



AgiOS and CStone Pharmaceuticals Announce Exclusive Collaboration and License Agreement to Develop and Commercialize Ivosidenib in Greater China

June 26, 2018

- Agios to Receive \$12 Million Upfront Payment and is Eligible to Receive Development, Regulatory, and Sales-Based Milestones and Tiered Royalties -

- Collaboration Provides Opportunity for Patients with IDH1m Cancers in Greater China to Benefit from Ivosidenib -

CAMBRIDGE, Mass. and SUZHOU, China, June 26, 2018 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, and CStone Pharmaceuticals, a privately-held biopharmaceutical company devoted to developing a new generation of innovative drugs, today announced an exclusive collaboration and license agreement for the development and commercialization of ivosidenib (TIBSOVO®; AG-120) in Mainland China, Hong Kong, Macau and Taiwan ("Territory"), either as a monotherapy or in combination with other therapies. Discovered and developed by Agios, ivosidenib is an investigational, first-in-class, oral, targeted inhibitor of the mutant isocitrate dehydrogenase-1 (IDH1) enzyme. CStone Pharmaceuticals will be responsible for conducting the development and commercialization activities for ivosidenib in hematologic and solid tumor indications in the Territory, with an initial focus in acute myeloid leukemia (AML) and cholangiocarcinoma. Agios will retain all rights to ivosidenib in the rest of the world.

"CStone Pharmaceuticals brings together a highly experienced leadership team and drug development capabilities that will enable us to reach patients with IDH1 mutant cancers in Greater China who could benefit from ivosidenib," said David Schenkein, M.D., chief executive officer at Agios. "In addition to the clinical development activities that CStone will be leading, we also have the opportunity to leverage CStone's network to expand our ongoing and future global clinical trials of ivosidenib into Greater China."

Subject to the terms of the agreement, Agios will receive an upfront payment of \$12 million and will be eligible to receive up to \$412 million in milestone payments, of which \$147 million are related to development and regulatory events and \$265 million to the achievement of certain sales levels. Approximately half of the milestone payments are related to the development and commercialization of ivosidenib in AML, cholangiocarcinoma and other potential indications. The other half are payable only if development and commercialization of ivosidenib in brain cancer indications, including glioma, are pursued as part of the collaboration at a later date. In addition, CStone Pharmaceuticals will pay Agios tiered royalties ranging from the mid to high-teens as a percentage of annual net sales of ivosidenib in the Territory. CStone Pharmaceuticals will be responsible for all costs associated with development and commercialization activities for ivosidenib conducted in the Territory under the agreement.

"We're very pleased to partner with Agios to advance the global development of ivosidenib, which has clearly demonstrated significant benefit to patients with AML as well as potential utility in other IDH1m cancers," said Frank Jiang, M.D., Ph.D., chief executive officer at CStone Pharmaceuticals. "Given ivosidenib is currently under U.S. FDA priority review for IDH1m relapsed or refractory AML patients, it is the most advanced program in our pipeline. The partnership will also allow us to explore ivosidenib in combination with other products in our portfolio."

About Ivosidenib (TIBSOVO® / AG-120)

Ivosidenib is an investigational first-in-class, orally available, selective, potent inhibitor of the mutated IDH1 protein and is a highly targeted investigational medicine for the treatment of patients with cancers that harbor an IDH1 mutation. IDH1 is a metabolic enzyme that is mutated in a wide range of cancers, including acute myeloid leukemia, cholangiocarcinoma and glioma. Ivosidenib is currently under U.S. FDA priority review for IDH1m R/R AML patients with a PDUFA action date of August 21, 2018. The following clinical trials of ivosidenib are ongoing:

- Phase 1 trial of ivosidenib or enasidenib in combination with 7+3 in patients with newly diagnosed IDHm AML who are eligible for standard-of-care chemotherapy
- Phase 3 AGILE trial of ivosidenib in combination with azacitidine in patients with newly diagnosed IDH1m AML who are not eligible for standard-of-care chemotherapy
- Phase 3 ClarIDHy trial of ivosidenib in advanced IDH1m cholangiocarcinoma
- Perioperative study comparing ivosidenib and AG-881 in IDH1m low-grade glioma

About CStone Pharmaceuticals

CStone Pharmaceuticals is a clinical stage biopharmaceutical company devoted to the development of innovative drugs. With a broad pipeline, the company engages in the development of cancer therapeutics with a special focus on immuno-oncology based combination therapies. All members of the management team are seasoned executives from top multinational pharmaceutical companies. CStone has successfully built up its core competency in clinical development and translational medicine. The company is backed by prestigious VC/PE funds via two financing rounds to date, raising \$150 million in a Series A round in July 2016, followed by \$260 million in a Series B round in May 2018. With an experienced team, a rich pipeline, a robust R&D model, and substantial funding, CStone is well positioned as the partner of choice for multinational pharmaceutical and biotech companies to develop drugs in China and the Asia-Pacific region. For more information about CStone Pharmaceuticals, please visit:

www.cstonepharma.com.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has an approved oncology precision medicine and multiple first-in-class investigational therapies in clinical and/or preclinical development. All Agios programs focus on genetically identified patient populations, leveraging our knowledge of metabolism, biology and genomics. For more information, please visit the company's website at www.agios.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of, and plans relating to, the collaboration and license agreement between Agios and CStone Pharmaceuticals, including anticipated milestone and other payments under the collaboration; the potential of IDH1 as a therapeutic target; the potential benefits of ivosidenib, either as monotherapy or in combination with other therapies, in treating patients, including patients in the Territory; expectations regarding Agios' ability to expand its program for ivosidenib in the Territory; and the benefit of each company's strategic plans and focus. The words "could," "milestone," "opportunity," "potential," "will" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs. For example, there can be no guarantee that any product candidate will be successfully developed or complete necessary preclinical and clinical phases, or that development of any product candidates will successfully continue. There can be no guarantee that any positive developments will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including: results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, including those in the Territory, investigational review boards at clinical trial sites and publication review bodies; the ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; the ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates; the ability to maintain key collaborations, including its collaborations with Celgene Corporation and CStone Pharmaceuticals; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Agios expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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