



AgiOS Reports First Quarter 2017 Financial Results

May 4, 2017

– IDHIFA® (enasidenib) Granted Priority Review by FDA with August 30, 2017 PDUFA Date; Updated Data from Phase 1/2 Trial in IDH2m R/R AML to be Presented at ASCO –

– Ivosidenib Phase 1 125-Patient Expansion Cohort in IDH1m R/R AML Fully Enrolled; NDA Submission on Track for Year End 2017 –

– AG-348 Granted Fast Track Designation in PK Deficiency by FDA; Updated Data from DRIVE PK Trial to be Presented at EHA –

– \$287 Million Follow-on Offering Extends Cash Runway Through at Least the End of 2019 –

CAMBRIDGE, Mass., May 04, 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 first quarter ended March 31, 2017. In addition, Agios highlighted select corporate milestones and data presentations for its preclinical and clinical development programs.

“Our team made significant progress during the first quarter advancing our late-stage pipeline and preparing for the first product launch from our research and discovery engine, which is an important milestone for our organization,” said David Schenkein, M.D., chief executive officer at Agios. “We are on track to execute against our remaining key 2017 goals, including finalizing the design of our global pivotal program for AG-348 in PK deficiency in the third quarter and submitting both an NDA for ivosidenib in R/R AML and our sixth IND, for AG-270, a development candidate focused on MTAP-deleted tumors, by the end of the year.”

FIRST QUARTER 2017 HIGHLIGHTS & RECENT PROGRESS

IDH Mutant Inhibitors:

- A New Drug Application (NDA) was filed with the U.S. Food and Drug Administration (FDA) for IDHIFA® (enasidenib) in relapsed and/or refractory (R/R) acute myeloid leukemia (AML) with an isocitrate dehydrogenase 2 (IDH2) mutation; the NDA was granted Priority Review and has been given a Prescription Drug User Fee Act (PDUFA) action date of August 30, 2017.
- Completed enrollment of the 125-patient expansion cohort for the Phase 1 trial of ivosidenib in patients with IDH1m positive R/R AML.
- On April 26, 2017, the FDA granted ivosidenib Orphan Drug Designation for the treatment of cholangiocarcinoma.

Rare Genetic Diseases:

- On April 27, 2017, the FDA granted AG-348 Fast Track Designation for the treatment of patients with pyruvate kinase (PK) deficiency. Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need.
- Completed enrollment of 258 patients in the PK deficiency Natural History Study being conducted with Boston Children’s Hospital.

Research:

- Presented preclinical data on molecules with potential for the treatment of methylthioadenosine phosphorylase (MTAP) deleted tumors, at the Keystone Tumor Metabolism Meeting in Whistler, British Columbia.
- Celgene designated AG-270 for the treatment of MTAP-deleted cancers as a development candidate under the master research and collaboration agreement dated May 17, 2016, triggering an \$8 million milestone payment to Agios.
- In April, Agios and Aurigene Discovery Technologies Limited entered into a global license agreement to research, develop and commercialize small molecule inhibitors of an undisclosed cancer metabolism target.

Corporate:

- Also in April, Agios completed an underwritten public offering of 5,808,080 shares of common stock, which includes the full exercise of the underwriters’ option to purchase an additional 757,575 shares, at the offering price of \$49.50 per share, resulting in proceeds, net of underwriting discounts and commissions, of approximately \$270.2 million.

ANTICIPATED 2017 DATA PRESENTATIONS

- Updated data from the Phase 1/2 trial evaluating IDHIFA® (enasidenib) in patients with IDH2m positive R/R AML at the American Society of Clinical Oncology (ASCO) Annual Meeting being held June 2-6, 2017 in Chicago
- First data from the cholangiocarcinoma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m

positive solid tumors at ASCO

- Updated data from the AG-348 Phase 2 DRIVE PK trial in PK deficiency at the 22nd Congress of the European Hematology Association (EHA) taking place June 22-25, 2017 in Madrid, Spain
- Updated data from DRIVE PK, including longer follow-up and secondary analyses, and updated data from the PK deficiency Natural History Study in the second half of 2017
- First data from the expansion phase of the ongoing Phase 1 trial of ivosidenib in R/R AML in the second half of 2017
- First data from the ongoing Phase 1b combination trial of IDHIFA[®] (enasidenib) or ivosidenib with standard-of-care intensive chemotherapy in newly diagnosed AML in the second half of 2017
- Updated data from the glioma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m positive solid tumors in the second half of 2017

KEY UPCOMING MILESTONES

IDH Mutant Inhibitors in Hematologic Malignancies

- Potential approval and co-commercialization of IDHIFA[®] (enasidenib) in the U.S. for IDH2m positive R/R AML in collaboration with Celgene with a PDUFA action date of August 30, 2017.
- Initiate a global, registration-enabling Phase 3 study (AGILE) combining ivosidenib and VIDAZA[®] in newly diagnosed AML patients with an IDH1 mutation ineligible for intensive chemotherapy in the first half of 2017.
- Submit an NDA to the U.S. FDA for ivosidenib for IDH1m positive R/R AML by the end of 2017.

