Agios Reports Third Quarter 2016 Financial Results and Reviews Recent Progress in IDH and PKR Development Programs

November 3, 2016

– Enasidenib (AG-221) on Track for NDA Submission in IDH2m Positive Relapsed/Refractory AML by Year End –

– Follow-on Offering Raised Approximately $173 Million in September; Updated 2016 Year End Cash Position Expected to be More than $550 Million –

– Seven Abstracts Accepted for Presentation at ASH, Including Updated Data from AG-348 Phase 2 DRIVE PK, AG-519 Phase 1 Healthy Volunteer and AG-120 Phase 1 Dose Escalation Studies –

CAMBRIDGE, Mass., Nov. 03, 2016 (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, 2016.

“We have made significant progress during 2016, establishing proof of concept with our lead pyruvate kinase-R (PKR) activator, executing late-stage clinical development for both of our lead isocitrate dehydrogenase (IDH) mutant inhibitors in hematologic malignancies and strengthening our balance sheet through our recent financing," said David Schenkein, M.D., chief executive officer at Agios. “As we head into year end, we are focused on supporting the enasidenib NDA submission with our partner Celgene and planning for our next steps in clinical development for our PKR and IDH portfolios based on data we will present at a number of upcoming medical meetings this quarter.”

THIRD QUARTER 2016 HIGHLIGHTS & UPDATES

IDH Mutant Inhibitors in Hematologic Malignancies:

In September, Agios and Celgene announced plans to submit a new drug application (NDA) to the U.S. Food and Drug Administration for enasidenib (AG-221) in relapsed and/or refractory (R/R) acute myeloid leukemia (AML) with an IDH2 mutation by year-end. The NDA will be based on data from an ongoing Phase 1/2 trial in patients with relapsed and/or refractory AML and other advanced hematologic malignancies with an IDH2 mutation.

Agios plans to explore a similar regulatory path for AG-120, its wholly owned, first-in-class, oral, potent inhibitor of mutant IDH1, which could lead to a NDA submission in 2017 in the U.S.

Corporate:

In September, Agios completed an underwritten public offering of common stock for 3,876,403 shares at the offering price of $44.50 per share, resulting in gross proceeds of approximately $173 million.

Agios recently announced the appointment of Andrew Hirsch to chief financial officer. Mr. Hirsch has more than 20 years of experience in a range of strategic and operating roles. He most recently served as president and chief executive officer of BIND Therapeutics. Prior to joining BIND, he was chief financial officer at Avila Therapeutics until its acquisition by Celgene and held roles of increasing responsibility during his nearly 10-year tenure at Biogen.

UPCOMING MEDICAL MEETING PRESENTATIONS

- First data from the expansion phase of the ongoing Phase 1 study of AG-120 in advanced IDH1 mutant positive chondrosarcoma at the Connective Tissue Oncology Society (CTOS) Annual Meeting on November 10, 2016 in Lisbon, Portugal.
- Preliminary data from the expansion phase of the ongoing Phase 1 study of AG-120 in advanced IDH1 mutant positive low-grade glioma at the Society for NeuroOncology (SNO) Annual Meeting on November 18, 2016 in Scottsdale, AZ.
- Updated data from the AG-348 Phase 2 DRIVE-PK study, the AG-519 Phase 1 healthy volunteer study, the Natural History Study of pyruvate kinase deficiency (PKD), and new AG-348 metabolic data in PKD patients at the American Society of Hematology (ASH) Annual Meeting on December 3-6, 2016 in San Diego, CA.
- Updated follow-up and molecular data from the completed dose escalation portion of the AG-120 Phase 1 study in R/R AML at ASH.

