Agios Reports Second Quarter 2017 Financial Results

August 8, 2017

– IDHIFA® (enasidenib) Granted Approval from FDA as First Targeted Therapy for Patients with IDH2m R/R AML and First Product Approved from Agios' Discovery Platform –

– Trial Designs Finalized for Two Pivotal Studies for AG-348 in Pyruvate Kinase Deficiency; Studies on Track to Begin in 1H2018 –

– Initiated Ivosidenib Phase 3 Study (AGILE) in Newly Diagnosed IDH1m AML; NDA Submission for R/R AML on Track for Year End 2017 –

CAMBRIDGE, Mass., Aug. 08, 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 second quarter ended June 30, 2017. In addition, Agios highlighted select corporate milestones and preclinical and clinical data from our clinical development programs.

"Our second quarter progress was followed by a remarkable milestone for any biotechnology company, the full approval of our first product, IDHIFA ®, a treatment for patients with IDH2m R/R AML developed in partnership with Celgene," said David Schenkein, M.D., chief executive officer at Agios. "We are pursuing a similar regulatory strategy for ivosidenib, our wholly owned IDH1m inhibitor, and we remain on track to submit our NDA by year-end. With the pivotal program designed for AG-348 in PK deficiency, we are working to initiate two trials in the first half of 2018 for this rare anemia, which currently has no approved therapies."

SECOND QUARTER 2017 HIGHLIGHTS & RECENT PROGRESS

IDH Mutant Inhibitors:

- On August 1, 2017, the U.S. Food and Drug Administration (FDA) granted Celgene full approval of IDHIFA® (enasidenib) for the treatment of patients with relapsed or refractory AML (R/R AML) with an isocitrate dehydrogenase-2 (IDH2) mutation as detected by an FDA approved test. IDHIFA®, an oral targeted inhibitor of the IDH2 enzyme, is the first and only FDA-approved therapy for patients with R/R AML and an IDH2 mutation.
- Presented updated data from the Phase 1 trial of IDHIFA® in IDH2m R/R AML at the American Society of Clinical Oncology (ASCO) Annual Meeting and updated data from the Phase 1/2 trial of IDHIFA® in IDH2m R/R AML at the 22ndCongress of the European Hematology Association (EHA). The data presentations demonstrated durable complete responses in patients with IDH2m R/R AML and a safety profile consistent with previously reported data. Read the ASCO data here and the EHA data here.
- Presented the first data from the cholangiocarcinoma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m positive solid tumors at ASCO. Read the ASCO data here.
- Initiated 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.
- Completed enrollment of the dose-escalation phase of the ongoing Phase 1 study of AG-881 in IDHm positive glioma.

Rare Genetic Diseases

- Finalized two global, pivotal trial designs evaluating AG-348 in adults with pyruvate kinase (PK) deficiency:
  - A randomized, placebo-controlled trial with a 1:1 randomization expected to enroll approximately 80-100 non-transfusion dependent patients. The primary endpoint of the study is the proportion of patients who achieve at least a 1.5 gram per deciliter (g/dL) increase in hemoglobin.
  - A single arm trial of approximately 20 regularly transfused patients with a primary endpoint of reduction in transfusion burden over six months.
- Presented updated data from the AG-348 Phase 2 DRIVE PK trial in PK deficiency at EHA showing consistent safety and efficacy data. Read the EHA data here.

Discovery Research

- In April, we entered into a new global license agreement with Aurigene Discovery Technologies Limited to research, develop and commercialize small molecule inhibitors for an undisclosed cancer metabolism target.

Corporate:

- In April, Agios completed an underwritten public offering of 5,808,080 shares of common stock, which included the full exercise of the underwriters' option to purchase 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.

