Agios Reports Fourth Quarter and Full Year 2016 Financial Results and Highlights Key 2017 Milestones

February 16, 2017

- Ivosidenib (AG-120) NDA Submission for IDH1m R/R AML Planned by Year End 2017; First Phase 1 Expansion Data Expected in the Second Half of 2017 -

- AG-348 Pivotal Trial Design Update Expected in the Third Quarter of 2017; Updated DRIVE PK Data Planned for the First Half of 2017 -

- MTAP Pathway IND Submission Expected by Year End 2017; Updated Preclinical Data to be Presented at Keystone Tumor Metabolism Meeting in March -

- Company Ends 2016 in Strong Financial Position with $574M in Cash, Cash Equivalents and Marketable Securities -

CAMBRIDGE, Mass., Feb. 16, 2017 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2016. In addition, Agios highlighted select corporate milestones and data presentations for its preclinical and clinical development programs.

“Our 2016 accomplishments, including the enasidenib NDA submission with our collaboration partner Celgene and clear proof-of-concept data for our PK deficiency program, demonstrate our ability to transform our scientific discoveries into important precision medicines,” said David Schenkein, M.D., chief executive officer at Agios. “In 2017, we are focused on making the transition to a commercial stage company by delivering our lead cancer programs to patients, bringing our first rare genetic disease program into pivotal development and advancing our next research program, focused on MTAP-deleted cancers, into the clinic.”

KEY UPCOMING MILESTONES

The company expects to achieve the following key milestones:

IDH Mutant Inhibitors in Hematologic Malignancies

- Potential approval of enasidenib in the United States for IDH2m positive relapsed/refractory (R/R) acute myeloid leukemia (AML) in collaboration with Celgene by the end of 2017.
- Submit a new drug application (NDA) to the U.S. FDA for ivosidenib (AG-120) for IDH1m positive R/R AML by the end of 2017.
- Initiate a global, registration-enabling Phase 3 study (AGILE) combining ivosidenib (AG-120) and VIDAZA® in newly diagnosed AML patients with an IDH1 mutation ineligible for intensive chemotherapy in the first half of 2017.

IDH Mutant Inhibitors in Solid Tumors

- Complete the dose-escalation phase of the ongoing Phase 1 study of AG-881 in IDHm positive glioma in the first half of 2017.

Rare Genetic Diseases

- Finalize design for a global pivotal trial of AG-348 in pyruvate kinase (PK) deficiency in the third quarter of 2017.
- Initiate a global pivotal trial of AG-348 in PK deficiency in the first half of 2018.

Cancer Metabolism Research:

- Submit an Investigational New Drug (IND) application for the development candidate targeting methylthioadenosine phosphorylase (MTAP)-deleted tumors by the end of 2017. MTAP is a metabolic enzyme that is deleted in approximately 15 percent of all cancers.

ANTICIPATED 2017 DATA PRESENTATIONS

- First data from the expansion phase of the ongoing Phase 1 study of ivosidenib (AG-120) in R/R AML in the second half of 2017
- First data from the ongoing Phase 1b combination study of enasidenib or ivosidenib (AG-120) with standard-of-care intensive chemotherapy in newly diagnosed AML in the second half of 2017
- First data from the cholangiocarcinoma expansion cohort of the ongoing Phase 1 study of ivosidenib (AG-120) in advanced IDH1m positive solid tumors in the first half of 2017
- Updated data from the glioma expansion of the ongoing Phase 1 study of ivosidenib (AG-120) in advanced IDH1m positive

solid tumors in the second half of 2017
- Updated data from AG-348 Phase 2 DRIVE PK study in PK deficiency in both the first and second half of 2017
- Updated preclinical data for the program targeting MTAP-deleted tumors at the Keystone Tumor Metabolism Meeting taking place March 5-9, 2017 in Whistler, British Columbia

FOURTH QUARTER 2016 HIGHLIGHTS

- Supported Celgene's submission of an NDA for enasidenib in IDH2m positive R/R AML.
- Initiated a global, registration-enabling randomized Phase 3 study (ClarIDHy) for ivosidenib (AG-120) in IDH1m positive advanced cholangiocarcinoma. The FDA also granted ivosidenib Fast Track Designation for the treatment of patients with previously treated, unresectable or metastatic cholangiocarcinoma with an IDH1 mutation.
- Completed the dose-escalation phase of the Phase 1 study of AG-881 in IDHm positive hematologic malignancies. The study is now closed for enrollment.
- Selected a development candidate targeting MTAP-deleted tumors to enter IND-enabling studies.

