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Cornerstone Pharmaceuticals Initiates Phase II Clinical Trial of CPI-613 for the Treatment of Myelodysplastic Syndrome

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

CRANBURY, NEW JERSEY (September 25, 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 II clinical trial of CPI-613, its lead Altered Energy Metabolism Directed (AEMD) drug candidate. The pilot study, sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center, will evaluate the overall response rate for CPI-613 in patients with myelodysplastic syndrome (MDS) who have been unresponsive to previous therapies. Secondary endpoints include overall survival, progression-free survival, safety, and transfusion frequency.

Earlier this year, the Company announced the initiation of a Wake Forest Baptist-sponsored Phase I trial investigating the combination of CPI-613 with high dose cytarabine and mitoxantrone in relapsed or refractory acute myelogenous leukemia (AML) patients. Both of these trials have been launched following the encouraging results of a recently completed Phase I single agent trial of CPI-613 in patients with hematological malignancies similarly sponsored by CCCWFU. The results from this study were 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.

“The signals of activity and the tolerability profile demonstrated by CPI-613 in the earlier Phase I study have motivated us to move forward in the clinic with this novel compound,” 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 treatment-resistant MDS patients, with the hope of improving their outcomes and quality of life.”

“It has been a special honor to continue working with Wake Forest Baptist and its world class team of investigators,” said Robert Rodriguez, Cornerstone’s President and Chief Operating Officer. “In the earlier Phase 1 study we observed complete remission or stable disease in several relapsed/refractory



MDS patients. We look forward to the possibility of extending these encouraging results and offering a novel, better-tolerated, therapeutic alternative treatment for MDS patients.”

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 pathways that support, among other things, cancer cell growth and development. The platform is designed to produce drugs, such as the company's lead drug CPI-613, that disrupt energy-production pathways whose organization or regulation are altered specifically in cancer cells. CPI- 613 is currently being evaluated in Phase I, I/II and Phase II trials.

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.