Agios Reports Second Quarter 2015 Financial Results and Provides Updates on Clinical Development Progress

August 6, 2015

Global Phase 3 Registration-enabling Studies on Track to Begin in Second Half of 2015 for AG-221 & First Half of 2016 for AG-120

Phase 1 Expansion Studies for AG-221 & AG-120 Programs Ongoing

CAMBRIDGE, Mass., Aug. 6, 2015 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals (NASDAQ:AGIO), a leader in the fields of cancer metabolism and rare genetic metabolic disorders, today reported business highlights and financial results for the second quarter ended June 30, 2015.

"In the first half of 2015, we continued to drive rapid and meaningful progress across our development programs, as well as selecting our fourth molecule for development, AG-881, and advancing it into two Phase 1 studies," said David Schenkein, M.D., chief executive officer of Agios. "Additionally, we recently initiated DRIVE PK, a Phase 2 study of AG-348 in PK deficiency patients, and are on track to initiate two global, registration-enabling Phase 3 studies of AG-221 and AG-120 in patients with acute myeloid leukemia in the next six to 12 months. These efforts bring us closer to our goal of being a late-stage biopharmaceutical company capable of changing the treatment paradigm for people with cancer and rare genetic disorders."

KEY UPCOMING MILESTONES

Agios anticipates the following milestones from its IDH clinical development programs in collaboration with Celgene:

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

- Initiate a global Phase 3 registration-enabling study in relapsed/refractory acute myeloid leukemia (AML) patients who harbor an IDH2 mutation by the end of 2015.
- Initiate combination trials to evaluate AG-221 as a potential frontline treatment for patients with AML who harbor an IDH2 mutation by the end of 2015.

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

- Begin combination trials to evaluate AG-120 as a potential frontline treatment for AML patients who harbor an IDH1 mutation by the end of 2015.
- Initiate a global registration-enabling Phase 3 study in AML patients who harbor an IDH1 mutation in the first half of 2016.
- Present data on the ongoing Phase 1 study of AG-120 in IDH1 mutant-positive advanced solid tumors at a medical meeting in the fourth quarter of 2015.

RECENT DEVELOPMENT UPDATES IN CANCER METABOLISM

Agios has provided the following updates on its clinical development programs in collaboration with Celgene:

AG-221

- New data from the dose-escalation phase and expansion cohorts from the ongoing Phase 1 study evaluating single agent AG-221 were presented in June at the 20th Congress of the European Hematology Association (EHA). Read the full AG-221 data here.
- As of July, Agios completed the dose-escalation portion of the Phase 1 study of AG-221 in IDH2 mutant-positive hematologic malignances. The expansion cohorts are on track, including the fifth expansion cohort of 125 patients with IDH2 mutant-positive AML who are in second or later relapse, refractory to second-line induction or re-induction treatment, or have relapsed after allogeneic transplantation.
- Dose escalation continues in the Phase 1/2 trial in patients with advanced solid tumors and angioimmunoblastic T-cell lymphoma (AITL) who carry an IDH2 mutation.

AG-120

- New data from the dose-escalation phase of the ongoing Phase 1 study evaluating single agent AG-120 in advanced hematologic malignancies were presented in June at EHA. Read the full AG-120 data here.
- Also announced in June, the U.S. Food and Drug Administration (FDA) granted Agios orphan drug designation for AG-120 for the treatment of patients with IDH1 mutant-positive AML.
- In May, Agios announced that the FDA granted Fast Track designation to AG-120 for the treatment of patients with IDH1 mutant-positive AML.
- As of July, Agios completed the dose-escalation portion of the Phase 1 study of AG-120 in IDH1 mutant-positive

hematologic malignances. The three expansion cohorts to evaluate AG-120 in 175 patients with IDH1-mutated advanced hematologic malignancies, including one cohort with 125 patients with relapsed and/or refractory AML, are on track.

AG-881: a brain-penetrant, first-in-class, oral, potent pan-inhibitor of the mutated IDH1 and IDH2 proteins

- In June, Agios announced that the first patient was dosed in a Phase 1, open-label, dose-escalation and expansion study of single agent AG-881 in patients with IDH mutant-positive advanced solid tumors, including gliomas.
- In August, the first patient was dosed in a second dose-escalating and expansion trial for patients with IDH mutant-positive advanced hematologic malignancies, whose cancer has progressed on a prior IDH inhibitor therapy.

RECENT DEVELOPMENT UPDATES IN RARE GENETIC METABOLIC DISORDERS

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

- In June, final data from the Phase 1 multiple-ascending dose (MAD) study in healthy volunteers were presented at EHA, establishing clear proof-of-mechanism for AG-348. Read the full AG-348 data, as well as data from Boston Children's natural history study, here.
- Also in June, Agios initiated DRIVE PK, a global Phase 2, open-label safety and efficacy trial in adult, transfusion-independent patients with PK deficiency. The first patient was dosed in July.

