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CORNERSTONE PHARMACEUTICALS CLINICAL DATA ON CPI-613 TO BE PRESENTED AT ASCO 2014

AML patients demonstrated positive outcomes in CPI-613 combination clinical trial

CRANBURY, NEW JERSEY (May 14, 2014) – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced that data from a Phase I clinical trial of its proprietary first-in-class anticancer compound CPI-613 in patients with relapsed or refractory acute myeloid leukemia (AML) will be presented during a Poster Highlights Session at the American Society of Clinical Oncology (ASCO) Annual Meeting at McCormick Place, Chicago, IL, on May 31, 2014. The poster will be presented by Dr. Timothy S. Pardee, the Principal Investigator for the trial, which is sponsored by the Comprehensive Cancer Center of Wake Forest Baptist Medical Center.

The study data show that CPI-613 in combination with high dose Ara-C (HDAC) and mitoxantrone is a promising salvage therapy regimen, especially in older patients and those with high risk disease.

The abstract and poster information and timing of the presentation is provided below.

- **A phase I study of the mitochondrial metabolism inhibitor CPI-613 in combination with high dose Ara-C (HDAC) and mitoxantrone for relapsed or refractory acute myeloid leukemia (AML)**

Abstract:	#7028
Poster:	#20
First / Presenting Author:	Timothy Pardee, MD, Wake Forest Baptist Medical Center
General Poster Session:	Leukemia, Myelodysplasia, and Transplantation
Date, Location:	Saturday, May 31, 1:15 PM - 4:15 PM; S Hall 405

The information provided in the abstract reflects data from an ongoing dose escalation study and reports on 24 AML patients between the ages of 21 and 76, studying the safety and efficacy of CPI-613 in combination with high dose Ara-C (HDAC) and mitoxantrone. The study showed promising results with patients achieving a complete remission (CR/CRi) rate of 54% overall and 55% in patients 60 years or older. In particular, the CR/CRi rate was 53% in patients with poor risk cytogenetics, compared to only 25% in a historical cohort of patients treated with HDAC, mitoxantrone and asparaginase.

Dr. Pardee commented, “We are very encouraged by CPI-613 as a combination therapy for relapsed or refractory AML given the results demonstrated in this recent Phase I study. AML is the most common form of adult leukemia, especially with patients 60 years of age or older. Standard therapy for AML is

quite toxic, which precludes optimal treatment in many cases, particularly for the elderly who are generally less able to tolerate such treatment. We believe that by using CPI-613 in combination with HDAC and mitoxantrone, we can provide an effective treatment with acceptable levels of toxicity making it a viable option for a larger population of AML patients."

Dr. Robert Shorr, Chief Executive Officer of Cornerstone, said, "The data included in the poster presentation by Dr. Pardee at this year's ASCO meeting are a reflection of the clinical progress we have made to advance the strategic development plan for CPI-613 in cancers with unmet medical needs, such as AML. Our goal is to continue the clinical advancement of CPI-613 towards regulatory approval. The results achieved by Dr. Pardee and his team at Wake Forest Baptist are a great example of how the agents arising from our Altered Energy Metabolism Directed (AEMD) compound platform can target the reconfigured metabolism and bioenergetics of cancer in a selective manner, while also showing its versatility as a combination therapy."

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 and other molecules 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. CPI- 613 is currently being evaluated in Phase I, I/II and II clinical trials.

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 differences between normal cells and cancer cells. The company's primary objective is to produce 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. 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.