FULL YEAR 2016 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of December 31, 2016 were $573.6 million, compared to $375.9 million as of December 31, 2015. This increase was driven by cash received under our collaboration agreements with Celgene totaling $258.2 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 $33.2 million of program funding, net proceeds of $162.1 million received from the company's September 2016 public offering, and $7.9 million from stock award activities. These items were offset by a decrease in cash related to expenditures to fund operating activities and purchases of fixed assets of $230.6 million during the year ended December 31, 2016.

Collaboration revenue was $69.9 million for the year ended December 31, 2016, compared to $59.1 million for the prior year.

Research and development (R&D) expenses were $220.2 million, including $25.4 million of stock-based compensation expense, for the year ended December 31, 2016, compared to $141.8 million, including $17.4 million in stock-based compensation expense, for the year ended December 31, 2015. 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 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) expenses were $50.7 million, including $16.7 million of stock-based compensation expense, for the year ended December 31, 2016, compared to $36.0 million, including $14.5 million of stock-based compensation expense, for the year ended December 31, 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 year ended December 31, 2016 was $198.5 million, compared to a net loss of $117.7 million for the year ended December 31, 2015.

CASH GUIDANCE

Based on its current operating plans, the company expects that its existing cash, cash equivalents and marketable securities as of December 31, 2016, together with anticipated interest income, and anticipated payments from Celgene under our collaboration agreements, but excluding any additional program-specific milestone payments, 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 fourth quarter and full year 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 61135222. The live webcast can be accessed under "Events & Presentations" in the Investors 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 diseases 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 focused on cancer metabolism. Under the terms of the 2010 collaboration agreement, 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 assuming achievement of certain milestones and royalties on net sales. Agios and Celgene intend to co-commercialize enasidenib in the U.S. 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. The program focused on MTAP deleted cancers is part of a 2016 global co-development and co-commercialization agreement with Celgene focused on metabolic immuno-oncology. Celgene has the option to participate in a worldwide 50/50 cost and profit share with Agios, under which Agios is eligible for up to $169 million in clinical and regulatory milestone payments for the program.

Cautionary Note Regarding Forward-Looking Statement

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 Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including enasidenib, ivosidenib (AG-120), AG-881 and AG-348; the potential benefits of Agios' product candidates; its key milestones for 2017; its plans regarding future data presentations; its financial guidance regarding the period in which it will have capital available to fund its operations; 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," "strategy," "milestone," "will," 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 or its collaborator, Celgene, 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, 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)

	December 31, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	$ 573,564	$ 375,907
Collaboration receivable – related party	4,886	8,225
Total assets	619,094	420,065
Deferred revenue – related party	190,210	24,364
Stockholders' equity	358,591	345,118

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

	Three Months Ended December 31,		Years Ended December 31,	
	2016	2015	2016	2015
Collaboration revenue – related party	$ 22,648	$ 6,218	$ 69,892	$ 59,119
Operating expenses:				
Research and development	64,678	36,933	220,163	141,827
General and administrative	15,379	10,182	50,714	35,992
Total operating expenses	80,057	47,115	270,877	177,819
Loss from operations	(57,409)	(40,897)	(200,985)	(118,700)
Interest income	923	276	2,514	968
Net loss	(56,486)	(40,621)	(198,471)	(117,732)
Net loss per share– basic and diluted	$ (1.34)	$ (1.08)	$ (5.07)	$ (3.15)

Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	42,110,541	37,660,033	39,126,400	37,429,262

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