Agios Pharmaceuticals Reports Third Quarter 2014 Financial Results

November 7, 2014

Data from Three Lead Clinical Stage Medicines to be Presented at Major Medical Meetings by end of 2014

CAMBRIDGE, Mass., Nov. 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 third quarter ended September 30, 2014.

"2014 is an important year for the company, as we make progress on key clinical objectives for all three of our investigational medicines: AG-221, AG-120 and AG-348," said David Schenkein, M.D., chief executive officer of Agios. "We recently broadened the clinical development program for AG-221 with the initiation of Phase 1 expansion cohorts in advanced hematologic malignancies and the initiation of a Phase 1/2 clinical trial in advanced solid tumors. We look forward to presenting the first data on AG-120 in advanced hematologic malignancies later this month at EORTC-NCI-AACR. In addition, we look forward to presenting the first clinical data for AG-348 from the healthy volunteer studies at the American Society of Hematology (ASH) meeting in December. These advancements and data presentations in the coming weeks at EORTC-NCI-AACR and ASH will help to inform our development plans in 2015 and beyond with the goal of bringing transformational medicines to patients in need."

RECENT BUSINESS HIGHLIGHTS AND POTENTIAL MILESTONES

Cancer Metabolism: IDH Mutant Inhibitors in Collaboration with Celgene

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

- An oral presentation of AG-221 in advanced hematologic malignancies will be presented at the American Society of Hematology (ASH) Annual Meeting and Exhibition in San Francisco. The presentation, "*AG-221, an Oral, Selective, First-in-Class, Potent Inhibitor of the IDH2 Mutant Metabolic Enzyme, Induces Durable Remissions in a Phase I Study in Patients with IDH2 Mutation Positive Advanced Hematologic Malignancies,*" will be presented by Eytan Stein, M.D., lead investigator and physician in the leukemia service at Memorial Sloan-Kettering Cancer Center on Sunday, December 7, 2014 at 4:30 p.m. PST (7:30 p.m. EST). Also at ASH, two abstracts related to AG-221 were accepted for poster presentations. The accepted abstracts are available online on the ASH conference website: ash.confex.com/ash /2014/webprogram/start.html.
- Agios plans to host an investor event in San Francisco on Monday, December 8, 2014 beginning at 12:00 p.m. PST (3:00 p.m. EST) to discuss AG-221 and the preclinical and clinical data presented at ASH. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors & Media section of the company's website at www.agios.com.
- Agios continues to conduct the Phase 1 study in IDH2-mutant positive advanced hematologic malignancies with the primary goals of establishing the safety profile, determining the maximum tolerated dose (MTD), and assessing the clinical activity of AG-221. In October, the company initiated four expansion cohorts. The Phase 1 expansion cohorts will assess the safety and tolerability of AG-221 at 100 mg once daily in approximately 100 patients with IDH2-mutant hematologic malignancies, including acute myelogenous leukemia (AML).
- In August, the U.S. Food and Drug Administration (FDA) granted Agios Fast Track Designation for AG-221 for the treatment of patients with AML that harbor an IDH2 mutation. Fast Track is granted to facilitate frequent interactions with the FDA in an effort to expedite clinical development and submission of a new drug application for medicines with the potential to treat serious conditions.
- In October, Agios initiated a Phase 1/2 trial of AG-221 in patients with advanced solid tumors as the next step in evaluating AG-221's potential in a broad range of cancers that harbor the IDH2 mutation.

