

Agios Publication Identifies First Potent Inhibitors of Mutant IDH1 that Lower Tumor Oncometabolite

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Important step toward potential first-in-class therapeutic for cancer patients

Cambridge, Mass. – October 11, 2012 – Agios Pharmaceuticals, the leading biopharmaceutical company focused on discovering and developing novel drugs in the fields of cancer metabolism and rare metabolic genetic diseases, announced today a recent publication that highlights the discovery of the first potent inhibitors of mutant isocitrate dehydrogenase (IDH) that lower tumor 2-HG *in vivo*. The article “Discovery of the First Potent Inhibitors of Mutant IDH1 That Lower Tumor 2-HG *in Vivo*” was published in *American Chemical Society Medicinal Chemistry Letters* online on September 17, 2012 and in the October 2012 issue.

“IDH1 and IDH2 remain our lead programs in our cancer metabolism portfolio,” stated Scott Biller, Ph.D., chief scientific officer at Agios. “We are very excited about the promise of this first-in-class program and look forward to advancing into clinical studies in the near future, bringing us an important step closer to our ultimate goal of transforming the lives of cancer patients.”

In the article, Agios scientists reported the first inhibitors of mutant IDH1 that show good potency in a glioblastoma cell line harboring IDH1 mutation and achieve robust tumor 2-HG inhibition in the tumor xenograft model. High levels of the oncometabolite 2-HG have been shown to alter the epigenetic state and biology of cells, and these inhibitors will help assess the biological consequences of IDH mutations and the potential of IDH inhibitors for treating IDH mutant tumors. The findings of this publication were first disclosed in August at the 2012 Medicinal Chemistry Gordon Conference in the first session in the history of the conference on metabolic cancer targets, which was chaired by Agios associate director of chemistry and lead author Janeta Popovici-Muller, Ph.D.

The connection between cancer and metabolism has been the focus of scientists at Agios, who were the first to identify the activity of IDH1 mutations and the production of the oncometabolite 2-HG in research published in *Nature* in 2009. These insights revealed the potential of IDH1 mutations as a therapeutic target in cancer. The IDH gene mutation was initially discovered in brain cancers in 2008 by researchers at Johns Hopkins and more recently has also been linked to acute myelogenous leukemia (AML), one of the most common types of leukemia in adults, as well as other cancers.

About Agios Pharmaceuticals

Agios is the leading biopharmaceutical company focused on discovering and developing novel drugs in the fields of cancer metabolism and rare metabolic genetic diseases. Agios has multiple first-in-class programs in cancer metabolism and inborn errors of metabolism advancing toward the clinic, in addition to an active research and discovery pipeline across both therapeutic areas. The company has a significant collaboration with Celgene focused on developing new treatments for cancer leveraging Agios’ capabilities and insights into cancer metabolism. For more information, please visit our website at www.agios.com.