Agios Reports Fourth Quarter and Full Year 2015 Financial Results and Highlights Key 2016 Milestones

February 18, 2016

- First Data from Phase 2 DRIVE PK Study for AG-348 in PK Deficiency and Phase 1 Healthy Volunteer Study for AG-519 to be Submitted for Presentation at EHA -

- Late-stage AG-221 and AG-120 IDH Program in AML on Track with 125-Patient Expansion Cohorts Enrolling and Multiple Frontline Trials Ongoing and Planned -

- First Data from AG-120 Dose Expansion Cohort in Low Grade Glioma Expected in the Second Half of 2016 -

CAMBRIDGE, Mass., Feb. 18, 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 fourth quarter and year ended December 31, 2015. In addition, Agios highlighted select corporate milestones for its preclinical and clinical development programs.

“2015 marked a year of significant achievements for Agios as we rapidly advanced our IDH inhibitors in AML, presented initial data in solid tumors and selected our fifth molecule for clinical development,” said David Schenkein, M.D., chief executive officer at Agios. “We are in a strong position entering 2016, focusing on rapid and broad late-stage clinical development for our lead IDH mutant inhibitors, executing clinical trials of our PKR activators and advancing our research programs. We look forward to several important milestones, beginning with presenting the first data from both of our PKR activators in the first half of the year. These milestones will bring us closer to our vision of helping people with cancer and rare genetic disorders.”

2016 EXPECTED MILESTONES IN CANCER METABOLISM PROGRAMS

AG-221, AG-120 and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation.

IDH Mutant Inhibitors in Hematologic Malignancies:

- Complete enrollment of both 125-patient expansion cohorts for the Phase 1/2 study of AG-221 and Phase 1 study of AG-120 in patients with relapsed/refractory (R/R) acute myeloid leukemia (AML) in the second half of 2016
- Initiate a global, registration-enabling Phase 3 study of AG-120 in frontline AML patients with an IDH1 mutation in the second half of 2016
- Initiate an expansion arm in high-risk myelodysplastic syndrome patients for AG-221 in 2016
- Initiate a Phase 1/2 frontline combination study of AG-221 or AG-120 with VIDAZA® (azacitidine) in newly diagnosed AML patients not eligible for intensive chemotherapy in the first quarter of 2016
- Continue to enroll patients in the following ongoing clinical trials:
    - Phase 3 IDHENTIFY study of AG-221 vs. standard of care chemotherapy in R/R AML
    - Phase 1b frontline combination study of AG-221 or AG-120 with standard-of-care intensive chemotherapy in AML
    - Phase 1 dose-escalation and expansion study of AG-881 in IDH mutant positive hematologic malignancies

IDH Mutant Inhibitors in Solid Tumors:

- Present data from the expansion phase of the ongoing Phase 1 study of AG-120 in advanced IDH1 mutant positive low grade glioma in the second half of 2016
- Initiate a randomized Phase 2 study of AG-120 in IDH1 mutant positive cholangiocarcinoma in the second half of 2016
- Continue to enroll patients in the following ongoing clinical trials:
    - Expansion phase of the ongoing Phase 1 study of AG-120 in advanced IDH1 mutant positive solid tumors
    - Phase 1 dose-escalation and expansion study of AG-881 in IDH mutant positive solid tumors

Cancer Metabolism Research:

- Present preclinical findings on a new research program focused on MTAP (methylthioadenosine phosphorylase) deleted cancers at the Keystone Symposia on New Frontiers in Understanding Tumor Metabolism taking place February 21-25, 2016 in Banff, Alberta, Canada
- Initiate preclinical development activities for the first molecule in the next wave of novel investigational medicines

2016 EXPECTED MILESTONES IN RARE GENETIC METABOLIC DISORDERS PROGRAMS

- Plan to submit the first data from DRIVE PK, a global Phase 2, open-label safety and efficacy trial of AG-348 in adult, transfusion-independent patients with pyruvate kinase (PK) deficiency, for presentation at the 21st Congress of the European Hematology Association (EHA) in June 2016
- Plan to submit the first data from the Phase 1 study of AG-519 in healthy volunteers for presentation at EHA in June 2016.

Preclinical findings about the molecule will also be submitted for presentation at EHA.
- Outline the clinical development plans for Agios' PKR activators in beta-thalassemia in the second half of 2016
- Present new findings from the Natural History Study of PK deficiency being conducted with Boston Children's Hospital in the second half of 2016

FOURTH QUARTER 2015 HIGHLIGHTS OF CANCER METABOLISM PROGRAMS

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

IDH Mutant Inhibitors in Hematologic Malignancies:

- New data from the dose-escalation phase and expansion cohorts from the ongoing Phase 1/2 study for AG-221 and the Phase 1 study of AG-120 were presented in December at the 2015 American Society of Hematology Annual Meeting (ASH). Read the full AG-221 data here and the AG-120 data here.
- In December, Agios initiated a Phase 1b, multicenter, international, open-label study of AG-221 or AG-120 in combination with induction and consolidation therapy in patients with newly diagnosed AML with an IDH mutation who are eligible for intensive chemotherapy.