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

- The first data from the ongoing Phase 1 trial of AG-120 in advanced IDH1-mutant positive hematologic malignancies will be presented in a late-breaking oral presentation at the 26th Symposium on Molecular Targets and Cancer Therapeutics hosted by the European Organization for Research and Treatment of Cancer (EORTC), the National Cancer Institute and the American Association for Cancer Research (EORTC-NCI-AACR), taking place in mid-November in Barcelona, Spain. The presentation will be given by Daniel Pollyea, M.D., lead investigator and physician of leukemia services at the University of Colorado School of Medicine on Wednesday, November 19, 2014, at 2:50 p.m. CET (8:50 a.m. EST). In accordance with the symposium's embargo policy, these data remain under embargo until the day of the presentation on Wednesday, November 19 at 12:01 a.m. CET (Tuesday, November 18 at 6:01 p.m. EST).
- Agios plans to host a conference call and webcast on Wednesday, November 19 at 4:00 p.m. CET (10:00 a.m. EST) to review the Phase 1 clinical data for AG-120 being presented at the symposium. To participate in the conference call, please dial 1-877-377-7098 (domestic) or 1-631-291-4547 (international) and refer to conference ID 31391928. A replay of the call will be available approximately two hours after the conclusion of the call. The webcast can be accessed live or in

archived form under "Events & Presentations" in the Investors & Media section of the company's website at www.agios.com.

- Agios continues to advance two separate Phase 1 clinical trials evaluating AG-120 in patients with IDH1-mutant advanced 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. The company expects to provide an update on the Phase 1 study evaluating patients with advanced solid tumors with an IDH1 mutation at a medical conference in 2015.

Rare Genetic Disorders of Metabolism: Wholly Owned PKR Activator

AG-348: a novel, first-in-class, oral activator of pyruvate kinase-R (PKR) for the treatment of pyruvate kinase (PK) deficiency

- Agios researchers plan to present the first clinical data from healthy volunteer studies with AG-348 in a poster presentation at the ASH Annual Meeting in December. Key results to be presented during the meeting include the first data on safety, pharmacokinetics and pharmacodynamics from the completed single ascending dose (SAD) and multiple ascending dose escalation (MAD) studies in healthy volunteers.
- The company plans to review data presented for AG-348 at ASH in conjunction with the planned investor event on December 8, 2014 referenced above for AG-221.
- Agios recently completed enrollment in the ongoing Phase 1 MAD trial in healthy volunteers. Based on meeting the primary endpoints 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 in early 2015 in patients with PK deficiency.
- A natural history study of PK deficiency is also ongoing and patient enrollment is 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. Agios expects to report initial data from its study of the natural history of PK deficiency at a medical conference in 2015.

THIRD QUARTER 2014 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of September 30, 2014 were $237.9 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 the company's public offering of 2,300,000 shares of common stock during the second quarter of 2014. The company also received a $20 million payment related to Celgene's decision to extend the discovery phase under our collaboration agreement through April 2015 during the second quarter. These inflows in 2014 have been offset by cash used to fund operating activities of approximately $71.6 million.

Collaboration revenue was $33.9 million for the third quarter of 2014, compared to $6.3 million for the comparable period in 2013. The increase was due to the application of new accounting guidance to the company's collaboration agreement with Celgene. In July 2014, the company amended its collaboration agreement with Celgene. As a result, for the third quarter of 2014 through the amendment date, the company recognized a total of $25.9 million under the previous accounting guidance and upon the modification. The company recognized $8.0 million in revenue subsequent to the modification date.

Research and development (R&D) expenses were $25.5 million, including $1.4 million of stock-based compensation expense, in the third quarter of 2014, compared to $14.8 million, including $0.9 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' three lead investigational medicines. Celgene is responsible for all development costs for AG-221, and reimburses Agios for development costs it incurs for this investigational medicine. As of September 30, 2014, the company has recorded a collaboration receivable of $18.7 million related to reimbursable development costs for AG-221.

General and administrative (G&A) expenses were $5.2 million, including $1.4 million of stock-based compensation expense, in the third quarter of 2014, compared to $2.5 million, including $0.2 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 growing operations.

Net income for the third quarter of 2014 was $3.7 million, compared to a net loss of $11.2 million for the comparable period in 2013. The third quarter of 2014 includes additional revenue recognition related to the amendment of the company's collaboration agreement with Celgene.