EXPECTED 2H 2017 DATA PRESENTATIONS

- First preclinical data of AG-881 in IDHm solid and hematologic malignancies have been submitted for presentation at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in October.
- Updated data from the glioma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m positive solid tumors have been submitted for presentation at the 2017 Society for NeuroOncology Annual Meeting in November.
- First data from the expansion phase of the ongoing Phase 1 trial of ivosidenib in IDH1m R/R AML and advanced hematologic malignancies have been submitted for presentation at the 2017 American Society of Hematology Annual Meeting and Exposition (ASH) in December.
- First data from the ongoing Phase 1 combination trial of IDHIFA® or ivosidenib with standard-of-care intensive chemotherapy ("7 +3" and consolidation) in patients with newly diagnosed AML with an IDH2 or IDH1 mutation have been submitted for presentation at ASH.
- Updated data from the Phase 2 DRIVE PK trial with AG-348 in patients with PK deficiency, including longer follow-up and secondary analyses, have been submitted for presentation at ASH.
- Updated data from the PK Deficiency Natural History Study being conducted with Boston Children's Hospital have been submitted for presentation at ASH.

KEY UPCOMING MILESTONES

The company expects to achieve the following key milestones:

- Submit an NDA (New Drug Application) to the U.S. FDA for ivosidenib for IDH1m positive R/R AML by the end of 2017.
- Initiate two global, pivotal trials of AG-348 in PK deficiency in the first half of 2018.
- Submit an Investigational New Drug (IND) application for AG-270, the development candidate targeting MTAP-deleted tumors, by the end of 2017.

SECOND QUARTER 2017 FINANCIAL RESULTS

Collaboration revenue was $11.3 million for the quarter ended June 30, 2017, compared to $7.0 million for the comparable period in 2016. Collaboration revenue increased compared to the prior year period partially due to reimbursement by Celgene of our share of the commercialization effort for IDHIFA®.

Research and development (R&D) expense was $79.8 million, including $8.2 million of stock-based compensation expense, for the quarter ended June 30, 2017, compared to $50.8 million, including $6.6 million in stock-based compensation expense, for the quarter ended June 30, 2016. The increase in R&D expense was primarily attributable to activities related to the ivosidenib program, including manufacturing-related activities needed to prepare for a potential NDA submission in 2017, start-up costs for the Phase 3 AGILE clinical trial, and on-going site activation and patient enrollment of the Phase 3 ClarIDHy clinical trial. In addition, Celgene was responsible for approximately half of the development costs for ivosidenib during the quarter ended June 30, 2016. As of August 2016, Agios is responsible for all ivosidenib development costs. R&D expense also increased compared to the quarter ended June 30, 2016 due to preparations for initiation of the AG-348 pivotal program in the first half of 2018, including manufacturing-related activities. Lastly, the $3.0 million upfront payment as part of the Aurigene license agreement was included in R&D during the quarter ended June 30, 2017.

General and administrative (G&A) expense was $16.1 million, including $4.0 million stock-based compensation expense, for the quarter ended June 30, 2017, compared to $12.6 million, including $4.4 million of stock-based compensation expense, for the quarter ended June 30, 2016. The increase in G&A expense was attributed to an increase of $1.0 million in personnel costs related to an increase in our internal headcount and an increase of $1.9 million related to support our growing commercial organization.

Net loss for the quarter ended June 30, 2017 was $83.1 million, compared to a net loss of $56.0 million for the quarter ended June 30, 2016.

Cash, cash equivalents and marketable securities as of June 30, 2017 were $715.9 million, compared to $573.6 million as of December 31, 2016. The increase in cash was driven by net proceeds of $270.2 million from the April financing, $8.1 million of program funding received under our collaboration agreements with Celgene and $6.8 million received from employee stock award transactions. This was offset by expenditures to fund operations of $142.5 million during the six months ended June 30, 2017.

The company expects that its cash, cash equivalents and marketable securities as of June 30, 2017, together with anticipated interest income, and anticipated expense reimbursements 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 second 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 61600194. 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 IDHIFA®

IDHIFA® (enasidenib) is indicated for the treatment of adult patients with relapsed or refractory acute myeloid leukemia with an isocitrate dehydrogenase-2 (IDH2) mutation as detected by an FDA-approved test.

