Agios Reports First Quarter 2016 Financial Results

May 5, 2016

Five Abstracts Accepted for Presentation at EHA in June, Including First Data from AG -348 Phase 2 DRIVE PK Study in PK Deficiency and AG-519 Phase 1 Healthy Volunteer Study
Enrollment Complete in AG-221 Phase 2 Expansion Cohort in Relapsed/Refractory (R/R) Acute Myeloid Leukemia (AML)

CAMBRIDGE, Mass., May 05, 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 first quarter ended March 31, 2016.

"We have been focused on executing against the important milestones we laid out in January, and I'm proud of the progress across both our cancer and rare genetic disorders programs to date," said David Schenkein, M.D., chief executive officer at Agios. "Notably, enrollment is complete ahead of schedule for the Phase 2 expansion cohort for the Phase 1/2 study of AG-221 in advanced AML. This achievement is crucial to getting this potential new therapy to patients as quickly as possible. Additionally, we are pleased that the first data from our Phase 2 DRIVE PK study of AG-348 will be presented at EHA this June, along with the first data from the Phase 1 study of AG-519 in healthy volunteers."

FIRST QUARTER 2016 HIGHLIGHTS & RECENT PROGRESS

PKR Activators:

- An abstract for 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 has been accepted for presentation at the 21st Congress of the European Hematology Association (EHA) in June 2016. An abstract for preclinical data for AG-348 in beta-thalassemia has also been accepted for presentation at EHA.
- Three abstracts on AG-519, including from the Phase 1 study in healthy volunteers and preclinical findings on the molecule, have been accepted for presentation at EHA.

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

IDH Mutant Inhibitors in Hematologic Malignancies:

- Completed enrollment of the Phase 2 expansion cohort for the Phase 1/2 study of AG-221 in patients with R/R AML in May 2016
- Initiated 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 March 2016
- Received EMA Orphan Drug Designation for AG-221 for the treatment of AML in April 2016

Cancer Metabolism Research:

- In April, Agios published preclinical findings from its program focused on MTAP (methylthioadenosine phosphorylase) deleted cancers in the peer-reviewed journal *Cell Reports*

2016 EXPECTED MILESTONES IN CANCER METABOLISM PROGRAMS

IDH Mutant Inhibitors in Hematologic Malignancies:

- Complete enrollment of the 125-patient expansion cohort for the Phase 1 study of AG-120 in patients with R/R 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
- 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 1/2 frontline combination study of AG-221 or AG-120 with VIDAZA® 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:

- Initiate preclinical development activities for the first molecule in the MTAP program in 2016

2016 EXPECTED MILESTONES IN RARE GENETIC METABOLIC DISORDERS PROGRAMS

- Present new findings from the Natural History Study of PK deficiency being conducted with Boston Children's Hospital in the second half of 2016
- Outline the clinical development plans for Agios' PKR activators in beta-thalassemia in the second half of 2016

FIRST QUARTER 2016 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of March 31, 2016 were $355.8 million, compared to $375.9 million as of December 31, 2015. The decrease was driven by cash expenditures to fund operating activities of $54.1 million, which was offset by funding of $35.1 million from Celgene during the quarter ended March 31, 2016 related to our collaboration agreements.

Collaboration revenue was $31.3 million for the quarter ended March 31, 2016, compared to $34.2 million for the comparable period in 2015. In the first quarter of 2016, the Company received and recognized as revenue $25.0 million related to a substantive clinical development milestone for the AG-221 program.

Research and development (R&D) expense was $44.0 million, including $5.5 million of stock-based compensation expense, for the quarter ended March 31, 2016, compared to $32.4 million, including $2.6 million in stock-based compensation expense, for the quarter ended March 31, 2015. 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 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) expense was $10.8 million, including $3.6 million of stock-based compensation expense, for the quarter ended March 31, 2016, compared to $7.0 million, including $2.4 million of stock-based compensation expense, for the quarter ended March 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 quarter ended March 31, 2016 was $23.2 million, compared to a net loss of $5.0 million for the quarter ended March 31, 2015.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:30 a.m. ET to discuss first quarter 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 4063167. 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 of IDH1/IDH2 and pyruvate kinase-R mutations as therapeutic targets; 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; 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" 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' Annual Report on Form 10-K for the year ended December 31, 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, except as required by law.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	March 31, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	$ 355,754	$ 375,907
Collaboration receivable – related party	6,814	8,225
Total assets	396,117	420,065
Deferred revenue – related party	18,472	24,364
Stockholders' equity	332,373	345,118

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

	Three Months Ended March 31,	
	2016	2015
Collaboration revenue – related party	$ 31,281	$ 34,202
Operating expenses:		
Research and development (net of $8,794 and $4,366 of cost reimbursement from related party for the three months ended March 31, 2016 and 2015, respectively)	44,038	32,443
General and administrative	10,837	6,954
Total operating expenses	54,875	39,397
Loss from operations	(23,594)	(5,195)
Interest income	396	238
Net loss	$ (23,198)	$ (4,957)
Net loss per share – basic and diluted	$ (0.61)	$ (0.13)
Weighted-average number of common shares used in net loss per share – basic and diluted	37,864,084	37,214,747

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