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Cornerstone Pharmaceuticals Commences Phase I Clinical Trial of CPI-613 for the Treatment of B-Cell Non-Hodgkin Lymphoma

CRANBURY, NEW JERSEY (December 5, 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 assessing the safety and efficacy of escalating doses of CPI-613, in combination with bendamustine and rituximab, in patients with relapsed or refractory B-cell Non-Hodgkin lymphoma (NHL). 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.

Steve Carchedi, Chief Executive Officer of Cornerstone, said, "Initiating this Phase I trial marks the fourth ongoing trial evaluating our lead compound CPI-613 in advanced hematologic malignancies and overall the eighth study being sponsored by notable, National Cancer Institute-designated comprehensive cancer center, Wake Forest Baptist Medical Center. Furthermore, this study represents the continued interest in CPI-613 by the medical community as a unique approach to fighting cancer by blocking the energy producing pathways that support the growth and development of many cancer cells. We look forward to the active enrollment of B-cell Non-Hodgkin Lymphoma patients in this study as we seek to demonstrate the potential of our lead product candidate as part of a combination therapy."

NHL is the most common cancer of the lymphatic system, a part of the immune system, with more than 320,000 Americans suffering from the disease and over 65,000 cases diagnosed each year in the United States.[i] B-cell NHL is the most common form of NHL, comprising more than 85% of all NHL cases.[i]

The single site study, sponsored by Wake Forest Baptist Medical Center and in collaboration with the National Cancer Institute, 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 and rituximab. Secondary outcomes including response rate, overall survival rate, disease control rate and progression-free survival will also be assessed.

Bendamustine is a chemotherapy drug that has shown single agent activity in heavily pretreated patients with rituximab-refractory (resistant), indolent NHL, showing an overall response rate of approximately

77% in clinical trials.[ii] An immunotherapy agent, rituximab was the first monoclonal antibody approved for cancer treatment.[iii] It targets a specific antigen called CD-20 found on most B-cell lymphomas[ii] and has been shown to result in a 40-50% response rate in patients who relapse with indolent B-cell lymphomas.[iv]

Zanetta Lamar, MD, of Wake Forest Baptist Medical Center and the Principal Investigator for the trial, commented, "Survival rates for Non-Hodgkin Lymphoma differ extensively and largely depend on lymphoma type, stage and age of the patient. The disease can be devastating for patients, which is reflected in an overall 5-year relative survival rate of approximately 69% and a 10-year relative survival rate of approximately 58%.[v] In this study, our goal is to determine if the combination of chemotherapy, immunotherapy and CPI-613 as a targeted anti-cancer metabolism therapy is effective and safe for patients with relapsed of refractory B-cell Non-Hodgkin Lymphoma."

CPI-613 is the lead drug candidate from Cornerstone's proprietary AEMD platform. Cornerstone's AEMD drug platform disrupts the metabolism (energy producing) pathways that support the growth and development of many types of cancer cells. CPI-613 has been shown in-vitro to be highly selective in inducing the simultaneous inhibition of two key mitochondrial enzymes involved in cancer cell metabolism: pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH). Disruption of PDH and KGDH function cuts off the tumor's energy supply, culminating in cell death. CPI-613 is currently being evaluated in several 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 Emulsiphan lipid nanoemulsion based drug delivery system.

Currently, Cornerstone's deep oncology portfolio of clinical trials evaluating CPI-613 at notable

institutions includes ongoing studies in both hematologic malignancies and in solid tumors. Overall, CPI-613 has been shown to have broad utility in both solid tumors and hematologic malignancies.

For more information, visit 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.

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