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### **Cornerstone Pharmaceuticals Receives FDA Orphan Drug Designation of CPI-613 for Treatment of Myelodysplastic Syndrome**

**CRANBURY, NEW JERSEY (October 3, 2013)** – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to CPI-613, the Company’s lead Altered Energy Metabolism Directed (AEMD) drug candidate, for the treatment of myelodysplastic syndrome (MDS).

MDS is a form of cancer that occurs when the blood-forming cells in the bone marrow are damaged, which leads to low numbers of one or more type of blood cells.<sup>1</sup> In about one-third of MDS cases, the disease progresses to a rapidly growing cancer of bone marrow cells called acute myeloid leukemia (AML). Currently no cure for MDS exists for patients with this life-threatening disease.

Robert Rodriguez, Cornerstone’s President and Chief Operating Officer, said, “Approximately 15,000 people are diagnosed with MDS in the United States each year. Treatment options for MDS patients are limited, especially for patients with relapsed or refractory disease, and are often associated with myelosuppressive side effects. We are encouraged by the lack of myelosuppression, good tolerability and signals of activity that were observed in MDS patients in the Phase I trial. We look forward to expanding our experience with CPI-613 as a monotherapy in the treatment of relapsed / refractory MDS patients in the recently launched Phase II MDS clinical trial as well as the possibility of establishing CPI-613 as an alternative treatment option for this rare and devastating disease.”

Orphan drug designation is granted by the FDA to therapeutics intended to treat rare diseases that affect fewer than 200,000 individuals in the United States. The designation entitles Cornerstone Pharmaceuticals to seven years of U.S. marketing exclusivity for CPI-613 upon regulatory approval, as well as an opportunity to apply for grant funding from the U.S. government to defray costs of clinical trial expenses, tax credits for clinical research expenses, and a potential waiver of the FDA’s application user fee. In addition to MDS, Cornerstone Pharmaceuticals has previously received orphan drug designation for the use of CPI-613 in the treatments of AML and pancreatic carcinoma.



CPI-613 induces cancer-specific inhibition of pyruvate dehydrogenase (PDH) and alpha ketoglutarate dehydrogenase (KGDH), key mitochondrial enzymes involved in cancer cell metabolism. Disruption of PDH and KGDH function leads to catastrophic disruption of tumor mitochondrial metabolism. As a result, tumor cells are starved of energy and biosynthetic intermediates, culminating in cell death. CPI-613 is currently being evaluated in Phase I, I/II and Phase II trials in hematologic malignancies and solid tumors. The results from the Phase I single agent trial were presented at the 2013 American Society for Clinical Oncology (ASCO) Annual Meeting and hand-selected for inclusion in the Best of ASCO 2013 educational series.

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 to energy that occur in many types of cancer cells. These essential "bioenergetic" differences are linked to critical pathways, particularly those that support cancer cell growth and development.

For more information on Cornerstone Pharmaceuticals and its technology, visit [www.cornerstonepharma.com](http://www.cornerstonepharma.com).

### **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](http://www.cornerstonepharma.com).

### **Forward-Looking Statements**

This release contains forward-looking statements. These statements relate to future events or each 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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### **References**



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<sup>i</sup> **American Cancer Society.** "What is myelodysplastic syndrome?" Last accessed October 1, 2013. Available at: <http://www.cancer.org/cancer/myelodysplasticsyndrome/detailedguide/myelodysplastic-syndromes-what-is-m-d-s>