Agios Reports Third Quarter 2017 Financial Results

November 1, 2017

IDHIFA® Approval for IDH2m R/R AML Validates Precision Medicine Approach; Ivosidenib NDA Submission for IDH1m R/R AML on Track for Year End 2017

First Expansion Data from Ivosidenib Phase 1 Trial in IDH1m R/R AML and First Data from Phase 1 Combination Trials in Newly Diagnosed IDHm AML Accepted for Presentation at ASH

IND Submission for AG-270 Targeting MTAP-deleted Tumors on Track for Year End 2017

CAMBRIDGE, Mass., Nov. 01, 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 third quarter ended September 30, 2017. In addition, Agios highlighted select corporate milestones and preclinical and clinical data from its development programs.

"We achieved two key 2017 goals in the third quarter with the approval and launch of IDHIFA ® with our partner Celgene for patients with IDH2m R/R AML and the design completion of the AG-348 pivotal program in PK deficiency," said David Schenkein, M.D., chief executive officer at Agios. "We are now focused on completing the NDA for our first wholly owned product, ivosidenib for IDH1m R/R AML and will present the core data from the submission next month at ASH. In addition, the year-end submission of our IND for AG-270 targeting MTAP-deleted tumors remains on track, highlighting our commitment to remain a research-focused organization pursuing novel science with the potential to change patients' lives."

THIRD QUARTER 2017 HIGHLIGHTS & RECENT PROGRESS

- The U.S. Food and Drug Administration (FDA) granted Celgene full approval of IDHIFA® (enasidenib) for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (R/R AML) with an isocitrate dehydrogenase-2 (IDH2) mutation as detected by an FDA-approved diagnostic 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.
- 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 the first preclinical data for AG-881 in IDHm solid and hematologic malignancies at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in October. The data support that AG-881 potently suppresses 2-hydroxyglutarate (2-HG) production by both IDH1 (isocitrate dehydrogenase-1) and IDH2 mutant proteins in biochemical, cell-based and in vivo systems.

FOURTH QUARTER 2017 DATA PRESENTATIONS

IDH Mutant Inhibitors:

- Updated data from the glioma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m positive solid tumors at the 2017 Society for NeuroOncology Annual Meeting on November 17 in San Francisco.
- First data from the expansion phase of the ongoing Phase 1 trial of ivosidenib in IDH1m R/R AML and advanced hematologic malignancies at the 2017 American Society of Hematology Annual Meeting and Exposition (ASH) on December 9-12 in Atlanta.
- First data from the ongoing Phase 1 combination trial of enasidenib or ivosidenib with standard-of-care intensive chemotherapy ("7 +3" and consolidation) in patients with newly diagnosed AML with an IDH2 or IDH1 mutation at ASH.
- First data from the ongoing Phase 1/2 combination trial of enasidenib or ivosidenib with VIDAZA® in patients with newly diagnosed AML with an IDH2 or IDH1 mutation ineligible for intensive chemotherapy at ASH.

Rare Genetic Diseases:

- Updated data from the AG-348 Phase 2 DRIVE PK study in PK deficiency at ASH.
- Updated data from the PK Deficiency Natural History Study being conducted with Boston Children's Hospital at ASH.

KEY UPCOMING MILESTONES

The company expects to achieve the following 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.
- Submit an Investigational New Drug (IND) application for AG-270, the development candidate targeting MTAP-deleted tumors, by the end of 2017.
- Initiate two global, pivotal trials of AG-348 in PK deficiency in the first half of 2018.
- Initiate a global registry for adult and pediatric patients with PK deficiency in the first half of 2018. The registry will include approximately 60 sites in 20 countries and will follow patients for at least two years.
- Initiate a perioperative 'window' study with ivosidenib and AG-881 in low grade glioma to further investigate their effects on brain tumor tissue in the first half of 2018.

THIRD QUARTER 2017 FINANCIAL RESULTS

Revenue for the quarter ended September, 30, 2017 was $11.4 million, which includes $10.7 million of collaboration revenue and $0.7 million of royalty revenue from net sales of IDHIFA®. Revenue for the comparable period in 2016, was $9.0 million. Revenue increased compared to the prior year period primarily due to reimbursement by Celgene of our share of the commercialization effort for IDHIFA® and the IDHIFA® royalty revenue.

Research and development (R&D) expense was $72.9 million, including $7.6 million of stock-based compensation expense, for the quarter ended September 30, 2017, compared to $60.6 million, including $7.9 million in stock-based compensation expense, for the quarter ended September 30, 2016. The increase in R&D expense was primarily attributable to the ivosidenib program, including 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. R&D expense also increased compared to the quarter ended September 30, 2016 due to IND enabling activities for AG-270 as well as ongoing research efforts across our discovery platform programs.

General and administrative (G&A) expense was $17.5 million, including $4.6 million stock-based compensation expense, for the quarter ended September 30, 2017, compared to $11.9 million, including $4.2 million of stock-based compensation expense, for the quarter ended September 30, 2016. The increase in G&A expense was attributed to an increase of $5.7 million related to support our growing commercial organization for the launch of IDHIFA® and the potential launch of ivosidenib in 2018.

Net loss for the quarter ended September 30, 2017 was $77.1 million, compared to a net loss of $62.8 million for the quarter ended September 30, 2016.

Cash, cash equivalents and marketable securities as of September 30, 2017 were $641.7 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, $12.3 million of cost reimbursements related to our collaboration agreements with Celgene and $12.4 million received from employee stock transactions. This was offset by expenditures to fund operations of $226.4 million during the nine months ended September 30, 2017.

The company expects that its cash, cash equivalents and marketable securities as of September 30, 2017, together with anticipated interest income, anticipated expense reimbursements, and royalty 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 third 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 99771605. 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 an approved oncology precision medicine and multiple first-in-class investigational therapies 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 (methylthioadenosine phosphorylase)-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 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 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 and 2018; 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)

	September 30, 2017	December 31, 2016
Cash, cash equivalents, and marketable securities	$ 641,732	$ 573,564
Collaboration receivable – related party	3,489	4,886
Royalty receivable – related party	715	—
Total assets	687,200	619,094
Deferred revenue – related party	170,069	190,210
Stockholders' equity	449,806	358,591

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Collaboration revenue – related party	$ 10,643	$ 8,985	$ 32,497	$ 47,244
Royalty revenue – related party	715	—	715	—
Total revenue	11,358	8,985	33,212	47,244
Operating expenses:				
Research and development	72,917	60,643	215,465	155,485
General and administrative	17,458	11,854	48,411	35,335
Total operating expenses	90,375	72,497	263,876	190,820
Loss from operations	(79,017)	(63,512)	(230,664)	(143,576)
Interest income	1,880	678	4,279	1,591
Net loss	$ (77,137)	$ (62,834)	$ (226,385)	$ (141,985)
Net loss per share – basic and diluted	$ (1.59)	$ (1.63)	$ (4.94)	$ (3.72)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	48,459,424	38,548,153	45,851,203	38,124,425

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