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Cornerstone Pharmaceuticals, Inc. Presents Phase I Data on CPI-613 in Acute Myeloid Leukemia (AML) and T-Cell Non-Hodgkin's Lymphoma (T-cell NHL) at ASH 2016

First Reported Studies of CPI-613-based Combination Therapy in these Difficult-to-Treat Malignancies Show Encouraging Efficacy and Safety Results

Cranbury, New Jersey, December 5, 2016 -- (PR NEWSWIRE) -- Cornerstone Pharmaceuticals, Inc., a privately held, clinical-stage, oncology-focused pharmaceutical company, today presented data from two Phase I trials evaluating its lead compound, CPI-613, in poster sessions at the 58th annual meeting of the American Society of Hematology (ASH) in San Diego, California. The two datasets show encouraging efficacy and safety results in patients with acute myeloid leukemia (AML) and T-cell non-Hodgkin's lymphoma (T-cell NHL), respectively, supporting the clinical rationale for combining CPI-613 with standard chemotherapy in two hematologic malignancies that have historically been difficult to treat.

CPI-613 is Cornerstone Pharmaceuticals' 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. It selectively targets the mitochondrial tricarboxylic acid (TCA) cycle in cancer cells, an indispensable process essential to cell multiplication and survival. The two datasets presented at ASH are from the first reported studies of CPI-613-based combination therapy in patients with AML and T-cell NHL.

"Our ASH data presentations underscore the viability of targeting the TCA cycle as a novel approach to anticancer treatment," said Howard Jonas, Chairman of the Board, Cornerstone Pharmaceuticals. "The results suggest that blood-based disorders such as acute myeloid leukemia and T-cell non-Hodgkin's lymphoma are amenable to combination therapy with CPI-613, potentially filling unmet medical needs in these underserved patient populations."

AML Data

In the first poster presentation, researchers presented data from an open-label Phase I dose-escalation trial designed to determine the maximum tolerated dose (MTD), safety, and efficacy of CPI-613 administered intravenously in combination with high-dose cytarabine (HiDAC) and mitoxantrone in patients with relapsed or refractory AML. A total of 67

patients (median age of 60 years, with ages ranging from 21-79 years) were enrolled, of whom 62 were ultimately evaluable. The overall response rate (ORR) was 50%, including 26 patients in complete remission (CR) and 5 in CR with incomplete blood count recovery (CRi); median survival was 6.7 months. Response was significantly associated with survival, with a median survival of 13.2 months in responders versus 3 months for all others.

Among 24 patients with poor-risk cytogenetics, the ORR was 46% (9 CR + 2 CRi), with a median survival of 5.5 months. The researchers described this result as "surprising," given data from a historical cohort of patients treated with HDAC, mitoxantrone, and asparaginase, in which only 19% (3/16) of patients with poor-risk cytogenetics responded, with a median survival of 2.8 months (p=0.0571 for the comparison between CPI-613-based combination therapy and the historical cohort). Among 15 elderly (age ≥60 years) patients with poor-risk cytogenetics, CPI-613-based combination therapy was associated with a significant improvement in overall survival, compared to the historical cohort (p=0.0359).

The most common toxicities in patients receiving CPI-613-based combination therapy were diarrhea and nausea. Thirteen patients (21%) went on to receive allogeneic stem cell transplantation.

"Combination therapy with CPI-613 is a novel approach to the treatment of refractory acute myeloid leukemia, especially in elderly patients and those with poor-risk cytogenetics," commented lead investigator Timothy S. Pardee, MD, PhD, Associate Professor, Internal Medicine, in the Section on Hematology and Oncology at the Comprehensive Cancer Center of Wake Forest Baptist Medical Center in Winston-Salem, North Carolina, Chief Oncologist, Cornerstone. "It appears to improve overall survival without increasing the toxicity of high-dose cytarabine, and mitoxantrone. In addition to its direct effects on leukemia cells, inhibition of the TCA cycle may alter immune responses in the tumor microenvironment. Additionally, the availability of gene expression profiling and other technologies may help identify patients likely to respond to CPI-613, a benefit that may facilitate targeting of metabolic pathways as a logical step in the evolution of anticancer therapy."

T-cell NHL Data

In the second poster presentation, researchers presented data from a Phase I, open-label, modified 3+3 dose-escalation trial evaluating the combination of CPI-613 and bendamustine in patients with relapsed/refractory T-cell NHL. CPI-613 was given at escalating doses starting at 2,000 mg/m^2 over two hours on days 1-4 of the study, and on days 8, 11, 15, and 18. Bendamustine was infused at 90 mg/m^2 on days 4 and 5 of each four-week treatment cycle; the treatment cycles were repeated up to six cycles. There was no intra-patient dose escalation.

As of October 21, 2016, eight patients had been dosed; all eight were evaluable for safety and six were evaluable for efficacy. The most common grade 3 or higher toxicities, lymphopenia and neutropenia, occurred in four patients. The protocol was later amended

to discontinue dose escalation at doses of 2,750 mg/m^2 or higher for dose-limiting toxicities and to expand the 2,550 mg/m^2 cohort.

The ORR was 83%. Three patients with peripheral T-cell lymphoma not otherwise specified (NOS) attained a complete response. Two patients – one with mycosis fungoides and one with angioimmunoblastic T-cell lymphoma – had a partial response. One patient with T-cell acute lymphoblastic leukemia experienced progressive disease.

"There is no standard treatment for relapsed or refractory T-cell lymphoma, highlighting an unmet clinical need among patients living with this devastating group of hematologic malignancies," noted lead investigator Zanetta S. Lamar, MD, Assistant Professor of Hematology/Oncology at Wake Forest Baptist Medical Center in Winston-Salem, North Carolina. "This first reported study of CPI-613 in combination with bendamustine in patients with relapsed or refractory T-cell lymphoma showed a good safety profile and an overall response rate exceeding 80%. Although the numbers are small, these results warrant continued investigation of this novel combination in this poor-risk patient population."

About Cornerstone Pharmaceuticals, Inc.

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. Cornerstone's primary objective is to develop highly selective and effective agents with minimal toxic effects on normal cells and tissues. Cornerstone's first-in-class clinical lead compound, CPI-613 is being evaluated in multiple Phase I, I/II, and II clinical studies. The U.S. Food and Drug Administration (FDA) has designated CPI-613 an orphan drug for the treatment of acute myeloid leukemia (AML), pancreatic cancer and myelodysplastic syndromes (MDS). The company's investors include IDT Corporation (NYSE: IDT). For more information, visit: www.cornerstonepharma.com.

Safe Harbor Statement

This press 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

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