Agios Reports Third Quarter 2015 Financial Results

November 5, 2015

– New AG-221 and AG-120 Data in Solid Tumors and Hematologic Malignancies to be Presented at AACR-NCI-EORTC and ASH Annual Meeting –

– R&D Day Highlights Included Initiation of AG-221 Phase 3 "IDHENTIFY" Study, Design of AG-221 and AG-120 Frontline Combination Studies, and Selection of Second PKR Activator, AG-519, for Clinical Development –

CAMBRIDGE, Mass., Nov. 5, 2015 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and rare genetic metabolic disorders, today reported business highlights and financial results for the third quarter ended September 30, 2015.

"We have made significant progress this year toward realizing our goal of what's possible for patients with our IDH inhibitors, by deploying a comprehensive development strategy of speed and breadth with AG-221 and AG-120 in AML and other cancers," said David Schenkein, M.D., chief executive officer at Agios. "In addition, we are pleased to have selected our fifth molecule, AG-519, for clinical development in PK deficiency. This coupled with DRIVE PK, our ongoing Phase 2 study of AG-348, may optimize our potential to help people with this rare genetic disorder."

KEY UPCOMING MILESTONES IN CANCER METABOLISM

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

- Present new data from the ongoing Phase 1 dose-escalation and expansion studies of AG-221 in advanced IDH2-mutant positive hematologic malignancies at the American Society of Hematology (ASH) Annual Meeting taking place December 5-8, 2015 in Orlando.

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

- Present first data from the ongoing Phase 1 dose-escalation trial of AG-120 in advanced IDH1-mutant positive solid tumors in an oral presentation at AACR-EORTC-NCI International Conference on Molecular Targets and Cancer Therapeutics on November 8, 2015 in Boston.
- Present new data from the ongoing Phase 1 dose-escalation and expansion studies of AG-120 in advanced IDH1-mutant positive hematologic malignancies at the ASH Annual Meeting.
- Initiate a global registration-enabling Phase 3 study in patients with acute myeloid leukemia (AML) harboring an IDH1 mutation in the first half of 2016.

AG-221 and AG-120 front-line AML combination trials

- Initiate a Phase 1b combination study of either AG-221 or AG-120 with standard induction (7+3, Ara-C and idarubicin/daunorubicin) and consolidation (Ara-C, or mitoxantrone with etoposide) chemotherapy in newly diagnosed AML patients eligible for intensive chemotherapy by the end of 2015.
- Initiate a Phase 1/2 combination study of either AG-221 or AG-120 with VIDAZA® (azacitidine) in newly diagnosed AML patients not eligible for intensive chemotherapy in the first quarter of 2016.

KEY UPCOMING MILESTONES 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

- Present data from the Phase 1 healthy volunteers study of AG-348 and new findings from the Natural History Study of PK deficiency (being conducted with Boston Children's Hospital) at the ASH Annual Meeting.

AG-519: a novel, oral activator of PKR for the treatment of PK deficiency

- Initiate an integrated single ascending dose (SAD) and multiple ascending dose (MAD) placebo-controlled Phase 1 study in healthy volunteers in the first quarter of 2016.

RECENT DEVELOPMENT UPDATES IN CANCER METABOLISM

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

AG-221

- IDHENTIFY, the Phase 3 study of AG-221, was initiated in October. This is an international, multi-center, open-label, randomized clinical trial designed to compare the efficacy and safety of AG-221 versus conventional care regimens in

patients 60 years or older with IDH2 mutant-positive AML that is refractory to or relapsed after second- or third-line therapy. This study is being conducted by Celgene.
- The expansion phase of the Phase 1 trial of AG-221 is on track and continues to enroll. It includes four cohorts with 25 patients each and a fifth expansion cohort of 125 patients with IDH2 mutant-positive AML who are in second or later relapse, refractory to second-line induction or reinduction treatment, or have relapsed after allogeneic transplantation.
- The ongoing Phase 1 trial of AG-221 in IDH2-mutated advanced solid tumors and angioimmunoblastic T-cell lymphoma continues to enroll patients.

AG-120

- The expansion phase of the Phase 1 trial of AG-120 is on track and continues to enroll. It includes three expansion cohorts of a total of 175 patients with IDH1-mutated advanced hematologic malignancies, including one cohort with 125 patients with relapsed and/or refractory AML.
- The ongoing Phase 1 trial of AG-120 in IDH1-mutated advanced solid tumors continues to enroll.

