Agios Pharmaceuticals Reports Fourth Quarter and Full Year 2014 Financial Results

February 17, 2015

Positive early clinical results in three distinct investigational medicines and a strong financial position support advancement of the Agios portfolio into late stage development

CAMBRIDGE, Mass., Feb. 17, 2015 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq:AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2014. In addition, Agios highlighted selected corporate milestones and updated guidance for its lead clinical development programs:

- AG-221 global registration program in hematologic malignancies expected to commence in second half 2015
- AG-120 Phase 1 expansion cohorts in hematologic malignancies expected to start in first half 2015, and first data from AG-120 Phase 1 solid tumor study expected to be presented in second half 2015
- AG-348 Phase 2 study in patients with pyruvate kinase deficiency expected to start in first half 2015

"Since our inception, we have remained steadfast in our commitment to patients, our vision and our culture in order to fundamentally change patients lives through our scientific leadership in dysregulated metabolism and development using precision medicine," said David Schenkein, M.D., chief executive officer of Agios. "Early data presented in 2014 for our three distinct investigational medicines provide validation of precision medicine development and the promise this targeted approach could have in helping patients. In 2015, we are focused on three key priorities: executing on our planned registration programs, building out our capabilities and continuing to invest in research. This is an exciting time at Agios and, with a clear path forward, we are on the way to realizing our vision of building a multi-product biopharmaceutical company."

Dr. Schenkein continued, "I am also thrilled to welcome to our leadership team Megan Pace as senior vice president of strategic operations and communications and Melissa McLaughlin as vice president of human resources. Megan was senior vice president of corporate communications at Vertex Pharmaceuticals. She served on their senior management team, where she led global corporate and employee communications programs as the company prepared for and successfully launched its first two medicines. Melissa was the former vice president, human resources at Expedia, Inc., the world's largest online travel company. She brings unique strengths to Agios with experience in managing a global employee base within a fast growing company. Both Megan and Melissa share our passion for patients and culture and have deep experience at building out teams and addressing challenges during significant growth and transformation."

RECENT DEVELOPMENT PROGRAM AND BUSINESS HIGHLIGHTS

Cancer Metabolism: IDH Mutant Inhibitors in Collaboration with Celgene

AG-221: a first-in-class, oral, selective, potent inhibitor of the mutated IDH2 protein being evaluated in a broad range of blood and solid tumor cancers

- Agios continues to conduct the Phase 1 study in IDH2-mutant positive advanced hematologic malignancies, including acute myeloid leukemia (AML), with the primary goals of establishing the safety profile, determining the maximum tolerated dose (MTD) and assessing the clinical activity of AG-221 as a single agent. In parallel, the company continues to enroll patients in four expansion cohorts of 25 patients each as part of the Phase 1 study to assess the safety and tolerability of AG-221 once daily in patients with IDH2-mutant positive hematologic malignancies.
- In December 2014 at the 56th Annual American Society of Hematology (ASH) Annual Meeting, new data in 73 patients with several types of advanced hematologic malignancies, including AML, were presented from the ongoing Phase 1 dose escalation study of AG-221. The data showed a favorable safety profile of AG-221 as a single agent as well as durable clinical activity for up to eight months and ongoing, with an overall response rate of 56 percent (25 of 45 evaluable) in these patients.
- In October 2014, Agios initiated a Phase 1/2 trial of AG-221 in patients with advanced solid tumors, including gliomas, as well as angioimmunoblastic T-cell lymphoma (AITL). This is designed to explore AG-221's potential beyond hematologic malignancies in a broad range of cancers that harbor an IDH2 mutation.

