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Cornerstone Pharmaceuticals Initiates Phase I Clinical Trial of CPI-613 for the Treatment of Hodgkin's or T-Cell Non-Hodgkin's Lymphoma

Study Sponsored by a leading medical center for cancer research and development

CRANBURY, NEW JERSEY (October 21, 2014) – Cornerstone Pharmaceuticals, Inc., a clinical stage company and leader in the growing field of cancer metabolism-based therapeutics, today announced the initiation of a Phase I clinical trial of CPI-613, in combination with bendamustine (Treanda), for patients with relapsed or refractory classic Hodgkin's lymphoma or T-cell non-Hodgkin's lymphoma who may or may not have received hematopoietic cell transplant. CPI-613 is the company's lead Altered Energy Metabolism Directed (AEMD) drug candidate, designed to disrupt the altered energy-production pathways in cancer cells.

Non-Hodgkin's lymphoma (NHL) and Hodgkin's lymphoma (HL) are two common types of cancers of the lymphatic system, part of the immune system, that compromise the body's ability to fight infections. Classic Hodgkin's lymphoma is the more common type of HL, with approximately 95 percent of cases considered to be classic HL. There are projected to be approximately 70,000 new cases of NHL, with close to 19,000 deaths, and over 9,000 new cases of HL, with approximately 1,200 deaths, in the United States during 2014.

The single site study, sponsored by Wake Forest Baptist Medical Center and in collaboration with the National Cancer Institute (NCI), is a Phase I, open label, dose-escalating study designed to establish the maximum tolerated dose of CPI-613 when used in combination with bendamustine. Response and overall survival rates will also be measured in this study. Bendamustine has shown single agent activity in the relapsed lymphoma setting with response rates of approximately 50 percent for B and T-cell NHL as well as in classic HL. The study will include patients with relapsed or refractory classic HL or T-cell NHL who may or may not have received hematopoietic cell transplant.

Robert Rodriguez, Cornerstone's President and Chief Operating Officer, said, "This clinical trial is a key milestone for Cornerstone, further demonstrating the potential applicability of our lead product candidate, CPI-613, in these types of hematologic malignancies, and the first study to examine the drug in the classic Hodgkin's or T-cell non-Hodgkin's lymphoma-specific patient population when combined



with bendamustine. We look forward to working with the investigators at Wake Forest Baptist Medical Center on this study as we continue to explore the use of our AEMD approach in the treatment of a wide array of cancers.”

Zanetta Lamar, MD, of Wake Forest Baptist Medical Center and the Principal Investigator for the trial, remarked, “Relapsed and refractory lymphomas can be extremely debilitating. The long-term survival rate for the majority of these patients is low, and the impact on the ability of the immune system to stave off disease and infection creates further complications. We hope that utilizing CPI-613 in conjunction with bendamustine has the potential to offer a beneficial alternative for the treatment of classic HL and T-cell NHL patients.”

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 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 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 Emulsiphon lipid nanoemulsion based drug delivery system. www.cornerstonepharma.com.

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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.