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Cornerstone Pharmaceuticals Initiates Phase I Clinical Trial of CPI-613 for the Treatment of Acute Myelogenous Leukemia

Proprietary Anticancer Compound Selected for Best of ASCO 2013

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

CRANBURY, NEW JERSEY (August 12, 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 human clinical trial, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, to evaluate the tolerability, safety, maximum tolerated dose (MTD), pharmacokinetics, and anti-tumor activities of Cornerstone's lead Altered Energy Metabolism Directed (AEMD) drug candidate CPI-613 administered in combination with high dose cytarabine and mitoxantrone in relapsed or refractory acute myelogenous leukemia (AML) patients. The trial has been launched following a recently completed Phase I single agent trial of CPI-613 in patients with hematological malignancies similarly sponsored by Wake Forest Baptist.

"We are very encouraged by the tolerability and signals of activity seen in AML and other patients in the earlier Phase I study," said Dr. Timothy Pardee, MD, Ph.D. of Wake Forest Baptist, who also serves as the Principal Investigator for this trial. "We look forward to further evaluating CPI-613 in the early relapsed/refractory AML patient setting when administered in combination with a standard chemotherapeutic regimen with the hope of improving the outcomes and the quality of life for these patients through the combined use of this mechanistically novel agent."

Phase I, Single Agent Study in Hematologic Malignancies

The completed Phase I single agent study in hematologic malignancies enrolled a total of 26 patients afflicted with a variety of hematologic malignancies, 21 of whom were evaluable at the conclusion of the study. In addition to identifying the MTD of CPI-613 when dosed twice weekly for three consecutive weeks followed by a week of rest, a favorable tolerability profile was observed in patients

of both genders ranging in age from 19 to 81 years old at doses at or below the MTD and, notably, without inducing myelosuppression. Significant signals of activity were observed in several patients, including achievement of a morphologic leukemia free state in a refractory AML patient. The results from this study were recently presented by Dr. Pardee at the 2013 American Society for Clinical Oncology (ASCO) Annual Meeting and was hand-selected for inclusion in the Best of ASCO 2013 educational series.

"We are very encouraged by the results of the Phase I study sponsored by Wake Forest Baptist and are delighted to continue our work with Wake Forest Baptist and its world class team of investigators" said Robert Rodriguez, Cornerstone's President and Chief Operating Officer. "The tolerability profile demonstrated by CPI-613 thus far may be of particular benefit to the majority of elderly AML patients who often are precluded from receiving available therapies due to their toxicities. We look forward to the possibility of offering a novel, better tolerated, therapeutic alternative treatment for AML patients".

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.