"Agios continues to maintain a strong balance sheet ending the quarter with a cash position of $237.9 million," said Glenn Goddard, senior vice president of finance at Agios. "Overall, we are pleased with our balance sheet strength, and we will continue to prioritize our development investment in line with the progress of our three investigational medicines, and maintain our investment in research to support the growth of our pipeline."

FINANCIAL GUIDANCE FOR THE FULL YEAR 2014

Agios is updating today its 2014 cash guidance, and expects to end the year with more than $220 million of cash, cash equivalents and marketable securities.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:00 a.m. EST to discuss the third 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 26002343. The live webcast can be accessed under "Events & Presentations" in the Investors & 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 AG-221 and IDH2

IDH1 and IDH2 are metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies, including AML. Normally, IDH enzymes help to break down nutrients and generate energy for cells. When mutated, IDH creates a molecule that alters the cells' genetic programming, and instead of maturing, the cells remain primitive and proliferate quickly. Agios believes that inhibition of these mutated proteins may lead to clinical benefit for the subset of cancer patients whose tumors carry them. AG-221 was developed by Agios as a selective, potent inhibitor of the mutated IDH2 protein. While most cancer treatments attempt to destroy the cancerous cells, AG-221 uniquely attacks its target – mutated IDH2 – and aids the maturation of the cells into functioning blood cells. AG-221 has received Orphan Drug Designation for AML and Fast Track Designation for patients with IDH2-mutant AML.

About Agios/Celgene Collaboration

AG-221 is a part of Agios' global strategic collaboration with Celgene Corporation, an integrated global biopharmaceutical company. In June 2014, Celgene exercised its exclusive option to license AG-221 and gained worldwide development and commercialization rights for AG-221. Agios continues to conduct early clinical development activities within the AG-221 development program. The companies are also collaborating on the development of AG-120, which is being studied in two Phase 1 trials in patients whose hematologic malignancies and solid tumors carry an IDH1 mutation. Agios retains U.S. development and commercialization rights for AG-120, and Celgene has an exclusive option to the ex-U.S. rights.

About AG-348 and 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 investigational medicines 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 amount of cash and cash equivalents that the company will have as of December 31, 2014, and the potential 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 June 30, 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

(in thousands)

(Unaudited)

	September 30, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	$ 237,887	$ 193,894
Collaboration receivable – related party	18,759	476

Total assets	269,608	201,205
Deferred revenue – related party	45,199	57,639
Stockholders' equity	207,749	131,482

Consolidated Statements of Operations Data

(in thousands, except share and per share data)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	**2013**	**2014**	**2013**
Gross collaboration revenue – related party	$33,900	$6,268	$50,722	$18,804
Operating expenses:				
Research and development	25,526	14,803	65,509	39,223
General and administrative	5,166	2,534	12,619	6,222
Total operating expenses	30,692	17,337	78,128	45,445
Income (loss) from operations	3,208	(11,069)	(27,406)	(26,641)
Interest income	48	13	118	26
Income (loss) before provision for income taxes	3,256	(11,056)	(27,288)	(26,615)
(Benefit) provision for income taxes	(448)	121	(448)	410
Net income (loss)	3,704	(11,177)	(26,840)	(27,025)
Cumulative preferred stock dividends	—	(567)	—	(4,162)
Net income (loss) applicable to common stockholders	$3,704	$(11,744)	$(26,840)	$(31,187)
Net income (loss) per share applicable to common stockholders – basic	$0.11	$(0.52)	$(0.81)	$(3.08)
Net income (loss) per share applicable to common stockholders – diluted	$0.10	$(0.52)	$(0.81)	$(3.08)
Weighted-average number of common shares used in net income (loss) per share applicable to common stockholders – basic	34,495,076	22,744,486	33,176,801	10,111,735
Weighted-average number of common shares used in net income (loss) per share applicable to common stockholders – diluted	36,592,683	22,744,486	33,176,801	10,111,735

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