www.cornerstonepharma.com.

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This release contains forward-looking statements. These statements relate to future events or the 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.