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Cornerstone Pharmaceuticals Lead Compound CPI-613 Demonstrates Ability to Disrupt Growth of Cancer Cells

Culture cell studies published in Cancer & Metabolism

CRANBURY, NEW JERSEY (March 12, 2014) – Cornerstone Pharmaceuticals, Inc., a leader in the growing field of cancer metabolism-based therapeutics, today announced that its first-in-class cancer metabolism targeted therapeutic, CPI-613, demonstrated the ability to inhibit tumor cell growth in the published studies. The research was conducted by Stony Brook University researchers and members of Cornerstone’s cancer metabolism research team and Scientific Advisory Board, Paul M. Bingham, PhD, and Zuzana Zachar, PhD.

Research by Drs. Bingham, Zachar and their colleagues at Stony Brook University has led to the development of technology to design drugs that disrupt cancer metabolism. Targeting cancer cell metabolism is a promising area for cancer chemotherapeutic development. In 2001, Cornerstone and Stony Brook researchers initiated a collaboration to evaluate the basic mechanisms of actions behind this class of agents. Clinical trials were initiated in 2008 and since then, the Cornerstone-sponsored clinical trials have progressed steadily, moving into Phase II trials in 2013.

Robert Shorr, CEO and Chief Scientific Officer of Cornerstone, stated, “The publication of this data for CPI-613 in a highly reputable journal such as *Cancer & Metabolism* offers additional validation of our science for the potential treatment of a wide variety of cancers. We have initiated seven clinical trials in the last year for CPI-613, including four Phase II studies, two Phase I/II studies, and one Phase I study. We are encouraged by the clinical progress achieved thus far and look forward to additional positive milestones in 2014.”

Results of cultured cell studies by the research team, titled, “A strategically designed small molecule attacks alpha-ketoglutarate dehydrogenase in tumor cells through a redox process,” are published in the March 2014 edition of *Cancer & Metabolism*.

Previously, in a paper published by the *Journal of Molecular Medicine* in 2011 Drs. Bingham and Zachar showed that CPI-613 attacked pyruvate dehydrogenase complex PDH by activation of regulatory kinases. In contrast, alpha-ketoglutarate complex KGDH is auto-regulated by oxidation-reduction (redox) processes. The new study reveals that CPI-613 shuts down KGDH completely by way of an artificial hyper-stimulation of this redox autoregulatory process, destroying the energy producing capacity of the cells.



Dr. Bingham, Associate Professor in the Department of Biochemistry and Cell Biology at Stony Brook University, commented, “We discovered that CPI-613 acts as a ‘cocktail of one,’ meaning the single agent kills cancer cells selectively by simultaneously attacking two crucial metabolic enzymes in cancer cells. Unlike other current anti-cancer agents, a unique feature of CPI-613 is its ability to attack tumor metabolism more robustly than most other single-target agents and be less vulnerable to evolved drug resistance.”

Dr. Bingham added that CPI-613’s two-pronged attack on this cancer cell cycle efficiently and selectively induces cancer cell death in a variety of cancers, including solid tumors and also in hematological malignancies such as leukemia and lymphoma. The research team hopes that by leveraging CPI-613’s mechanisms of action to attack at least two cancer metabolic targets simultaneously, this class of anti-cancer agents will form the foundation for the development of effective treatment for a variety of cancers by way of shutting down multiple cancer cell metabolic regulation targets.

Drs. Bingham and Zachar’s co-authors on the paper include: Shawn Stuart of Stony Brook University; Alexandra Schauble and Sunita Gupta, and Adam D. Kennedy and Brian R. Keppler of Metabolon, Inc.

CPI-613 is the lead drug candidate from Cornerstone's proprietary Altered Energy Metabolism Directed (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.

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.

About Stony Brook Medicine

Stony Brook Medicine integrates and elevates all of Stony Brook University’s health-related initiatives: education, research and patient care. It includes five Health Sciences schools — Dental Medicine, Health Technology and Management, Medicine, Nursing and Social Welfare — as well as Stony Brook University Hospital and more than 50 community-based healthcare settings throughout Suffolk County. To learn more, visit www.stonybrookmedicine.edu.

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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.