Agios Pharmaceuticals Reports Fourth Quarter and Full Year 2013 Financial Results

March 6, 2014

Pipeline Advancing with Additional Programs Moving into Clinical Development

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 6, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism, today reported business highlights and financial results for the fourth quarter and full year ended December 31, 2013.

"In 2013, we continued to apply our scientific and clinical leadership in the field of cellular metabolism towards our goal of transforming the lives of patients with cancer and inborn errors of metabolism," said David Schenkein, M.D., chief executive officer of Agios. "We successfully filed two investigational new drug applications, or INDs, for novel first-in-class therapeutic candidates and completed IND activities for our third program. In early 2014, we continue to make important progress across our pipeline. Dose escalation in the AG-221 clinical trial continues, and we now plan to present initial clinical data at the 2014 American Association for Cancer Research Annual Meeting in April. We are on track to initiate two clinical trials with AG-120 early this year, and we also plan to initiate clinical trials with AG-348, our first inborn errors of metabolism drug candidate, in mid-2014."

Recent Business Highlights

- **Continued enrollment and dose escalation in the Phase 1 study of AG-221, an IDH2 mutant inhibitor for the treatment of cancer in patients with an IDH2 mutation.** This Phase 1, multi-center study is evaluating the safety, pharmacokinetics, pharmacodynamics and clinical activity of AG-221 in patients with advanced hematologic malignancies that harbor an IDH2 mutation. AG-221 is an orally available, selective, potent inhibitor of the mutated IDH2 protein, making it a highly targeted therapeutic candidate for the treatment of patients with cancers with an IDH2 mutation. Agios expects to present initial clinical data from the dose escalation portion of the ongoing Phase 1 study of AG-221 at the 2014 American Association for Cancer Research Annual Meeting (AACR) in early April.
- **Completed investigational new drug (IND) filing for AG-120, an IDH1 mutant inhibitor for the treatment of cancer in patients with an IDH1 mutation.** The Food and Drug Administration (FDA) recently accepted Agios' IND for AG-120, and the company remains on track to initiate two Phase 1 clinical trials in early 2014. AG-120 is an orally available, selective, potent inhibitor of the mutated IDH1 protein, and has been demonstrated to be a highly targeted therapeutic candidate for the treatment of patients with cancers that harbor an IDH1 mutation.
- **Exercised the option to U.S. rights to AG-120**. Agios elected to exercise the option to U.S. development and commercial rights for AG-120, in accordance with the terms of its agreement with Celgene Corporation, with Celgene retaining its option to ex-U.S. rights.
- **Advanced AG-348, an activator of pyruvate kinase R (PKR) for the treatment of patients with pyruvate kinase deficiency (PK deficiency), toward clinical development.** Agios has completed IND-enabling studies of AG-348 and expects to initiate Phase 1 clinical trials for AG-348 in mid-2014. AG-348 is an orally available, potent, selective small molecule activator of the PKR enzyme, which, when mutated, leads to PK deficiency, a form of hereditary hemolytic anemia.
- **Extended cancer metabolism collaboration with Celgene.** In December, Agios announced an extension of one additional year to the period of exclusivity for its strategic cancer metabolism collaboration with Celgene. As a result, the exclusive research collaboration between Agios and Celgene has been extended through April 2015, and Agios will receive a $20 million extension payment to be paid mid-2014.

Upcoming Milestones

- Agios expects to present initial clinical data from the dose escalation portion of the ongoing Phase 1 study of AG-221 at the 2014 American Association for Cancer Research Annual Meeting (AACR) in early April. Agios expects to continue enrollment in its Phase 1 study of AG-221 and initiate expansion cohorts in late 2014.
- Agios plans to initiate two Phase 1 clinical trials for AG-120 in early 2014 (one in advanced solid tumors and one in hematologic malignancies) in patients whose cancers carry an IDH1 mutation. These studies will leverage the clinical trial sites of Agios' Phase 1 study of AG-221.
- Agios anticipates initiating single and multiple ascending dose-escalation studies for AG-348 in healthy volunteers in mid-2014.

