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Cornerstone Pharmaceuticals Announces Initiation of Phase I/II Clinical Trial of CPI-613 for the Treatment of Advanced Bile Duct Cancers

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

CRANBURY, NEW JERSEY (August 8, 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 I/II clinical trial of CPI-613 for the treatment of patients with advanced or metastatic cholangiocarcinoma (bile duct cancer) that cannot be removed by surgery. 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.

Bile duct cancer starts in the bile duct, a thin tube that reaches from the liver to the small intestines. Approximately 3,000 Americans develop bile duct cancer each year, with the majority of cases being found during the late stages of the disease after it has spread too far to be completely removed by surgery.¹ The most common symptom of the disease is jaundice, a yellowing of the skin and eyes, which is caused by a blocked bile duct.

The interventional, open-label clinical study, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, is expected to enroll approximately 22-40 patients with advanced or metastatic bile duct cancers that cannot be removed by surgery and have failed current available therapies. In the week prior to the first course of treatment, patients participating will receive CPI-613, intravenously, for five days. Afterwards, patients will receive CPI-613 twice a week, as a single treatment agent, for three weeks of a four week cycle. 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.

Dr. Angela Alistar, MD, of Wake Forest Baptist, who serves as the Principal Investigator for this trial, remarked, “Most of the Bile duct cancers are usually diagnosed in advanced stages after the patient develops symptoms, which signals that the tumor has significantly grown to a point at which surgical removal may no longer be a treatment option for them. There is currently only one first line of systemic treatment for bile duct carcinoma which leaves unmet need for second line therapies. CPI-613 holds



considerable promise of being a novel and successful treatment option for these patients faced with extremely poor survival outcomes.”

Robert Rodriguez, Cornerstone’s President and Chief Operating Officer, said, “In previous pre-clinical studies, CPI-613 has consistently demonstrated its ability to significantly disrupt the metabolic and regulatory processes required for cell growth in solid tumor cell lines. We look forward to working with Dr. Alistar on this Phase I/II study to evaluate the efficacy and safety of CPI-613 in patients suffering from bile duct cancers, with the potential of providing these individuals with an alternative treatment option for this rare and often catastrophic disease.”

CPI-613 induces cancer-specific inhibition of pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH), key mitochondrial enzymes involved in cancer cell metabolism. Disruption of PDH and KGDH function cuts off the tumor’s energy supply and starves the cancer cell, 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.

CPI-613 is the lead drug candidate from Cornerstone's proprietary AEMD platform. The AEMD drug platform disrupts the 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.

ⁱ **American Cancer Society.** "Bile Duct Cancer (Cholangiocarcinoma)" Last accessed October 9, 2013. Available at: <http://www.cancer.org/acs/groups/cid/documents/webcontent/003084-pdf.pdf>