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Cornerstone Pharmaceuticals Presents Updated Phase I Data for CPI-613 at 2013 American Society of Hematology Annual Meeting

Study Sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center

CRANBURY, NEW JERSEY (December 9, 2013) – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced updated results from a Phase I clinical study of its first-in-class cancer metabolism targeted therapeutic, CPI-613, for the treatment of advanced hematological malignancies. The results were presented by the principal investigator, Timothy S. Pardee, MD, of the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, during an oral presentation on Monday, December 9, 2013, at the 2013 American Society of Hematology (ASH) Annual Meeting and Exposition, in New Orleans, LA.

The presentation, titled, “A Phase I study of the safety, efficacy and pharmacokinetics of the first-in-class pyruvate dehydrogenase complex inhibitor CPI-613 in patient with advanced hematologic malignancies,” follows 21 evaluable patients with relapsed or refractory blood malignancies who were given CPI-613 as a single agent. As previously reported, no marrow suppression was observed in the patients treated with CPI-613, which is a common side effect of currently available treatment options. Of the 21 patients, eight achieved a response of stable disease or better, for a response rate of 38%.

Dr. Pardee commented, “Many of these patients who enrolled in this clinical study were running out of treatment options, some of them were even considering hospice as the next step. A 19 year old patient with Burkitt’s lymphoma enrolled in our trial after relapsing. Since treatment with CPI-613, the patient achieved a partial response sustained for 17 months and ultimately underwent surgical resection of her disease. She remains disease free now 10 months after surgery off all therapy. These results are very encouraging and we look forward to further evaluating CPI-613, with the hope of improving the outcomes and life expectancy of more patients.”

In addition to the Burkitt’s lymphoma patient, all three of the patients with myelodysplastic syndrome (MDS) achieved a response of stable disease or better. In one case, a 49 year old patient with therapy related MDS was transfusion dependent prior to enrollment. Shortly following the first cycle of treatment with CPI-613, the patient achieved significant and durable improvement in their white blood



cell and platelet counts and became transfusion independent. By the eighth cycle, the patient achieved complete remission which has lasted for almost three years.

Robert Rodriguez, President and Chief Operating Officer of Cornerstone, stated, “We believe the results to be presented regarding patients in this Phase I study justifies the continued clinical development of CPI-613 for the treatment of hematologic malignancies. We look forward to the presentation of the results from the ongoing Phase I/IIa acute myelogenous leukemia and Phase II myelodysplastic syndrome clinical trials Dr. Pardee is conducting at Wake Forest in the near future.”

CPI-613 is a first-in-class agent that induces cancer-specific inhibition of pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH), key mitochondrial enzymes involved in fueling cancer cell metabolism. Disruption of PDH and KGDH function cuts off the tumor’s energy supply and starves the cancer cell, leading to cancer cell death. CPI-613 is currently being evaluated in Phase I, I/II and II human clinical trials in solid tumors and hematological malignancies.

CPI-613 is the lead drug candidate from Cornerstone's proprietary Altered Energy Metabolism Directed (AEMD) platform. Cornerstone’s AEMD drug platform disrupts biochemical alterations in the conversion of glucose to energy that occur in many types of cancer cells. These essential bioenergetic differences are linked to critical pathways, particularly those that support cancer cell growth and development.

About Wake Forest

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 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. 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.