AG-120: a first-in-class, oral, selective, potent inhibitor of the mutated IDH1 protein being evaluated in a broad range of blood and solid tumor cancers

- Agios continues to advance two separate Phase 1 clinical trials evaluating AG-120 in patients with IDH1-mutant advanced hematologic malignancies and solid tumors, including glioma. The Phase 1 trials are multicenter, open-label, dose-escalation clinical studies designed to assess the safety and tolerability of AG-120 as a single agent in these cancers.
- In November 2014, the first data from the ongoing Phase 1 study of AG-120 in patients with IDH1-mutant advanced hematologic malignancies were presented at the 26th Annual EORTC-NCI-AACR Symposium. The data from 17 patients with relapsed and/or refractory AML showed a promising favorable safety profile, objective responses in seven out of 14 evaluable patients, including four complete remissions, early evidence of durability and a reduction of the 2HG biomarker. These results provided early validation of mutant IDH1 as a target in AML.

- In January 2015, Celgene agreed that it would exercise its option to obtain an exclusive license outside the United States in accordance with the terms of collaboration agreement between the parties, subject to receipt of any required regulatory approvals, including any applicable clearance under the Hart-Scott-Rodino Act. Agios retains U.S. development and commercial rights to AG-120.

Rare Genetic Disorders of Metabolism: Wholly Owned PKR Activator

AG-348: a novel, first-in-class, oral activator of pyruvate kinase-R (PKR) for the treatment of pyruvate kinase (PK) deficiency

- Dosing has been completed in the Phase 1 multiple ascending dose (MAD) clinical trial of AG-348 in healthy volunteers.
- In December 2014 at the 56th Annual ASH Annual Meeting, Agios researchers presented final clinical data from its Phase 1 single ascending dose (SAD) clinical trial and data from the first two cohorts of the Phase 1 MAD clinical trial of AG-348 in healthy volunteers. The data showed early proof-of-mechanism for AG-348 through substantial effects on two key biomarkers of pyruvate kinase activity and pathway activation.
- A natural history study of PK deficiency is also ongoing and has enrolled approximately 100 patients in more than 20 clinical sites globally. Natural history studies are important to confirm and further understand clinical characteristics, symptoms and disease complications and potentially support the design of future clinical trials.

Business Highlights

- Agios announced today that the company has expanded its leadership team. Agios appointed Megan Pace to the newly created position of senior vice president of strategic operations and communications. Prior to joining Agios, Ms. Pace was senior vice president, corporate communications at Vertex Pharmaceuticals. Before that, she was senior director, public affairs and advocacy relations at Genentech, Inc. Agios also appointed Melissa McLaughlin to vice president of human resources (HR). Ms. McLaughlin was former vice president, HR for Hotels.com and the Expedia Affiliate Network. Before that, she held various HR leadership positions at Johnson & Johnson and The Gillette Company.
- In December 2014, Celgene elected to extend the period of its exclusivity for an additional year to April 2016 under its global strategic collaboration agreement with Agios. The extension marks the final year for the discovery phase of the collaboration and gives Celgene an exclusive option to certain drug candidates generated by Agios' cancer metabolism platform during that time. Agios will receive a $20 million payment as a result of the extension, which it expects to receive in the second quarter of 2015.
- In December 2014, Agios strengthened its board of directors. Its board elected Kaye Foster-Cheek as an independent director and a member of its compensation committee. Ms. Foster-Cheek is the former senior vice president, global human resources at Onyx Pharmaceuticals, Inc. an Amgen subsidiary.

UPCOMING MILESTONES

Cancer Metabolism Program in Collaboration with Celgene

AG-221: Multiple studies of AG-221 planned for 2015 support speed and breadth in development for patients with cancers that carry an IDH2 mutation

- **Phase 1 trial in advanced hematologic malignancies**: Beginning in mid-2015, Agios expects to present new data from the ongoing Phase 1 trial at medical conferences in the year, including early data from the ongoing expansion cohorts, as well as molecular information from patient data.
- **Global registration program:** The company expects to initiate in the second half of 2015 a global registration program in hematologic malignancies.
- **Frontline therapy trials:** Also in the second half of 2015, Agios plans to initiate combination trials to evaluate AG-221 as a potential frontline treatment for patients with AML and a broad range of hematologic malignancies.
- **Phase 1/2 solid tumor study:** In 2015, Agios expects to continue dose escalation in the Phase 1/2 trial in patients with advanced solid tumors that carry an IDH2 mutation.

