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Cornerstone Pharmaceuticals Announces Positive Phase I Data for CPI-613 in Relapsed or Refractory AML Patients to be Presented at 2014 ASH Annual Meeting

Study demonstrates high degree of activity of CPI–613 in difficult to treat patients when given in combination with HiDAC and mitoxantrone

CRANBURY, NEW JERSEY (November 6, 2014) – Cornerstone Pharmaceuticals, Inc., a development stage company and leader in the growing field of cancer metabolism-based therapeutics, today announced positive data from an ongoing Phase I study evaluating lead compound CPI-613 given in combination with high dose Ara-C, or cytarabine, (HiDAC) and mitoxantrone in patients with relapsed or refractory acute myeloid leukemia (AML). Results from the study, which is sponsored by Wake Forest Baptist Medical Center, will be presented at the 56th American Society of Hematology (ASH) Annual Meeting, being held December 6-9, 2014 in San Francisco, California.

CPI-613 is the company's lead Altered Energy Metabolism Directed (AEMD) drug candidate, a first-in-class anticancer compound designed to disrupt the altered energy-production pathways in cancer cells by targeting mitochondrial metabolism.

Steve Carchedi, Chief Executive Officer of Cornerstone Pharmaceuticals, commented, "Data from our ongoing AML salvage study further demonstrate the high degree of activity of CPI-613 in advanced hematologic malignancies and the continued progress we've made in its development. We are encouraged by these clinical responses and look forward to sharing further analysis of the trial evaluating this novel drug as a salvage regimen in older and high risk patients to meet this huge unmet medical need."

Mr. Carchedi continued, "Our successes continue to validate cancer metabolism as a unique approach to cancer treatment. This is an area that has experienced growing interest in recent years as seen by the several companies targeting cancer metabolism, including Agios. However, Cornerstone continues to demonstrate its leadership in the cancer metabolism-based therapy field as we are the farthest along in active clinical development with over 180 patients having been enrolled across 11 studies to date, including ongoing Phase II trials."

The Phase I trial was designed to determine the maximum tolerated dose (MTD), safety, and efficacy of CPI-613 given in combination with HiDAC and mitoxantrone in patients with relapsed or refractory AML. In the study, patients were given escalating doses of CPI-613 daily for five consecutive days at a

starting dose of 500mg/m^2. On day three patients were also started on a dose of HiDAC (3,000 mg/m^2 or 1,500 mg/m^2 for patients 60 years of age or older) given every 12 hours for five doses as well as three doses of mitoxantrone (6 mg/m^2) given daily.

The study findings, poster information and timing of the presentation are provided below.

Publication #3744: The Mitochondrial Metabolism Inhibitor CPI-613 is Highly Active in Combination with High Dose Ara-C (HiDAC) and Mitoxantrone in a Phase I Study for Relapsed or Refractory Acute Myeloid Leukemia (AML)

- Session Name: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster III
- Date: Monday, December 8, 2014
- Presentation Time: 6:00 PM - 8:00 PM
- Location: Moscone Center, North Building, Hall E

Overall, the regimen was found to be well tolerated. The information provided in the abstract reports on 36 patients, with median age of 60. Eight patients had refractory disease and nine received two or more previous lines of therapy.

In patients with relapsed disease, where data were available, the median duration of complete response was 8.4 months. The study showed promising results with patients in the intent to treat group achieving complete remission or complete remission with incomplete blood count recovery (CR/CRi) response rate of 50% (16 CR and 2 CRi; total 18 of 36 patients) and patients 60 years or older also achieving CR/CRi response rate of 50% (10 of 20 patients). In particular, the CR/CRi rate was higher, 53%, in patients with poor risk cytogenetics, compared to only 25% in a historical cohort of patients treated with HDAC, mitoxantrone and asparaginase. Additionally, nine patients (25%) went on to allogeneic stem cell transportation which compared favorably with the 17% recorded in historical data.

Median survival for the entire cohort was 6.4 months, with a median of 3.1 months of follow-up. Five patients (14%) died on or before day 30 and were not evaluable. The study showed that the combination regimen is well tolerated with no dose limiting toxicities observed to date.

“Our observance of high response rates in patients with poor risk cytogenetics and the elderly, groups which typically comprise some of the most difficult to treat cases, highlight the promise in CPI-613’s unique mechanism of action and its potential as a first-in-class drug,” said Timothy Pardee, M.D., Ph.D., Director of Leukemia Translational Research at Wake Forest Baptist Medical Center and Principal Investigator of the study. “The potential of this regimen to offer an effective treatment and be a viable option for a larger population of AML patients is encouraging.”

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 Wake Forest Baptist Medical Center

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 CPI-613

CPI-613 is the lead drug candidate from Cornerstone's proprietary AEMD platform. Cornerstone's AEMD drug platform disrupts the essential "bioenergetic" differences that support the growth and development of many types of cancer cells. In the case of CPI-613, the compound has been shown to selectively induce inhibition of pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH), key mitochondrial enzymes involved in cancer cell metabolism in-vitro. Disruption of PDH and KGDH function cuts off the tumor's energy supply, culminating in cell death. CPI-613 is currently being evaluated in Phase I, I/II and II human clinical trials in solid tumors and hematological malignancies.

About Cornerstone Pharmaceuticals

Cornerstone Pharmaceuticals, Inc. is a privately held clinical stage, oncology-focused pharmaceutical company committed to the development and commercialization of therapies that exploit the metabolic differences between normal cells and cancer cells. The company's primary objective is to develop 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 independent technology platforms: altered energy metabolism directed, or AEMD, compounds and an Emulsiphan lipid nanoemulsion based drug delivery system. www.cornerstonepharma.com.

Safe Harbor Statement

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.