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Cornerstone Pharmaceuticals Announces Initiation of Phase II Clinical Trial of CPI-613 for the Treatment of Small Cell Lung Cancer

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

CRANBURY, NEW JERSEY (October 24, 2013) – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced the initiation of a pilot Phase II clinical trial of CPI-613 for the treatment of relapsed or refractory small cell lung cancer (SCLC) patients. CPI-613 is the company’s lead Altered Energy Metabolism Directed (AEMD) drug candidate, which is designed to disrupt the altered energy-production pathways in cancer cells.

The study, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, is expected to enroll approximately 20 patients with relapsed or refractory SCLC. Patients participating in the study will receive CPI-613 twice a week, as a single treatment agent, for three weeks of a four week cycle. The study aims to evaluate the disease control rate, overall response rate, progression free survival and overall survival.

Robert Rodriguez, Cornerstone’s President and Chief Operating Officer, said, “We look forward to working with Dr. Ruiz on this Phase II study evaluating the safety and activity of CPI-613, the Company’s first-in-class cancer metabolism directed chemotherapeutic agent, in patients suffering from SCLC. This study represents another milestone in the advancement of CPI-613 towards clinical and commercial fruition and as a potential therapeutic alternative for patients diagnosed with a disease in which the search for life extending treatments has historically been disappointing.”

Jimmy Ruiz, MD, of Wake Forest Baptist, who also serves as the Principal Investigator for this trial, remarked, “SCLC is one of the most aggressive forms of lung cancer, with a rapid doubling time and metastatic potential that leads to significant morbidity and mortality in patients with this disease. The science behind CPI-613, which causes the disruption of energy pathways found specifically in cancer cells, could potentially be a novel and successful treatment option for patients with extremely poor outcomes despite current existing treatment options.”



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

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