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Cornerstone Pharmaceuticals' CPI-613 Phase I Hematologic Malignancies Trial Data Published in AACR's *Clinical Cancer Research*

Further validation of proprietary Altered Energy Metabolism Directed (AEMD) technology platform

CRANBURY, NEW JERSEY (October 7, 2014) – 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 advanced hematological malignancies has been published in the recent online issue of *Clinical Cancer Research*, the official journal of the American Association of Cancer Research (AACR). 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 publication of these encouraging data was previously announced by Wake Forest Baptist Medical Center. Data from the trial were first presented at the 2013 American Society of Clinical Oncology (ASCO) Annual Meeting by Dr. Timothy S. Pardee, the Principal Investigator for the trial which is sponsored by Wake Forest Baptist. In addition, these data were selected for inclusion in the 2013 Best of ASCO educational series.

"The online publication of the manuscript from the completed Phase I study evaluating our lead drug candidate CPI-613 in relapsed or refractory hematological malignancies is an important achievement for Cornerstone Pharmaceuticals as it further supports the science behind our Altered Energy Metabolism Directed (AEMD) technology platform in a highly-respected, peer-reviewed journal," said Robert Rodriguez, President and Chief Operating Officer of Cornerstone Pharmaceuticals. "Results of the trial conducted by our colleagues at Wake Forest Baptist show that CPI-613 provides signals of activity in a heavily pre-treated cohort of patients suffering from a variety of hematologic malignancies. We look forward to continuing clinical trials in this and additional patient populations to develop the therapy for patients who have unmet needs across a broad spectrum of cancers."

The open-label, dose-escalation monotherapy Phase I trial was designed to determine the maximum tolerated dose (MTD), safety, and efficacy of CPI-613 given as a single agent by IV infusion. A total of 26 patients with advanced relapsed or refractory hematological malignancies, which include a variety of leukemias and lymphomas, were enrolled in the trial and administered CPI-613 on days one and four of each week for three weeks every 28 days ("a cycle"). Dose escalation was performed in six cohorts, with a starting dose of 420 mg/m^2 to a final dose of 3780 mg/m^2. A total of six patients who received at least a full cycle of treatment were treated at a dose of 2940 mg/m^2 over two hours, with no dose limiting toxicities observed, establishing this as the MTD in this study. In addition, CPI-613 was well tolerated with no bone marrow suppression observed.

Of the 21 evaluable patients who completed the trial, eight were deemed to have derived benefit from treatment, representing disease control rate of 38%, and six, or 29%, achieved objective responses. Such responses included a patient with relapsed myelodysplastic syndrome (MDS) who achieved a complete remission which has been maintained for over three years and who continues to be treated with CPI-613 as well as a patient enrolled in July of 2011 with acute myeloid leukemia (AML) who failed to respond to five different prior chemotherapeutic regimens. This patient achieved a morphologic leukemia free state after two cycles of treatment and who, to date, remains alive with no evidence of leukemia. Two relapsed lymphoma patients, one with Burkitt's lymphoma and a second with cutaneous T-cell lymphoma, achieved prolonged partial responses in this study. The Burkitt's lymphoma patient received 17 cycles of treatment with CPI-613 prior to electing to have surgical resection of her residual disease and is currently alive with no evidence of disease now more than one year post surgery. The relapsed cutaneous T-cell lymphoma patient has continued on CPI-613 therapy for over two years, having relapsed from 10 prior treatment regimens prior to enrollment in this study. Additionally, stable disease was observed in two patients with multiple myeloma and two patients with MDS.

"We are highly encouraged by the clinical responses produced by CPI-613 in the Phase I study," said Timothy Pardee, M.D., Ph.D., director of leukemia translational research at Wake Forest Baptist and Principal Investigator of the study. "Through its unique mechanism of action, the first-in-class drug is selectively taken up by cancer cells and inhibits the production of energy in the mitochondria of these cells. However, what is rather astounding in the Phase I study is CPI-613's ability to allow patients to achieve and maintain varying degrees of response including one complete remission. We look forward to evaluating CPI-613 in future studies and select patient populations."

About Wake Forest Baptist Medical Center

Wake Forest Baptist Medical Center (wakehealth.edu) is a fully integrated academic medical center located in Winston-Salem, N.C. The institution comprises Wake Forest School of Medicine, a leading center for medical education and research; Wake Forest Baptist Health, the integrated clinical structure that includes nationally ranked Brenner Children's Hospital; Wake Forest Innovations, which promotes the commercialization of research discoveries and operates Wake Forest Innovation Quarter, an urban research and technology park; plus a network of affiliated community hospitals, physician practices, outpatient services and other medical facilities. Wake Forest Baptist clinical programs and the School of Medicine are regularly ranked among the best in the country by U.S. News & World Report.

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.