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CORNERSTONE PHARMACEUTICALS PRESENTS DATA AT 2013 ANNUAL ASCO MEETING

Proprietary anticancer compound CPI-613 selected for Best of ASCO 2013

Study by Comprehensive Cancer Center of Wake Forest Baptist Medical Center demonstrates anti-cancer activities of CPI-613 in variety of hematologic malignancies

CRANBURY, NEW JERSEY (June 3, 2013)– Cornerstone Pharmaceuticals, Inc., (www.cornerstonepharma.com), a leader in the growing field of cancer metabolism-based therapeutics, today announced data from a Phase I clinical trial of proprietary first-in-class anticancer compound CPI-613 in patients with advanced hematologic malignancies. The data will be presented at the 2013 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago, Illinois by Dr. Timothy S. Pardee the Principal Investigator for the trial sponsored by Comprehensive Cancer Center of Wake Forest Baptist Medical Center.

In addition to being selected for a discussion session at the annual meeting, Dr. Pardee's poster has been hand-selected for inclusion in the Best of ASCO educational series. The series is a two-day condensed presentation of the highlights of the annual ASCO meeting held in three major US metropolitan centers as well as at numerous international locations to reach oncologists unable to attend the annual meeting. Posters are presented at this series only after meeting specific criteria regarding relevant and significant research in oncology today. The dates and locations of 2013's Best of ASCO in the US are: Chicago, August 9-10; Los Angeles, August 16-17; and Boston, August 23-24 as well as several locations internationally.

The trial was designed to determine the maximum tolerated dose (MTD), safety, and anti-cancer activities of CPI-613 given as a single agent by IV infusion. A total of 21 evaluable patients with advanced relapsed or refractory hematologic malignancies were administered CPI-613 over a two-hour infusion on days one and four for three weeks every 28 days with a starting dose of 420 mg/m². Treatment could be continued if the patient experienced clinical benefit. Dose escalation was performed

in six cohorts to a final dose of 3780 mg/m², defining this dose as above the MTD. A total of six patients were treated at a dose of 2940 mg/m² over two hours with no dose limiting toxicities observed, establishing this as the MTD. Of the 21 patients evaluable for a response, eight achieved a response of stable disease or better for a disease control rate of 38%. Responses included a complete remission maintained over 23 cycles in a myelodysplasia syndrome patient and achievement of a morphologic leukemia free state in an AML patient. Sustained partial response in both a Burkitt's and a cutaneous T-cell lymphoma patient maintained over 16 and 15 cycles respectively. Additionally, stable disease was observed in two multiple myeloma and two myelodysplasia patients.

"We are very encouraged by the tolerability and signals of activity seen in several patients in this Phase I study for whom there is no available therapy shown to provide clinical benefit," said Dr. Pardee. "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 as well as in early relapsed or refractory MDS patients with the hope of improving the outcomes and the quality-of-life for these patients through the combined use of this mechanistically novel agent."

The details of the poster presentation are as follows:

"A phase I study of the first-in-class mitochondrial metabolism inhibitor CPI-613 in patients with advanced hematologic malignancies." Abstract # 2516, Poster Board # 4. Presenter: Timothy S. Pardee, M.D., Ph.D. Presented June 4, 2013, 8:00 AM to 12:00 PM, McCormick Place, E450A.

Dr. Robert Shorr, Chief Executive Officer of Cornerstone, stated, "We are very pleased with the successful results reached by our colleagues at Wake Forest University. It is a significant clinical advancement to have determined CPI-613's MTD, and having a 38% disease control rate in a Phase I clinical trial is especially satisfying. We wish to congratulate Wake Forest Baptist and Dr. Pardee for the quality of work they have performed, and it is a special honor for Dr. Pardee's work with CPI-613 to be included in ASCO's select Best of ASCO presentations."

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 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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