CORPORATE UPDATE

Agios plans to host and webcast an investor event in October 2015 in an effort to provide background and education on the novel programs it is targeting and the diseases the company aims to treat. Additional details will be provided in the coming weeks.

SECOND QUARTER 2015 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of June 30, 2015 were $434.0 million, compared to $467.4 million as of December 31, 2014. The decrease was driven by cash expenditures for operating activities of approximately $64.9 million, which was offset by cash received from Celgene of approximately $43.3 million during the six months ended June 30, 2015 related to our collaboration agreements.

Collaboration revenue was $13.2 million for the second quarter of 2015, compared to $8.4 million for the comparable period in 2014. The increase reflects revenues recognized under the company's collaboration agreements with Celgene, including $8.8 million related to the delivery of the U.S. and ex-U.S. licenses for AG-881.

Research and development (R&D) expense was $36.4 million, including $4.6 million of stock- based compensation expense in the second quarter of 2015, compared to $22.6 million, including $1.4 million in stock-based compensation expense for the comparable period in 2014. The increase in R&D expense was primarily due to increased costs to support advancement of the company's lead investigational medicines toward later-stage development.

General and administrative (G&A) expense was $8.9 million, including $3.6 million of stock-based compensation expense, in the second quarter of 2015, compared to $4.2 million, including $1.0 million of stock-based compensation expense, for the comparable period in 2014. The increase in G&A expense was largely due to increased headcount and other professional expenses to support growing operations.

Net loss for the second quarter of 2015 was $31.9 million, compared to net loss of $18.3 million for the comparable period in 2014.

ADJUSTED FINANCIAL GUIDANCE FOR THE FULL YEAR 2015

Today Agios raised its previous year end cash guidance and now expects to end 2015 with more than $350.0 million of cash, cash equivalents and marketable securities. The anticipated year end 2015 cash position does not include any additional program-specific milestone payments. Agios expects that its cash, cash equivalents and marketable securities would be sufficient to fund its operating expenses and capital expenditure requirements until late 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 2015 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 85893695. 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 Agios/Celgene Collaboration

AG-221, AG-120 and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation. Under the terms of the collaboration, Celgene has worldwide development and commercialization rights for AG-221. Agios continues to conduct clinical development activities within the AG-221 development program and is eligible to receive up to $120 million in payments on achievement of certain milestones and royalties on net sales. For AG-120, Agios retains U.S. development and commercialization rights. Celgene has an exclusive license outside the United States. Celgene is eligible to receive royalties on net sales in the U.S. Agios is eligible to receive royalties on net sales outside the U.S. and up to $120 million in payments on achievement of certain milestones. For AG-881, the companies have a joint worldwide development and 50/50 profit share collaboration, and Agios is eligible to receive regulatory milestone payments of up to $70 million.

About Agios Pharmaceuticals

Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic metabolic disorders 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 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 the company's website at 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 Agios' product candidates targeting IDH1/IDH2 or pyruvate kinase-R mutations, including AG-221, AG-120, AG-881 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, cash equivalents and marketable securities that the company will have as of December 31, 2015; and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "potential," "hope," "could," "would" 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, 2015, and 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.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	June 30, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	$ 434,037	$ 467,447
Collaboration receivable – related party	10,474	6,492
Total assets	474,383	491,904
Deferred revenue – related party	31,997	38,411
Stockholders' equity	404,165	424,366

Consolidated Statements of Operations Data
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Collaboration revenue – related party (1)	$13,219	$8,411	$47,421	$16,822
Operating expenses:				
Research and development (2)	36,423	22,576	68,866	39,982
General and administrative	8,929	4,165	15,883	7,454
Total operating expenses	45,352	26,741	84,749	47,436

Loss from operations	(32,133)	(18,330)	(37,328)	(30,614)
Interest income	236	34	474	70
Net loss	(31,897)	(18,296)	(36,854)	(30,544)
Net loss per share– basic and diluted	$ (0.85)	$ (0.54)	$ (0.99)	$ (0.94)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	37,329,220	33,602,472	37,272,300	32,506,739

Note 1 (Collaboration revenue): The collaboration revenue increase was primarily due to the application of new accounting guidance to the Company's collaboration arrangements with Celgene, which include the July 2014 amendment of the 2010 agreement and the April 2015 execution of the AG-881 agreements. Previously, all arrangement consideration was recognized ratably over the estimated period of performance. Under the new accounting guidance, revenue is recognized as services or goods are delivered, which during the first quarter of 2015 included $15.8 million related to the delivery of an ex- U.S. license for AG-120, and during the second quarter of 2015 included $8.8 million related to the delivery of U.S. and ex-U.S. licenses for AG-881.

Note 2 (R&D expense): During the first quarter of 2015, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of costs related to our IDH programs. The R&D expense reported for the three and six months ended June 30, 2015 is presented net of $4.5 million and $8.9 million, respectively, of reimbursement compared to no offset for cost reimbursement for the comparable periods in 2014.

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