Agios Pharmaceuticals Reports Second Quarter 2014 Financial Results

August 7, 2014

- AG-221: Company on track to start four Phase 1 expansion cohorts in second half of 2014; abstract of additional Phase 1 data submitted to the 2014 ASH Annual Meeting -

- AG-348: Phase 1 healthy volunteer studies met primary endpoints; abstract of Phase 1 data has been submitted to the 2014 ASH Annual Meeting -

CAMBRIDGE, Mass., Aug. 7, 2014 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq:AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today reported business highlights and financial results for the second quarter ended June 30, 2014.

"The first half of the year was defined by substantial progress in all three of our clinical development programs, and we anticipate this progress will continue through the remainder of 2014," said David Schenkein, M.D., chief executive officer of Agios. "In cancer metabolism, we announced promising Phase 1 data for AG-221 at two major medical conferences and continue to enroll patients into this trial and the two Phase 1 trials for AG-120. This progress has increased our confidence in IDH mutations as important cancer targets. We also advanced AG-348 for pyruvate kinase deficiency into the clinic. These three first-in-class drug candidates represent potentially transformational medicines coming out of Agios' scientific expertise.

"In the second half of the year, we submitted additional data for AG-221 and the first data for AG-348 for presentation at the 2014 Annual Meeting of the American Society of Hematology. We are also focused on achieving several clinical development milestones to further characterize the profiles and potential of these exciting new drug candidates," concluded Dr. Schenkein.

SECOND QUARTER 2014 AND RECENT BUSINESS HIGHLIGHTS

Cancer Metabolism: IDH Mutant Inhibitors in Collaboration with Celgene

AG-221: a first-in-class, oral, selective, potent inhibitor of the mutated IDH2 protein

- Agios continues to enroll patients in the Phase 1 study of AG-221 with the primary goals of determining the maximum tolerated dose (MTD) and recommended Phase 2 dose.
- Data presented in June at the 19th Congress of the European Hematology Association from the company's ongoing Phase 1 trial of AG-221 in 35 patients with IDH2-mutant positive advanced hematologic malignancies built upon those presented earlier this year at the American Association for Cancer Research meeting. Specifically, the data showed complete and durable remissions in patients with IDH2-mutant positive acute myelogenous leukemia (AML) and other advanced hematologic cancers, and a well-tolerated safety profile. Additional information on these data is available in a press release issued by Agios on June 14, 2014.
- In June, Agios' collaboration partner Celgene exercised its option to an exclusive worldwide license to AG-221. Agios will continue to conduct early clinical development and regulatory activities within the development program in collaboration with Celgene.
- Also in June, the U.S. Food and Drug Administration (FDA) granted Agios Orphan Drug Designation for AG-221 for treatment of patients with AML. The FDA grants Orphan Drug Designation to support development of medicines that affect fewer than 200,000 people in the U.S.

AG-120: a first-in-class, oral, selective, potent inhibitor of the mutated IDH1 protein

- Agios continues to advance two separate Phase 1 trials evaluating AG-120 in patients with IDH1-mutant hematologic malignancies and IDH1-mutant advanced solid tumors. The Phase 1 trials are multicenter, open-label, dose-escalation clinical studies designed to assess the safety and tolerability of AG-120 as a single agent in these cancers.

IDH Research

- Agios' scientists and academic collaborators published nonclinical research in the July issue of the journal *Nature* showing that IDH mutations may drive tumorigenesis in a mouse model of intrahepatic cholangiocarcinoma (iCCA). iCCA is the second most common form of liver cancer.

Rare Genetic Disorders of Metabolism: Wholly Owned PKR Activator

AG-348: a novel, first-in-class, orally available activator of pyruvate kinase-R (PKR) for the treatment of pyruvate kinase (PK) deficiency, a cause of hemolytic anemia

- Agios today announced that the Phase 1 single ascending dose (SAD) escalation trial for AG-348 in healthy volunteers (n=48) is complete and successfully met the primary endpoint of the study. The Phase 1 multiple ascending dose (MAD) escalation trial has also met its primary endpoint. Dose escalation is ongoing in healthy volunteers in the MAD study. The

primary objectives as defined by the SAD and MAD study protocols are to identify a safe and pharmacodynamically active dose and schedule of AG-348 to be used in subsequent trials in patients with PK deficiency.
- A natural history study of PK deficiency is also ongoing with patient enrollment on track. Natural history studies are important to confirm and further understand clinical characteristics, symptoms and disease complications and potentially support the design of future clinical trials.

