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Cornerstone Pharmaceuticals Announces Initiation of Phase I/II Clinical Trial of CPI-613 with Modified FOLFIRINOX in Patients with Metastatic Pancreatic Cancer

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

CRANBURY, NEW JERSEY (April 22, 2013) – Cornerstone Pharmaceuticals, Inc., (www.cornerstonepharma.com), a leader in the growing field of cancer metabolism-based therapeutics, today announced the initiation of a Phase I/II combination clinical trial of CPI-613 and modified FOLFIRINOX for the treatment of patients with 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, open-label clinical study, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, is expected to enroll patients with metastatic pancreatic cancer and good performance status, which is defined as the patient being able to perform ordinary tasks and carry out daily activities. Patients will receive CPI-613, in combination with modified dose of FOLFIRINOX (mFOLFIRINOX), a chemotherapy combination composed of leucovorin, fluorouracil, irinotecan, and oxaliptatin. The primary endpoint of the study is to determine the maximum tolerated dose of CPI-613 with mFOLFIRINOX, with secondary endpoints evaluating the overall survival, 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, “Pancreatic cancer is sometimes described as a ‘silent’ disease because symptoms are either



not present or extremely vague. Typically, pancreatic cancer is diagnosed at an advanced stage when the patient has a poor prognosis with a low chance of survival. Adding CPI-613 to what is already considered one of the best treatment options available has the potential to improve treatment outcomes and boost survival in patients diagnosed with one of the most fatal forms of cancer.”

Robert Rodriguez, Cornerstone’s President and Chief Operating Officer, said, “The initiation of enrollment in the Phase I/II study is an exciting milestone in the clinical development of CPI-613, our lead AEMD product candidate. This is a truly unique cancer bioenergetics directed agent, which, in pre-clinical studies, has consistently demonstrated an ability to significantly disrupt the metabolic and regulatory process required for cell growth in solid tumor lines. We look forward to working with Dr. Alistar on this Phase I/II study to evaluate the combined safety and activity of CPI-613 when combined with a modified FLOFIRINOX chemotherapeutic regimen, as we work toward extending the duration and quality of life of those suffering from metastatic pancreatic cancer with good performance status.”

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>