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Cornerstone Pharmaceuticals Initiates Phase I Clinical Trial of CPI-613 for the Treatment of Metastatic Colorectal Cancer

Trial sponsored by prominent National Cancer Institute-designated Comprehensive Cancer Center

CRANBURY, NEW JERSEY (January 12, 2015) – Cornerstone Pharmaceuticals, Inc., a clinical stage company and leader in the field of cancer metabolism-based therapeutics, today announced the initiation of a Phase I clinical trial of CPI-613, in combination with fluorouracil (5-FU), for patients with non-resectable metastatic colorectal cancer who have failed prior therapy. CPI-613 is the company's lead Altered Energy Metabolism Directed (AEMD) drug candidate, designed to disrupt the altered energy-production pathways in cancer cells by targeting their altered mitochondrial metabolism.

Encouraging results and patient benefit observed in earlier trials including patients diagnosed with metastatic colorectal cancer support further evaluation of CPI-613 in this indication. The safety profile of CPI-613 in earlier trials, which shows the drug to be well tolerated, provides further support for evaluation of CPI-613 in combination with other drugs to maximize benefit.

Worldwide, colorectal cancer is the third most common cancer in men and the second in women and is diagnosed in nearly 1.3 million people annually, resulting in nearly 700,000 deaths each year.ⁱ Furthermore, most colorectal cancer patients have non-resectable tumors – meaning the tumors are unable to be removed by surgery – and about 50% of patients develop metastases.ⁱⁱ

The single site study, sponsored by Wake Forest Baptist Medical Center and in collaboration with the National Cancer Institute (NCI), is a Phase I, open label, dose-escalating study designed to determine the maximum tolerated dose of CPI-613 in combination with 5-FU. The study will also assess as secondary endpoints the pharmacokinetics, safety and efficacy of various doses of CPI-613 when used in combination with 5-FU. Overall response rates, progression-free survival (PFS) and disease control rates will also be measured. 5-FU has been used as a standard chemotherapeutic agent in the treatment of colon, rectum, and head and neck cancers with commonly accepted response rates of less than 20% and acquired drug resistance.ⁱⁱⁱ



Steve Carchedi, Cornerstone’s Chief Executive Officer, said, “We believe by working with the investigators at Wake Forest Baptist Medical Center in this trial, which will be supported by the National Cancer Institute, we will be able to further demonstrate the capabilities of our lead product candidate, CPI-613, and mark another important step forward for Cornerstone. The study is the sixth evaluating CPI-613 in solid tumors and highlights the broad potential utility of our unique cancer metabolism approach in both solid tumors and hematologic malignancies. We look forward to the results of this study as we work to enhance data for CPI-613 and are excited about the efficacy demonstrated by our AEMD approach in both hematological and solid tumors thus far.”

Angela Alistar, MD, of Wake Forest Baptist Medical Center and the Principal Investigator for the trial, remarked, “The high incidence of non-resectable, or un-removable, colorectal cancer tumors along with the high percentage of metastasis observed with this disease certainly point to a need for more effective therapies. We anticipate that the combination of CPI-613 and 5-FU may provide a viable treatment option for colorectal cancer patients and look forward to sharing results of the study once available.”

CPI-613 is the lead drug candidate from Cornerstone’s proprietary AEMD platform. Cornerstone’s AEMD drug platform disrupts the metabolism (energy producing) pathways that support the growth and development of many types of cancer cells. CPI-613 has been shown in-vitro to be highly selective in inducing the simultaneous inhibition of two key mitochondrial enzymes involved in cancer cell metabolism: pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH). Disruption of PDH and KGDH function cuts off the tumor’s mitochondrial energy supply, culminating in cell death. CPI-613 is currently being evaluated in several Phase I, I/II and II human clinical trials in solid tumors and hematological malignancies.

About Wake Forest Baptist Medical Center

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 clinical stage, oncology-focused pharmaceutical company committed to the development and commercialization of therapies that exploit the metabolic differences between normal cells and cancer cells. The company’s primary objective is to develop highly selective and effective agents with minimal toxic effects on normal cells and tissues. The company’s unique approach to targeting cancer metabolism has led to two distinct technology platforms: altered energy metabolism directed, or AEMD, compounds and an Emulsiphan lipid nanoemulsion based drug delivery system.



Currently, Cornerstone's deep oncology portfolio of clinical trials evaluating CPI-613 at notable institutions includes ongoing studies in both hematologic malignancies and in solid tumors. Overall, CPI-613 has been shown to have broad utility in both solid tumors and hematologic malignancies.

For more information, visit www.cornerstonepharma.com.

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

References

ⁱ **International Agency for Research on Cancer.** "Colorectal Cancer: Estimated incidence, mortality and prevalence worldwide in 2012." Available at: http://globocan.iarc.fr/Pages/fact_sheets_cancer.aspx

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