UPCOMING MILESTONES

Cancer Metabolism

AG-221

- Agios submitted an abstract on its ongoing Phase 1 study of AG-221 for potential presentation at the upcoming 56th Annual Meeting of the American Society of Hematology (ASH) in December. The company continues to enroll patients in the dose escalation portion of the Phase 1 study. The maximum tolerated dose has not been reached.
- Agios plans to select a dose and schedule from the ongoing Phase 1 study and initiate four expansion cohorts of 25 patients each in the second half of 2014. The expansion cohorts will evaluate relapsed or refractory AML patients 60 years of age and older and transplant in-eligible, relapsed or refractory AML patients under age 60, untreated AML patients who decline standard of care chemotherapy and patients with other IDH2-mutant positive hematologic malignancies.
- The company remains on track to initiate a Phase 1 study of AG-221 in patients with advanced solid tumors with an IDH2 mutation in the second half of 2014.

AG-120

- The company expects to provide an update on the two Phase 1 studies evaluating patients with advanced hematologic malignancies and advanced solid tumors with an IDH1 mutation at medical conferences in 2015.

Rare Genetic Disorders of Metabolism

AG-348

- Based on meeting the primary endpoints announced today in the Phase 1 healthy volunteer studies, the company believes it will be in a position to initiate a Phase 2 clinical trial for AG-348 by early 2015 in patients with PK deficiency.
- The company has submitted an abstract for the Phase 1 healthy volunteer studies for AG-348 to the ASH Annual Meeting in 2014.
- Agios expects to report initial data from its study of the natural history of PK deficiency at a medical conference in 2015.

CORPORATE UPDATE

Agios plans to host and webcast an investor event in October 2014 in an effort to provide background and education on the novel programs it is targeting and the diseases the company hope to treat. Additional details will be provided in the coming weeks.

SECOND QUARTER 2014 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of June 30, 2014 were $261.1 million, compared to $193.9 million as of December 31, 2013. The increase was primarily driven by the addition of $94.7 million of net proceeds received from our public offering of 2,300,000 shares of common stock during the second quarter of 2014. During the second quarter of 2014, the company also received a $20 million payment related to Celgene's decision to extend the discovery phase of the collaboration agreement through April 2015.

Collaboration revenue was $8.4 million for the second quarter of 2014, compared to $6.3 million for the comparable period in 2013. Collaboration revenue is primarily comprised of amortization of deferred revenue from payments received in previous periods from Agios' collaboration agreement with Celgene. The increase in collaboration revenue in the second quarter of 2014 was related to Celgene's December 2013 election to extend the discovery phase of the collaboration agreement through April 2015.

Research and development (R&D) expenses were $22.6 million, including $1.4 million of stock- based compensation expense, in the second quarter of 2014, compared to $13.0 million, including $0.3 million in stock-based compensation expense, for the comparable period in 2013. The increase in R&D expenses was due to ongoing development activities for Agios's three lead drug candidates. Celgene is responsible for all development costs for AG-221 and will reimburse Agios for any Phase 1 costs it incurs for this drug candidate. The first reimbursement payment will be in the form of a milestone payment for development costs incurred by Agios and is expected in the second half of 2014.

General and administrative (G&A) expenses were $4.2 million, including $1.0 million of stock-based compensation expense, in the second quarter of 2014, compared to $1.8 million, including $0.1 million of stock-based compensation expense, for the comparable period in 2013. The increase in G&A expense was largely due to increased headcount and other professional expenses to support public company operations.

Net loss for the second quarter of 2014 was $18.3 million, compared to net loss of $8.6 million for the comparable period in 2013.

"Agios continues to maintain a strong balance sheet, which we strengthened in the second quarter by raising approximately $95 million through an equity offering ending the quarter with a cash position of $261 million," said Glenn Goddard, senior vice president of finance at Agios. "We expect our financial position to provide us with ample funding to execute on our strategic business plan and drive our lead drug candidates to meaningful clinical milestones."

FINANCIAL GUIDANCE FOR THE FULL YEAR 2014

Agios is reiterating today that it expects to end 2014 with more than $200 million of cash, cash equivalents and marketable securities. The company believes this cash position will be sufficient to fund its operating expenses and capital expenditure requirements through mid-2017.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:30 a.m. EDT to discuss the second quarter 2014 financial results and recent business activities. To participate in the conference call, please dial 1-877-377-7098 (domestic) or 1-631-291-4547 (international) and refer to conference ID 78621204. The live webcast can be accessed under "Events & Presentations" in the Investors and Media section of the company's website at www.agios.com. The archived webcast will be available on the company's website beginning approximately two hours after the event.

