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CORNERSTONE PHARMACEUTICALS ANNOUNCES FDA ORPHAN DRUG DESIGNATION OF CPI-613 FOR PATIENTS WITH ACUTE MYELOID LEUKEMIA

CRANBURY, NJ, September 6, 2011– Cornerstone Pharmaceuticals, Inc., (www.cornerstonepharma.com), 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 product candidate, for the treatment of acute myeloid leukemia (AML).

“Orphan drug designation of CPI-613 for AML significantly strengthens its value proposition and provides further support toward advancing this promising therapeutic in the clinic for AML patients who have very few treatment options,” said Robert Shorr, Ph.D., Chief Executive Officer of Cornerstone.

CPI-613 induces cancer-specific inhibition of pyruvate dehydrogenase (PDH), a key mitochondrial enzyme involved in cancer cell metabolism. Disruption of PDH function leads to catastrophic disruption of tumor mitochondrial metabolism. Tumor cells are thereby starved of energy and biosynthetic intermediates, culminating in cell death. CPI-613 is being evaluated in a Phase I trial in hematologic malignancies as well as in Phase I and I/II trials in solid tumors.

Orphan drug designation entitles Cornerstone Pharmaceuticals to seven years of marketing exclusivity for CPI-613 upon regulatory approval, 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. Orphan status is granted by the FDA to promote the development of new therapies for the treatment of diseases that affect fewer than 200,000 individuals in the United States.



CPI-613 is the lead candidate from Cornerstone's proprietary Altered Energy Metabolism Directed (AEMD) drug platform, which discovers drugs that disrupt biochemical alterations in the conversion of glucose to energy that occur in many types of cancer cells. CPI-613 is being studied at Wake Forest Baptist's Comprehensive Cancer Center to evaluate safety, tolerability, maximum tolerated dose, efficacy, and pharmacokinetics for patients with relapsed and refractory hematologic malignancies. As presented at ASCO earlier this year (<http://bit.ly/qrSLd7>) CPI-613, has activity against several acute leukemia cell lines and may have activity in patients with relapsed disease.

About Cancer Metabolism

Cancer cell metabolism is an exciting and promising area for the development of drugs to treat cancer. While it has been known for nearly a century that cancer cells have a unique metabolism, only recently has there been a broad and significant renewal of scientific interest focused on exploring this unique metabolic difference to facilitate the discovery and development of groundbreaking therapies. Unlike normal cell metabolism, cancer cell metabolism utilizes less oxygen and has different nutritional requirements to survive and proliferate. This metabolic difference is considered to be fundamental to the transformation of normal cells into cancer cells and is believed to be conserved in all cancers, including solid tumors, lymphoma and leukemia. By better understanding these cancer-specific metabolic processes, researchers in the field hope to find new drugs to revolutionize cancer treatment.

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

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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.