IDH Mutant Inhibitors in Solid Tumors

- Complete enrollment of 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 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.

Research:

- Submit an Investigational New Drug (IND) application for AG-270, the development candidate targeting MTAP-deleted tumors, by the end of 2017.

FIRST QUARTER 2017 FINANCIAL RESULTS

Collaboration revenue was \$10.5 million for the quarter ended March 31, 2017, compared to \$31.3 million for the comparable period in 2016. The decrease in revenue was primarily due to the fact that in the first quarter of 2016, the company recognized a \$25 million milestone payment related to the initiation of the Phase 3 IDHENTIFY trial of IDHIFA[®] (enasidenib).

Research and development (R&D) expense was \$62.7 million, including \$7.0 million of stock-based compensation expense, for the quarter ended March 31, 2017, compared to \$44.0 million, including \$5.5 million of stock-based compensation expense, for the quarter ended March 31, 2016. 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 IDHIFA[®] (enasidenib) and certain development costs for AG-881, and reimburses Agios for development costs incurred for these investigational medicines. In addition, Celgene was responsible for approximately half of development costs for ivosidenib during the quarter ended March 31, 2016. As of August 2016, Agios is responsible for all ivosidenib development costs.

General and administrative (G&A) expense was \$14.8 million, including \$3.7 million of stock-based compensation expense, for the quarter ended March 31, 2017, compared to \$10.8 million, including \$3.6 million of stock-based compensation expense, for the quarter ended March 31, 2016. The increase in G&A expense was largely due to increased headcount and additional expenses to support our growing commercial organization.

Net loss for the quarter ended March 31, 2017 was \$66.2 million, compared to a net loss of \$23.2 million for the quarter ended March 31, 2016.

Cash & Cash Guidance

Cash, cash equivalents and marketable securities as of March 31, 2017 were \$503.2 million, compared to \$573.6 million as of December 31, 2016. The decrease in cash was driven by expenditures to fund operations of \$79.4 million during the quarter ended March 31, 2017. These items were offset by an increase in cash of \$5.0 million of program funding received under our collaboration agreements with Celgene and \$4.1 million received from employee stock award transactions.

In April, Agios completed an underwritten public offering of 5,808,080 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 757,575 shares, at the offering price of \$49.50 per share, resulting in proceeds, net of underwriting discounts and commissions, of approximately \$270.2 million.

The company expects that its cash, cash equivalents and marketable securities as of March 31, 2017, together with the net proceeds from the recent financing, anticipated interest income, and anticipated payments 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 2019.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss first quarter 2017 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 13557397. 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

IDHIFA[®] (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 IDHIFA[®] (enasidenib). Agios continues to conduct clinical development activities within the IDHIFA[®] (enasidenib) development program and is eligible to receive reimbursement for those development activities and up to \$95 million in remaining payments assuming achievement of certain milestones, and royalties on net sales.

Celgene and Agios intend to co-commercialize IDHIFA[®] (enasidenib) in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts. 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 Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including IDHIFA[®] (enasidenib), ivosidenib, AG-881, AG-348 and AG-270; 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," "expect," "intend," "potential," "milestone," "goal," "will," "on track," "upcoming," 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' public filings with the Securities and Exchange Commission. 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)

	March 31, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	\$ 503,181	\$ 573,564
Collaboration receivable – related party	11,265	4,886
Total assets	557,316	619,094
Deferred revenue – related party	188,286	190,210
Stockholders' equity	307,380	358,591

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

(Unaudited)

	Three Months Ended March 31,	
	2017	2016
Collaboration revenue – related party	\$ 10,508	\$ 31,281
Operating expenses:		
Research and development	62,732	44,038
General and administrative	14,823	10,837
Total operating expenses	77,555	54,875
Loss from operations	(67,047)	(23,594)
Interest income	881	396
Net loss	\$ (66,166)	\$ (23,198)
Net loss per share – basic and diluted	\$ (1.56)	\$ (0.61)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	42,280,525	37,864,084

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