IDH Mutant Inhibitors in Solid Tumors:

- The first data from the ongoing Phase 1 dose-escalation trial of AG-120 in advanced IDH1-mutant positive solid tumors were presented in an oral presentation at AACR-EORTC-NCI International Conference on Molecular Targets and Cancer Therapeutics in November. Read the full data here.

RECENT CORPORATE AND FINANCIAL UPDATES

Agios recently announced the appointment of Steve Hoerter to chief commercial officer. Mr. Hoerter has more than 20 years of extensive pharmaceutical and biotechnology commercial experience and most recently served as executive vice president and chief commercial officer at Clovis Oncology, Inc. Prior to Clovis, Mr. Hoerter held senior commercial roles at Genentech and Roche.

In January 2016, Agios received a $25 million milestone payment from Celgene for achievement of the first patient dosed in the Phase 3 IDHENTIFY study of AG-221 vs. standard of care chemotherapy in R/R AML. 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.

FULL YEAR 2015 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of December 31, 2015 were $375.9 million, compared to $467.4 million as of December 31, 2014. The decrease was driven by cash used to fund operating activities of approximately $161.8 million, which was offset by funding of approximately $64.7 million from Celgene during the year ended December 31, 2015 related to our collaboration agreements.

Collaboration revenue was $59.1 million for the year ended December 31, 2015, compared to $65.4 million for the prior year. Beginning in the first quarter of 2015, the company began offsetting research and development expense for amounts received from Celgene for reimbursement of certain development costs incurred on Celgene's behalf related to AG-221 which were presented as gross collaboration revenue during 2014.

Research and development (R&D) expenses were $141.8 million, including $17.4 million of stock-based compensation expense, for the year ended December 31, 2015, compared to $100.4 million, including $6.7 million in stock-based compensation expense, for the year ended December 31, 2014. The increase in R&D expenses 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 AG-221 and certain development costs for AG-120 and AG-881 and reimburses the company for development costs incurred for these investigational medicines.

General and administrative (G&A) expenses were $36.0 million, including $14.5 million of stock-based compensation expense, for the year ended December 31, 2015, compared to $19.1 million, including $4.8 million of stock-based compensation expense, for the year ended December 31, 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 year ended December 31, 2015 was $117.7 million, compared to a net loss of $53.5 million for the year ended December 31, 2014.

FINANCIAL GUIDANCE FOR THE FULL YEAR 2016

Agios announced today that it expects to end 2016 with more than $180 million of cash, cash equivalents and marketable securities. The anticipated year-end 2016 cash position does not include any additional program-specific milestone payments. The company 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. ET to discuss fourth quarter and full year 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 40733168. 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

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 (CC-90007). 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 and Celgene retains development and commercialization rights outside the U.S. 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 or other genetic 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, 2016; 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 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 September 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)

	December 31, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	$ 375,907	$ 467,447
Collaboration receivable – related party	8,225	6,492
Total assets	420,065	491,904
Deferred revenue – related party	24,364	38,411
Stockholders' equity	345,118	424,366

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

	Three Months Ended December 31,		Years Ended December 31,	
	2015	2014	2015	2014
Gross Collaboration revenue – related party (1)	$ 6,218	$ 14,636	$ 59,119	$ 65,358
Operating expenses:				
Research and development (1)	36,933	34,863	141,827	100,371
General and administrative	10,182	6,500	35,992	19,120
Total operating expenses	47,115	41,363	177,819	119,491

Loss from operations	(40,897)	(26,727)	(118,700)	(54,133)
Interest income	276	85	968	203
Loss before provision (benefit) for income taxes	(40,622)	(26,642)	(117,732)	(53,930)
Provision (benefit) for income taxes	-	22	-	(426)
Net loss	(40,621)	(26,664)	(117,732)	(53,504)
Net loss per share– basic and diluted	$ (1.08)	$ (0.76)	$ (3.15)	$ (1.59)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	37,660,033	35,121,705	37,429,262	33,667,024

Note 1 (Collaboration revenue and R&D expenses): Beginning in the first quarter of 2015, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of certain development costs incurred on Celgene's behalf related to AG-221 which were presented as gross collaboration revenue during 2014. In addition, beginning in the second quarter of 2015, the Company began offsetting R&D expense for amounts received from Celgene for reimbursement of certain development costs incurred on Celgene's behalf related to AG-120 and AG-881. The R&D expense reported for the three and twelve months ended December 31, 2015 is presented net of $8.4 million and $25.2 million, respectively, of reimbursement compared to no offset for cost reimbursement for the comparable periods in 2014.

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