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

- Two Phase 1, open-label, dose-escalation and expansion studies are on track and continue to enroll – the first in advanced IDH mutant-positive solid tumors and the second in patients with advanced IDH mutant-positive hematologic malignancies whose cancer has progressed on a prior IDHm inhibitor therapy.

RECENT DEVELOPMENT UPDATES IN RARE GENETIC DISORDERS OF METABOLISM

AG-348

- DRIVE PK, a global Phase 2, open-label safety and efficacy trial in adult, transfusion-independent patients with PK deficiency, is on track and enrolling.
- A natural history study of PK deficiency is also ongoing and patient enrollment is on track.

AG-519

- Agios selected a fifth molecule for clinical development, AG-519, a novel, oral activator of PKR for the treatment of PK deficiency.

THIRD QUARTER 2015 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of September 30, 2015 were $408.0 million, compared to $467.4 million as of December 31, 2014. The decrease was driven by cash used to fund operating activities of approximately $101.2 million, which was offset by funding of approximately $54.8 million made by Celgene during the nine months ended September 30, 2015 related to our collaboration agreements.

Collaboration revenue was $5.5 million for the third quarter of 2015, compared to $33.9 million for the comparable period in 2014. In July 2014, the company amended its collaboration agreement with Celgene. As a result, for the third quarter of 2014 the company recognized a total of $25.9 million under the previous accounting guidance and upon the modification in addition to $8.0 million in revenue subsequent to the modification through September 30, 2014.

Research and development (R&D) expense was $36.0 million, including $4.9 million of stock- based compensation expense in the third quarter of 2015, compared to $25.5 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 $9.9 million, including $4.5 million of stock-based compensation expense, in the third quarter of 2015, compared to $5.2 million, including $1.4 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 third quarter of 2015 was $40.3 million, compared to net income of $3.7 million for the comparable period in 2014. The third quarter of 2014 includes additional revenue recognition related to the amendment of the company's collaboration agreement with Celgene.

FINANCIAL GUIDANCE FOR THE FULL YEAR 2015

Agios is reiterating that it 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 8:00 a.m. ET to discuss the third quarter 2015 financial results and recent business activities. To participate in the conference call, please dial (877) 377-7098 (domestic) or (631) 291-4547 (international) and refer to conference ID 66586914. 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

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.

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.

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, AG-348 and AG-519; its plans and timelines for the clinical development of AG-221, AG-120, AG-881, AG-348 and AG-519; 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 June 30, 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)

	September 30, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	$ 407,986	$ 467,447
Collaboration receivable – related party	9,078	6,492
Total assets	449,086	491,904
Deferred revenue – related party	28,890	38,411
Stockholders' equity	375,657	424,366

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014

Gross Collaboration revenue – related party (1)	$5,480	$33,900	$52,901	$50,722
Operating expenses:				
Research and development (2)	36,028	25,526	104,894	65,509
General and administrative	9,927	5,166	25,809	12,619
Total operating expenses	45,955	30,692	130,703	78,128
Income (loss) from operations	(40,475)	3,208	(77,802)	(27,406)
Interest income	218	48	692	118
Income (loss) before benefit for income taxes	(40,257)	3,256	(77,110)	(27,288)
Benefit for income taxes	--	(448)	--	(448)
Net loss	(40,257)	3,704	(77,110)	(26,840)
Net loss per share– basic	$(1.07)	$0.11	$(2.06)	$(0.81)
Net loss per share– diluted	$(1.07)	$0.10	$(2.06)	$(0.81)
Weighted-average number of common shares used in net loss per share – basic	37,507,298	34,495,076	37,351,493	33,176,801
Weighted-average number of common shares used in net loss per share – diluted	37,507,298	36,592,683	37,351,493	33,176,801

Note 1 (Collaboration revenue): The collaboration revenue decrease for the three months ended September 30, 2015 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. The three months ended September 30, 2014 included recognized revenue at the July 2014 amendment date related to the excess of total consideration over the best estimate of selling price of undelivered elements, which fundamentally related to previously delivered elements under the agreement and includes the exclusive global license for development and commercialization of AG-221.

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 incurred on Celgene's behalf. The R&D expense reported for the three and nine months ended September 30, 2015 is presented net of $7.8 million and $16.7 million, respectively, of reimbursement compared to no offset for cost reimbursement for the comparable periods in 2014.

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