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Cornerstone Pharmaceuticals Announces Initiation of Phase II Clinical Trial of CPI-613 in Patients with Locally Advanced or Metastatic Pancreatic Cancer

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

CRANBURY, NEW JERSEY (October 31, 2013) – Cornerstone Pharmaceuticals, Inc., (www.cornerstonepharma.com), 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 patients with locally advanced or metastatic pancreatic cancer. 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.

Pancreatic cancer begins in the tissues of the pancreas – an organ in the abdomen that lies behind the lower part of the stomach and aids in digestion and helps regulate the metabolism of sugars.¹ In the United States, pancreatic cancer, which will be diagnosed in more than 45,000 Americans this year, is the fourth leading cause of cancer-related deaths due to the majority of cases being found during the late stages of the disease after it has spread.² The 1- and 5-year relative survival rates, for all stages combined, are 25% and 6%, respectively.³

The interventional clinical study, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, will enroll patients with locally advanced or metastatic pancreatic cancer documented as having progression on a scan and that cannot be removed by surgery and have failed current available therapies. The primary endpoint of the study is to evaluate the overall survival, with secondary endpoints evaluating the overall response rate, progression free survival, and safety. The US FDA granted orphan drug status to CPI-613 for the treatment of pancreatic cancer in 2006.

Dr. Angela Alistar, MD, of Wake Forest Baptist, who serves as the Principal Investigator for this trial, remarked, “Diagnosing pancreatic cancer is challenging due to the gradual development of symptoms, which are not always obvious during the early stages. In a majority of cases, the cancer is in an advanced stage when it is discovered and by this time the survival rate is very low. With an overall 5-year relative survival rate of only 6%, even if diagnosed in an early stage pancreatic cancer is one of the



more fatal forms of cancer. We believe that CPI-613 is a promising treatment option that has the potential to boost the chances of survival for all stages of this cancer.”

Robert Rodriguez, Cornerstone’s President and Chief Operating Officer, said, “We are pleased to have achieved another significant milestone for our lead AEMD candidate, CPI-613, with the initiation of a Phase II clinical trial for the treatment of pancreatic cancer in patients with poor performance status. During pre-clinical studies, this novel therapeutic has demonstrated its ability to significantly disrupt the metabolic and regulatory process required for cell growth in solid tumor lines by exploiting a highly conserved bioenergetic feature of cancer cell metabolism. We look forward to continuing our work with Dr. Alistar and the Wake Forest Baptist Medical Center as we seek to further validate the safety and activity of CPI-613.”

CPI-613 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 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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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.

¹ **Mayo Clinic**, "Pancreatic Cancer" Last accessed October 17, 2013. Available at <http://www.mayoclinic.com/health/pancreatic-cancer/DS00357>

² **National Health Institute**, "A Snapshot of Pancreatic Cancer" Last accessed October 17, 2013. Available at <http://www.cancer.gov/researchandfunding/snapshots/pancreatic>

³ **National Health Institute**, "Surveillance Epidemiology and End Results Fact Sheets: Pancreas" Last accessed October 17, 2013. Available at <http://seer.cancer.gov/statfacts/html/pancreas.html>