ADDITIONAL 2016 EXPECTED MILESTONES

IDH Mutant Inhibitors in Hematologic Malignancies:

- Complete enrollment of the 125-patient expansion cohort for the Phase 1 study of AG-120 in patients with R/R AML

IDH Mutant Inhibitors in Solid Tumors:

- Initiate a randomized study of AG-120 in IDH1 mutant positive cholangiocarcinoma

Cancer Metabolism Research:

- Initiate preclinical development activities for the first molecule in a program focused on MTAP (methylthioadenosine phosphorylase) deleted cancers

Rare Genetic Metabolic Disorders:

- Provide a development strategy update for our PKR activator program, including molecule selection
- Outline the clinical development plans for our PKR activators in beta-thalassemia

THIRD QUARTER 2016 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of September 30, 2016 were $622.6 million, compared to $375.9 million as of December 31, 2015. The increase in cash was driven by cash received from Celgene totaling $251.5 million, which includes a $200 million upfront payment from the May 2016 collaboration agreement, $25 million related to initiation of the enasidenib Phase 3 IDHENTIFY study and $26.5 million of program funding related to our collaboration agreements, and net proceeds of $162.1 million received from the company's September 2016 public offering. These items were offset by a decrease in cash related to expenditures to fund operating activities of $161.7 million and purchases of fixed assets, net of reimbursements, of $4.3 million during the nine months ended September 30, 2016.

Collaboration revenue was $9.0 million for the quarter ended September 30, 2016, compared to $5.5 million for the comparable period in 2015.

Research and development (R&D) expense was $60.6 million, including $7.9 million of stock-based compensation expense, for the quarter ended September 30, 2016, compared to $36.0 million, including $4.9 million in stock-based compensation expense, for the quarter ended September 30, 2015. 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. Celgene is responsible for all development costs for enasidenib and certain development costs for AG-881 and reimburses the company for development costs incurred for these investigational medicines.

General and administrative (G&A) expense was $11.9 million, including $4.2 million of stock-based compensation expense, for the quarter ended September 30, 2016, compared to $9.9 million, including $4.5 million of stock-based compensation expense, for the quarter ended September 30, 2015. The increase in G&A expense was largely due to increased headcount and other professional expenses to support growing operations.

Net loss for the quarter ended September 30, 2016 was $62.8 million, compared to a net loss of $40.3 million for the comparable period in 2015.

UPDATED FINANCIAL GUIDANCE FOR THE FULL YEAR 2016

As a result of the recent financing, Agios now expects to end 2016 with more than $550 million of cash, cash equivalents and marketable securities. The anticipated year-end 2016 cash position does not include any additional program-specific milestone payments from Celgene. Based on its current operating plans, the company expects that its existing cash, cash equivalents and marketable securities as of September 30, 2016, together with anticipated interest income, and anticipated expense reimbursements under our collaboration agreements with Celgene, but excluding any additional program-specific milestone payments from Celgene, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through at least the end of 2018.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss third quarter 2016 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 3681806. 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 www.agios.com.

About Agios/Celgene Collaboration
Enasidenib 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 enasidenib. Agios continues to conduct clinical development activities within the enasidenib development program and is eligible to receive up to $120 million in payments on achievement of certain milestones and royalties on net sales. Additionally, Agios has the right to co-promote enasidenib in the U.S. along with Celgene. 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 of IDH1/IDH2 and pyruvate kinase-R mutations, or other mutations, as therapeutic targets; the potential benefits of Agios' product candidates targeting IDH1/IDH2 or pyruvate kinase-R mutations or other genetic mutations, including enasidenib (AG-221), AG-120, AG-881, AG-348 and AG-519; its plans and timelines for regulatory submissions and clinical development of enasidenib (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, 2016; and the potential benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" 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 agreements 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, 2016, 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, except as required by law.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	September 30, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	$ 622,596	$ 375,907
Collaboration receivable – related party	8,182	8,225
Total assets	672,042	420,065
Deferred revenue – related party	210,356	24,364
Stockholders' equity	401,449	345,118

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Collaboration revenue – related party	$ 8,985	$ 5,480	$ 47,244	$ 52,901
Operating expenses:				
Research and development	60,643	36,028	155,485	104,894
General and administrative	11,854	9,927	35,335	25,809
Total operating expenses	72,497	45,955	190,820	130,703
Loss from operations	(63,512)	(40,475)	(143,576)	(77,802)
Interest income	678	218	1,591	692
Net loss	(62,834)	(40,257)	(141,985)	(77,110)
Net loss per share– basic and diluted	$ (1.63)	$ (1.07)	$ (3.72)	$ (2.06)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	38,548,153	37,507,298	38,124,425	37,351,493

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