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### **Cornerstone Pharmaceuticals to Present CPI-613 Phase I Results at 2013 American Society of Hematology Annual Meeting**

*Study on First-in-Class Agent in Hematologic Malignancies Sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center*

**CRANBURY, NEW JERSEY (November 11, 2013)** – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced that data from a Phase I clinical study of CPI-613 for the treatment of advanced hematological malignancies will be presented by the principal investigator, Timothy S. Pardee, MD, of the Comprehensive Center of Wake Forest Baptist Medical Center, at the 2013 American Society of Hematology (ASH) Annual Meeting and Exposition, to be held December 7<sup>th</sup>-10<sup>th</sup>, 2013, in New Orleans, LA.

The Phase I open-label, dose-escalation study, sponsored by Wake Forest Baptist, was designed to determine the maximum-tolerated dose, pharmacokinetics, safety and efficacy of CPI-613 given as a single agent in patients diagnosed with advanced hematologic malignancies.

- Title:** A Phase I study of the safety, efficacy and pharmacokinetics of the first in class pyruvate dehydrogenase complex inhibitor CPI-613 in patients with advanced hematologic malignancies
- Authors:** Timothy S. Pardee MD, PhD, King Lee PhD, RAC, John Luddy MS, Claudia Maturo MS, Robert Rodriguez BSc, MBA, Denise Levitan MD, David Hurd MD, Leslie R. Ellis MD, Scott Isom MS, Robin Harrelson BSN, Megan Manuel MSN, Sarah Dralle MSN, Susan Lyerly PA-C and Bayard L. Powell MD
- Session:** 604. Molecular Pharmacology, Drug Resistance: Molecular Mechanisms of Drug Resistance and Novel Targets in Acute Myeloid Leukemia; Paper Number 486
- Date & Time:** Monday, December 9<sup>th</sup>, 2013; 2:45 PM C.T.



CPI-613 is a first-in-class agent that 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.

Robert Rodriguez, Cornerstone's President and Chief Operating Officer, commented, "I believe the selection of Dr. Pardee's Phase I study results for oral presentation at ASH represents solid recognition of the potential value of CPI-613 in the hematologic malignancies setting as well as the scientific approach embodied in our AEMD compounds. Cornerstone is committed to bringing innovative cancer metabolism targeted therapies to patients with hematologic cancers, as well as other cancers, and we continue to be very encouraged by the data arising from our clinical studies of CPI-613."

CPI-613 is the lead drug candidate from Cornerstone's proprietary Altered Energy Metabolism Directed (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](http://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](http://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.