Fourth Quarter 2013 Financial Results & Financial Guidance

- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2013 were $193.9 million, compared to $128.0 million as of December 31, 2012. The increase was primarily driven by net proceeds of $111.0 million from Agios' IPO and $12.8 million from the concurrent private placement with Celgene, offset by cash used to fund operations.
- **Revenues:** Collaboration revenue was $6.7 million for the fourth quarter of 2013 and $25.5 million for the year ended December 31, 2013,compared to $6.3 million and $25.1 million in the comparable periods in 2012. Collaboration revenue is primarily comprised of amortization of deferred revenue from payments received in previous periods from Agios' collaboration agreement with Celgene.
- **R&D Expenses:** Research and development expenses were $15.3 million in the fourth quarter of 2013 and $54.5 million for the year ended December 31, 2013, compared to $11.2 million and $41.0 million in the comparable periods in 2012. The increase in R&D expense was largely due to increased spending on clinical activities as AG-221 entered Phase 1 development in September 2013, and as the company prepares to begin clinical development of AG-120 in early 2014, as well as IND-enabling activities for the company's AG-348 program.
- **G&A Expenses:** General and administrative expenses were $3.7 million in the fourth quarter of 2013 and $9.9 million in the year ended December 31, 2013, compared to $1.6 million and $7.1 million in the comparable periods in 2012. The increase in G&A expenses was largely due to incremental expenses to support public company operations.
- **Net Loss:** Net loss was $12.4 million for the fourth quarter of 2013 and $39.4 million for the year ended December 31, 2013, compared to net loss of $5.3 million and $20.1 million for the comparable periods in 2012.
- **Financial Guidance:** Agios expects that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements until late 2016.

"Agios continues to maintain a strong balance sheet, ending 2013 with $193.9 million in cash," said Glenn Goddard, senior vice president of finance at Agios. "We anticipate ending 2014 with more than $130 million in cash. We expect our financial position to provide us with ample funding to execute on our strategic business plan and drive our lead programs to meaningful clinical milestones."

Conference Call Information

Agios will host a conference call and live audio webcast today at 8:30 a.m. EST to discuss the fourth quarter and full year 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 4988636. The live webcast can be accessed under "Events & Presentations" in the Investors and 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 Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and inborn errors of metabolism, or IEMs, which are rare genetic metabolic 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 lead product candidates in cancer metabolism and IEMs 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 our website at www.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' product 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 period in which cash will be available to fund its operating expenses and capital expenditure requirements; and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would" 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, 2013, and other filings that Agios may make with the Securities and Exchange Commission (SEC) 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 (UNAUDITED)
(Amounts in thousands)

	December 31, 2013	December 31, 2012
Cash, cash equivalents, and marketable securities	$193,894	$127,976
Total assets	201,205	137,008
Deferred revenue	57,639	82,711
Preferred stock	-	115,922
Stockholders' equity (deficit)	131,482	(72,024)

AGIOS PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)
(Amounts in thousands except per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2013	2012	2013	2012
Total revenue	$6,744	$6,282	$25,548	$25,106
Operating expenses:				
Research and development	15,279	11,226	54,502	41,037
General and administrative	3,707	1,554	9,929	7,064
Total operating expenses	18,986	12,780	64,431	48,101
Loss from operations	(12,242)	(6,498)	(38,883)	(22,995)
Interest income	29	9	55	69
(Provision) benefit for income taxes	(169)	1,176	(579)	2,824
Net loss	($12,382)	($5,313)	($39,407)	($20,102)
Cumulative preferred stock dividends	-	(1,796)	(4,162)	(7,190)
Net loss applicable to common stockholders	($12,382)	($7,109)	($43,569)	($27,292)
Net loss per share applicable to common stockholders:				
Basic and diluted	($0.40)	($2.09)	($2.83)	($8.02)
Weighted average shares outstanding:				
Basic and diluted	31,153	3,402	15,415	3,402

Source: Agios Pharmaceuticals, Inc.

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