Important Safety Information

WARNING: DIFFERENTIATION SYNDROME

Patients treated with IDHIFA have experienced symptoms of differentiation syndrome, which can be fatal if not treated. Symptoms may include fever, dyspnea, acute respiratory distress, pulmonary infiltrates, pleural or pericardial effusions, rapid weight gain or peripheral edema, lymphadenopathy, bone pain, and hepatic, renal, or multi-organ dysfunction. If differentiation syndrome is suspected, initiate corticosteroid therapy and hemodynamic monitoring until symptom resolution.

WARNINGS AND PRECAUTIONS

Differentiation Syndrome: See Boxed WARNING. In the clinical trial, 14% of patients treated with IDHIFA experienced differentiation syndrome. Differentiation syndrome has been observed with and without concomitant hyperleukocytosis, as early as 10 days and at up to 5 months after IDHIFA initiation. If differentiation syndrome is suspected, initiate systemic corticosteroids and hemodynamic monitoring until improvement. Taper corticosteroids only after resolution of symptoms. Differentiation syndrome symptoms may recur with premature discontinuation of corticosteroids. If severe pulmonary symptoms requiring intubation or ventilator support and/or renal dysfunction persist for more than 48 hours after initiation of corticosteroids, interrupt IDHIFA until signs and symptoms are no longer severe. Hospitalization for close observation and monitoring of patients with pulmonary and/or renal manifestation is recommended.

Embryo-Fetal Toxicity: Based on animal embryo-fetal toxicity studies, IDHIFA can cause embryo-fetal harm when administered to a pregnant woman. Advise females of reproductive potential and males with female partners of reproductive potential to use effective contraception during treatment with IDHIFA and for at least 1 month after the last dose. Pregnant women, patients becoming pregnant while receiving IDHIFA, or male patients with pregnant female partners should be apprised of the potential risk to the fetus.

ADVERSE REACTIONS

- The most common adverse reactions (≥20%) included total bilirubin increased (81%), calcium decreased (74%), nausea (50%), diarrhea (43%), potassium decreased (41%), vomiting (34%), decreased appetite (34%), and phosphorus decreased (27%)
- The most frequently reported ≥Grade 3 adverse reactions (≥5%) included total bilirubin increased (15%), potassium decreased (15%), phosphorus decreased (8%), calcium decreased (8%), diarrhea (8%), differentiation syndrome (7%), non-infectious leukocytosis (6%), tumor lysis syndrome (6%), and nausea (5%)
- Serious adverse reactions were reported in 77.1% of patients. The most frequent serious adverse reactions (≥2%) were leukocytosis (10%), diarrhea (6%), nausea (5%), vomiting (3%), decreased appetite (3%), tumor lysis syndrome (5%), and differentiation syndrome (8%). Differentiation syndrome events characterized as serious included pyrexia, renal failure acute, hypoxia, respiratory failure, and multi-organ failure

LACTATION

Many drugs are excreted in human milk and because of the potential for adverse reactions in breastfed infants, advise women not to breastfeed during treatment with IDHIFA and for at least 1 month after the last dose.

Please see full Prescribing Information, including Boxed WARNING

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 certain 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 any net sales. Celgene and Agios are currently co-commercializing 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)

	June 30, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	$ 715,941	$ 573,564
Collaboration receivable – related party	4,842	4,886
Total assets	760,600	619,094
Deferred revenue – related party	179,026	190,210
Stockholders' equity	508,992	358,591

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue – related party	$ 11,346	$ 6,978	$ 21,854	38,259
Operating expenses:				
Research and development	79,816	50,804	142,548	94,842
General and administrative	16,130	12,644	30,953	23,481
Total operating expenses	95,946	63,448	173,501	118,323
Loss from operations	(84,600)	(56,470)	(151,647)	(80,064)
Interest income	1,518	517	2,399	913
Net loss	$ (83,082)	$ (55,953)	$ (149,248)	$ (79,151)
Net loss per share – basic and diluted	$ (1.78)	$ (1.47)	$ (3.35)	$ (2.09)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	46,745,760	37,956,383	44,525,478	37,910,233

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