Agios Reports Second Quarter 2016 Financial Results

August 4, 2016

- Data from PKR Activators at EHA Demonstrated Proof-of-Concept for AG-348 and Proof-of-Mechanism for AG-519; Program Moving Toward Pivotal Development in PK Deficiency with Molecule Selection by Year-End 2016 -

- $200 Million Upfront Payment Received for New Metabolic Immuno-Oncology Collaboration with Celgene -

CAMBRIDGE, Mass., Aug. 04, 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 second quarter ended June 30, 2016.

"Second quarter achievements marked tremendous progress towards our 2016 goals across our research and development programs," said David Schenkein, M.D., chief executive officer at Agios. "At EHA, we established clear proof-of-concept for AG-348 in pyruvate kinase deficiency, validating our novel approach to treat rare genetic metabolic disorders by correcting the underlying enzymatic defect and potentially establishing the first treatment for patients with this serious disease. This milestone sets the stage for pivotal development of our PKR activator program. In addition, our new strategic collaboration with Celgene enables Agios to expand into the emerging field of metabolic immuno-oncology, an important new field of cancer research. With full worldwide rights for AG-120, we now have another wholly owned investigational medicine we discovered with the potential to benefit patients waiting for better therapies. As we move into the second half of the year, our focus is on the execution of our late-stage programs and several clinical updates across both IDH and PKR portfolios."

SECOND QUARTER 2016 HIGHLIGHTS & UPDATES

PKR Activator Program for the Treatment of Pyruvate Kinase Deficiency:

Data presented at the 21st Congress of the European Hematology Association (EHA) in June validated Agios' novel approach to the treatment of rare genetic metabolic disorders by correcting the underlying enzymatic defect with a small molecule.

- AG-348 achieved proof-of-concept with rapid and sustained hemoglobin increases in the ongoing Phase 2 DRIVE-PK study of patients with pyruvate kinase (PK) deficiency. Read the AG-348 data from EHA here.
- Initial data from the AG-519 Phase 1 integrated single ascending dose (SAD) and multiple ascending dose (MAD) clinical trial in healthy volunteers provided proof-of-mechanism. As reported at EHA, one subject from the MAD portion of the study experienced a reversible Grade 2 platelet reduction consistent with drug-induced immune thrombocytopenia. Since the EHA presentation data cut-off, 18 additional healthy volunteers have been dosed with AG-519 for 14 days and there have been no additional cases of thrombocytopenia. Read the AG-519 data from EHA here.

Corporate:

In May, Agios and Celgene announced a new global strategic collaboration focused on metabolic immuno-oncology, an emerging field of cancer research focused on altering the metabolic state of immune cells to enhance the body's immune response to cancer.

- Agios received an upfront cash payment of $200 million and as a result updated expected 2016 ending cash, cash equivalents and marketable securities to more than $390 million. This revised cash balance is expected to be sufficient to fund Agios' operating expenses and capital expenditure requirements through mid-2018.
- The companies also amended certain rights from their 2010 collaboration agreement, including the transfer of ex-U.S. development and commercialization rights of AG-120 to Agios. For more information on the May 2016 agreement, read here.

2016 EXPECTED MILESTONES IN CANCER METABOLISM

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 relapsed/refractory acute myeloid leukemia (R/R AML) in the second half of the year
- Plan to present updated data from the completed dose escalation portion of the AG-120 Phase 1 study in R/R AML in the second half of the year
- Initiate an expansion arm in high-risk myelodysplastic syndrome patients for AG-221 in the second half of the year
- 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:

- Plan to 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 the year
- Initiate a randomized Phase 2 study of AG-120 in IDH1 mutant positive cholangiocarcinoma in the second half of the year
- 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 a program focused on MTAP (methylthioadenosine phosphorylase) deleted cancers

2016 EXPECTED MILESTONES IN RARE GENETIC METABOLIC DISORDERS

- Plan to present updated data from the AG-348 Phase 2 DRIVE-PK study, the AG-519 Phase 1 healthy volunteer study, and the Natural History Study of PK deficiency in the second half of the year
- Provide a development strategy update for our PKR activator program, including molecule selection, in the second half of the year
- Outline the clinical development plans for our PKR activators in beta-thalassemia in the second half of the year

SECOND QUARTER 2016 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of June 30, 2016 were $512.3 million, compared to $375.9 million as of December 31, 2015. The increase in cash was driven by cash received from Celgene totaling $243.5 million, which included a $200 million upfront payment from the May 2016 collaboration agreement, $25 million related to a substantive clinical development milestone for the AG-221 program and $18.5 million of program funding related to our collaboration agreements. The cash received from Celgene was offset by a decrease in cash related to expenditures to fund operating activities of $104.7 million during the six months ended June 30, 2016.

Collaboration revenue was $7.0 million for the quarter ended June 30, 2016, compared to $13.2 million for the comparable period in 2015. For the second quarter of 2015, collaboration revenue included $8.8 million related to the delivery of the U.S and ex-U.S. licenses for AG-881.

Research and development (R&D) expense was $50.8 million, including $6.6 million of stock-based compensation expense, for the quarter ended June 30, 2016, compared to $36.4 million, including $4.6 million in stock-based compensation expense, for the quarter ended June 30, 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-881 and reimburses the company for development costs incurred for these investigational medicines.

General and administrative (G&A) expense was $12.6 million, including $4.4 million of stock-based compensation expense, for the quarter ended June 30, 2016, compared to $8.9 million, including $3.6 million of stock-based compensation expense, for the quarter ended June 30, 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 June 30, 2016 was $56.0 million, compared to a net loss of $31.9 million for the comparable period in 2015.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:30 a.m. ET to discuss second 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 56383998. 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 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. Additionally, Agios has the right to co-promote AG-221 in the U.S. along with Celgene. 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; its financial guidance regarding the amount of cash, cash equivalents and marketable securities that the company will have as of December 31, 2016; 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' Quarterly Report on Form 10-Q for the quarter ended March 31, 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)

	June 30, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	$ 512,295	$ 375,907
Collaboration receivable – related party	7,853	8,225
Total assets	557,601	420,065
Deferred revenue – related party	215,009	24,364
Stockholders’ equity	289,078	345,118

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Collaboration revenue – related party	$ 6,978	$ 13,219	$ 38,259	$ 47,421
Operating expenses:				
Research and development	50,804	36,423	94,842	68,866
General and administrative	12,644	8,929	23,481	15,883
Total operating expenses	63,448	45,352	118,323	84,749
Loss from operations	(56,470)	(32,133)	(80,064)	(37,328)
Interest income	517	236	913	474
Net loss	(55,953)	(31,897)	(79,151)	(36,854)
Net loss per share– basic and diluted	$ (1.47)	$ (0.85)	$ (2.09)	$ (0.99)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	37,956,383	37,329,220	37,910,233	37,272,300

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