AG-120: Multiple studies planned for 2015/2016 support speed and breadth in development for patients with an IDH1 mutation

- **Phase 1 trial in advanced hematologic malignancies**: The company expects to provide new data from the ongoing Phase 1 study evaluating patients with IDH1 mutant positive advanced hematologic malignancies at a medical conference in mid-2015.
- **Phase 1 expansion cohorts in hematologic malignancies:** Agios plans to select a dose and schedule from the ongoing Phase 1 study and initiate expansion cohorts for AG-120 in hematologic malignancies as part of its ongoing Phase 1 study in the first half of 2015.
- **Phase 1 solid tumor trial:** The company expects to present the first data from the dose escalation portion of the Phase 1 advanced solid tumor trial at a medical conference in the second half of 2015.
- **Frontline therapy trials:** Agios plans to begin combination trials to evaluate AG-120 as a potential frontline treatment of AML and a broad range of hematologic malignancies in the second half of 2015.
- **Global registration program:** The company plans to initiate a global registration program in hematologic malignancies

that harbor an IDH1 mutation by early 2016.

Rare Genetic Disorders of Metabolism

AG-348: First patients with pyruvate kinase (PK) deficiency to be treated in Phase 2 trial

- **Phase 1 MAD clinical trial:** Agios plans to present final data from the MAD clinical trial in healthy volunteers at a medical conference in mid-2015.
- **Phase 2 clinical trial in patients:** Agios expects to initiate a Phase 2 trial for AG-348 in the first half of 2015 in patients with PK deficiency, a rare hemolytic anemia.
- **Natural History study:** Agios expects initial data from the study of the natural history of PK deficiency to be reported at a medical conference in mid-2015. This study is being conducted by Children's Hospital in Boston.

FULL YEAR 2014 FINANCIAL RESULTS

Cash, cash equivalents and marketable securities as of December 31, 2014 were $467.4 million, compared to $193.9 million as of December 31, 2013. The increase was driven by the addition of $332.6 million of net proceeds received from the company's two public offerings of 2,300,000 and 2,284,423 shares of common stock during the second and fourth quarters, respectively. The company also received $40.1 million from Celgene related to reimbursable costs from our AG-221 program and from Celgene's decision to extend the discovery phase under our collaboration agreement through April 2015. These inflows in 2014 have been partially offset by cash used to fund operating activities of approximately $99.5 million.

Collaboration revenue was $65.4 million for the year ended December 31, 2014, compared to $25.5 million for the prior year. The increase was due to the application of new accounting guidance to the company's collaboration agreement with Celgene as a result of the July 2014 amendment of the collaboration agreement with Celgene. As a result, for the period of January 1, 2014 through the amendment date, the company recognized a total of $42.7 million under the previous accounting guidance and upon the modification. The company recognized $22.7 million in revenue subsequent to the modification date.

Research and development (R&D) expenses were $100.4 million, including $6.6 million of stock-based compensation expense, for the year ended December 31, 2014, compared to $54.5 million, including $2.0 million in stock-based compensation expense, for the year ended December 31, 2013. The increase in R&D expenses was primarily due to ongoing development activities for the company's three lead investigational medicines. Celgene is responsible for all development costs for AG-221, and reimburses Agios for development costs it incurs for this investigational medicine.

General and administrative (G&A) expenses were $19.1 million, including $4.8 million of stock-based compensation expense, for the year ended December 31, 2014, compared to $9.9 million, including $1.0 million of stock-based compensation expense, for the year ended December 31, 2013. The increase in G&A expense was largely due to increased headcount and other professional expenses to support growing operations.

Net loss for the year ended December 31, 2014 was $53.5 million, compared to a net loss of $39.4 million for the year ended December 31, 2013.

"We believe Agios entered 2015 in a strong financial position to support several significant upcoming milestones, including multiple early and late stage clinical trials for our three investigational medicines," said Glenn Goddard, senior vice president of finance at Agios. "Our financial guidance for 2015 reflects this broad investment, planned hiring of key employees to support our clinical, regulatory and commercial expansion and continued investment in research to support our goal of becoming a multi-product biopharmaceutical company."

