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# FDA Provides Cornerstone Pharmaceuticals Approval to Initiate Pivotal Study for CPI-613, its Metabolism-Directed Anticancer Compound

## Successful End-of-Phase I Type B Meetings with FDA Clears Way for Pivotal Trials in Acute Myeloid Leukemia (AML) and Pancreatic Cancer

CRANBURY, N.J., March 23, 2017 /PRNewswire/ -- Cornerstone Pharmaceuticals, Inc., a clinical-stage, oncology-focused pharmaceutical company, announced a major milestone today. Following successful End-of-Phase I (EOP1) Type B meetings with the U.S. Food and Drug Administration (FDA), the Company has been given a clear clinical and regulatory path forward to conduct pivotal trials of its lead compound, CPI-613, in patients with acute myeloid leukemia (AML) and pancreatic cancer.

CPI-613 is Cornerstone Pharmaceuticals' first-in-class anticancer compound from its proprietary Altered Energy Metabolism Directed (AEMD) platform. The drug is designed to disrupt the altered energy production pathways in cancer cells by targeting the mitochondrial tricarboxylic acid (TCA) cycle, an indispensable process essential to cell multiplication and survival. The FDA has designated CPI-613 an orphan drug for the treatment of acute myeloid leukemia (AML), pancreatic cancer and myelodysplastic syndromes (MDS).

"Our productive dialogue with the FDA has cleared a path forward for initiating pivotal trials of CPI-613, which may be sufficient to support our proposed indications for the treatment of AML and pancreatic cancer," said Sanjeev Luther, Chief Operating Officer, Cornerstone Pharmaceuticals. "The FDA has provided valuable feedback to inform a design that will enhance the robustness of our trials. This would allow the Agency to assess them as registrational trials. We are confident in our ability to meet the FDA's requirements for the trials, and look forward to continued interaction with the FDA as the CPI-613 clinical development program advances."

During the EOP1 Type B meetings with the FDA, Cornerstone Pharmaceuticals presented updated results from Phase I trials of CPI-613 in patients with AML and pancreatic cancer.

"We believe the Phase II trial in AML can potentially lead to approval for treatment of a disease that historically has been extremely difficult to treat," said Jorge Cortes, M.D., lead investigator of the AML trials and Deputy Chair and Professor of Medicine in the Department of Leukemia at The University of Texas MD Anderson Cancer Center.

"We are encouraged that the FDA has granted us the opportunity to continue clinical trials in pancreatic cancer, a disease with large unmet clinical need, and are optimistic that we will continue to see positive results in advanced pancreatic cancer patients," commented Philip A. Philip, M.D., Ph.D., F.R.C.P., lead investigator of the pancreatic cancer trials and Professor of Oncology and Pharmacology, Leader, GI and

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Neuroendocrine Oncology, and Vice President of Medical Affairs at Karmanos Cancer Center, Wayne State University.

**About Cornerstone Pharmaceuticals, Inc.**

Cornerstone Pharmaceuticals, Inc. is a 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 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.

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