About Acute Myelogenous Leukemia (AML)

AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults. Undifferentiated blast cells proliferate in the bone marrow rather than maturing into normal blood cells. AML incidence significantly increases with age. Less than 10 percent of U.S. patients are eligible for bone marrow transplant, and the vast majority of patients do not respond to chemotherapy and progress to relapsed or refractory AML. AML prevalence is estimated to be approximately 115,000 to 160,000 patients worldwide, with approximately 20 percent of patients carrying an IDH mutation. The five-year survival rate for AML is approximately 20 to 25 percent.

About IDH Mutations and Cancer

IDH1 and IDH2 are two metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies. The prevalence of IDH mutations is expected to evolve as genomic analysis of tumors increase. Agios' research revealed the potential of IDH1 and IDH2 mutations as novel therapeutic targets in cancer, which may lead to clinical benefit for the subset of cancer patients whose tumors carry them. Patients carry either an IDH1 or IDH2 mutation, but not both.

Agios is developing two oral, first-in-class IDH mutant inhibitors: AG-221 is an IDH2 mutant inhibitor and AG-120 is an IDH1 mutant inhibitor. AG-221 is currently being evaluated in a Phase 1 dose-escalation study in patients with advanced hematologic malignancies. AG-120 is currently being evaluated in two Phase 1 trials, one in hematologic malignancies and another in solid tumors. Both compounds were discovered and developed in the laboratory of Agios.

About Pyruvate Kinase (PK) Deficiency, a Rare, Inherited Hemolytic Anemia

Pyruvate kinase (PK) deficiency, a rare, inherited hemolytic anemia affecting children and adults, is caused by mutations that affect the activity of the metabolic enzyme pyruvate kinase-R (PKR), the form of pyruvate kinase that is present in red blood cells. The current standard of care for PK deficiency is supportive, including blood transfusions, splenectomy, chelation therapy to address iron overload and/or interventions for other treatment, and disease-related morbidities. Currently, there is no approved therapy to treat the underlying cause of PK deficiency. AG-348 is a first-in-class orally available, potent, selective small molecule activator of PKR, which, when mutated, leads to PK deficiency. AG-348 was discovered in the laboratory of Agios, and the company retains worldwide development and commercialization rights.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and rare genetic disorders of metabolism through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has multiple first-in-class lead drug candidates 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 our website at www.agios.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, including those regarding Agios' expectations and beliefs about: the potential of IDH1/IDH2 and pyruvate kinase-R mutations as therapeutic targets; the potential benefits of Agios' drug candidates targeting IDH1/IDH2 or pyruvate kinase-R mutations, including AG-221, AG-120 and AG-348; its plans and timelines for the clinical development of AG-221, AG-120 and AG-348; its plans regarding future data presentations; its financial guidance regarding the period in which cash will be available to fund its operating expenses and capital expenditure requirements, and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could" 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 Agios' current expectations and beliefs. For example, there can be no guarantee that any product candidate Agios is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. There can be no guarantee that any positive developments in Agios' business 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: Agios' 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, investigational review boards at clinical trial sites and publication review bodies; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to maintain key collaborations, such as its agreement with Celgene, and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' Quarterly Report on Form 10-Q for the quarter ended March 31, 2014 as well as other filings that Agios may make with the Securities and Exchange Commission in the future. 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.

AGIOS PHARMACEUTICALS, INC.

Consolidated Balance Sheet Data *(Unaudited)*

(in thousands)

	June 30, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	$ 261,111	$ 193,894
Total assets	272,030	201,205
Deferred revenue – related party	60,341	57,639
Stockholders' equity	200,548	131,482

Consolidated Statements of Operations Data *(Unaudited)*

(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Collaboration revenue – related party	$ 8,411	$ 6,268	$ 16,822	$ 12,536
Operating expenses:				
Research and development	22,576	12,958	39,982	24,420
General and administrative	4,165	1,836	7,454	3,688
Total operating expenses	26,741	14,794	47,436	28,108
Loss from operations	(18,330)	(8,526)	(30,614)	(15,572)
Interest income	34	5	70	13
Loss before provision for income taxes	(18,296)	(8,521)	(30,544)	(15,559)
Provision for income taxes	--	(99)	--	(289)
Net loss	(18,296)	(8,620)	(30,544)	(15,848)
Cumulative preferred stock dividends	--	(1,798)	--	(3,595)
Net loss applicable to common stockholders	$ (18,296)	$ (10,418)	$ (30,544)	$ (19,443)
Net loss per share applicable to common stockholders – basic and diluted	$ (0.54)	$ (2.80)	$ (0.94)	$ (5.27)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	33,602,472	3,722,963	32,506,739	3,690,669

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