FINANCIAL GUIDANCE FOR THE FULL YEAR 2015

Agios announced today that it expects to end 2015 with more than $320 million of cash, cash equivalents and marketable securities. The anticipated year end 2015 cash position includes a $20 million research extension fee from Celgene, expected to be received in the second quarter of 2015, and does not include any additional program-specific milestone payments. The company expects that its cash, cash equivalents and marketable securities would be sufficient to fund its operating expenses and capital expenditure requirements until late 2017.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:30 a.m. EST to discuss the fourth quarter and full year 2014 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 84528573. 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/Celgene Collaboration

AG-221 and AG-120 are part of Agios' global strategic collaboration with Celgene Corporation, an integrated global biopharmaceutical company. Under the terms of the agreement, Celgene has worldwide development and commercialization rights for AG-221. Agios continues to conduct early clinical development activities within the AG-221 development program and is eligible to receive up to $120 million on achievement of certain milestones and royalties on any net sales. For AG-120, Agios retains U.S. development and commercialization rights. In January 2015, Celgene agreed that it would exercise its option to obtain an exclusive license outside the United States in accordance with the terms of the collaboration agreement, subject to receipt of any required regulatory approvals including any applicable clearance under the Hart-Scott-Rodino Act. Upon Celgene's exercise of its exclusive option, Celgene would lead development and commercialization outside the United States. Celgene would be eligible to receive royalties on any net sales in the U.S. Agios would be eligible to receive royalties on any net sales outside the U.S. and up to $120 million in payments on achievement of certain milestones.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic disorders of metabolism 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 agios.com.

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' expectations and beliefs about: the potential of IDH1/IDH2 and pyruvate kinase-R mutations as therapeutic targets; the potential benefits of Agios' drug candidates targeting IDH1/IDH2 or pyruvate kinase-R mutations, including AG-221, AG-120 and AG-348; its plans and timelines for the clinical development of AG-221, AG-120 and AG-348; 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, 2015; the potential benefit of its strategic plans and focus; and Celgene's exercise of its license option to AG-120, which is subject to receipt of any applicable required regulatory approvals. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could" 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 agreement 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 September 30, 2014 as well as 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.

AGIOS PHARMACEUTICALS, INC.

Consolidated Balance Sheet Data

(in thousands)

Unaudited

	December 31, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	$ 467,447	$ 193,894
Collaboration receivable – related party	6,492	476
Total assets	491,904	201,205
Deferred revenue – related party	38,411	57,639
Stockholders' equity	424,366	131,482

Consolidated Statements of Operations Data

(in thousands, except share and per share data)

Unaudited

	For the Three Months Ended December 31,		For the Years Ended December 31,	
	2014	2013	2014	2013
Collaboration revenue – related party	$ 14,636	$ 6,744	$ 65,358	$ 25,548
Operating expenses:				
Research and development	34,863	15,279	100,371	54,502

General and administrative	6,500	3,707	19,120	9,929
Total operating expenses	41,363	18,986	119,491	64,431
Loss from operations	(26,727)	(12,242)	(54,133)	(38,883)
Interest income	85	29	203	55
Loss before provision for income taxes	(26,642)	(12,213)	(53,930)	(38,828)
Provision (benefit) for income taxes	22	169	(426)	579
Net loss	(26,664)	(12,382)	(53,504)	(39,407)
Cumulative preferred stock dividends	—	—	—	(4,162)
Net loss applicable to common stockholders	$ (26,664)	$ (12,382)	$ (53,504)	$ (43,569)
Net loss per share applicable to common stockholders – basic and diluted	$ (0.76)	$ (0.40)	$ (1.59)	$ (2.83)
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	35,121,705	31,153,340	33,667,024	15,415,373

CONTACT: Agios Pharmaceuticals:
Lora Pike
617-649-8608
Senior Director, Investor Relations and Public Relations
Lora.Pike@agios.com

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