Agios Pharmaceuticals Reports Third Quarter 2013 Financial Results

November 7, 2013

Successfully Completed Initial Public Offering and Advanced IDH2 Cancer Metabolism Program into Clinical Development

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 7, 2013-- 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 third quarter ended September 30, 2013.

"Agios has made important progress this year toward realizing our long-term vision of developing transformational medicines and building a world-class biopharmaceutical company," said David Schenkein, M.D., chief executive officer at Agios. "We successfully completed our initial public offering, ending the period with $208.4 million in cash, cash equivalents and marketable securities. We initiated a Phase 1 clinical trial of our lead product candidate, AG-221, in a genetically defined population of patients carrying an IDH2 mutation. In addition to AG-221, we are continuing to make progress on our goal of advancing a broad portfolio of first-in-class cancer metabolism and inborn errors of metabolism drug candidates toward the clinic, including AG-120, an IDH1 inhibitor for the treatment of cancer in patients with an IDH1 mutation, and AG-348, an activator of pyruvate kinase for the treatment of patients with pyruvate kinase deficiency, and we remain on track with our plans to begin clinical testing of both of these drug candidates in 2014."

Recent Business Highlights

- **Completed initial public offering.** Agios announced on July 29, 2013 that it had completed an initial public offering (IPO) of common stock, raising net proceeds of $111.0 million. In addition, Celgene purchased $12.8 million of Agios common stock in a separate private placement concurrent with the completion of the IPO.
- **Initiated Phase 1 study of AG-221, an IDH2 mutant inhibitor for the treatment of cancer in patients with an IDH2 mutation.** In September, Agios announced that the first patient was dosed in a Phase 1 study of AG-221 in advanced hematologic malignancies 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. The first stage of the study is a dose-escalation phase in which cohorts of patients will receive ascending oral doses of AG-221 to determine the maximum tolerated dose and/or the recommended Phase 2 dose. At completion of the dose escalation phase, several expansion cohorts of patients will receive AG-221 to further evaluate the safety, tolerability and clinical activity of the maximum tolerated dose. Currently, four clinical trial sites are open and recruiting patients. 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.
- **Advanced AG-120, an IDH1 mutant inhibitor for the treatment of cancer in patients with an IDH1 mutation, toward Investigational New Drug (IND) filing.** Agios has substantially completed IND-enabling studies of AG-120 and remains on track for initiating clinical trials in early 2014. AG-120 is an orally available, selective, potent inhibitor of the mutated IDH1 protein, and a highly targeted therapeutic candidate for the treatment of patients with cancers that harbor an IDH1 mutation. Phase 1 trials are being planned for patients with advanced solid and hematological malignancies that carry an IDH1 mutation.
- **Advanced AG-348, an activator of pyruvate kinase R (PKR) for the treatment of patients with pyruvate kinase deficiency (PK deficiency), into IND-enabling studies.** Agios has initiated IND-enabling preclinical studies of AG-348, an orally available, potent small molecule activator of the PKR enzyme, which, when mutated, leads to PK deficiency, a form of hereditary hemolytic anemia. Pre-clinical *in vitro* data demonstrate that these activators can significantly enhance the activity of most of the common PKR mutations, making it a potential treatment for patients with PK deficiency.
- **Strengthened board of directors.** In September, Agios announced the addition of Paul J. Clancy, executive vice president and chief financial officer at Biogen Idec, to its board of directors.

Upcoming Milestones

- Agios expects to present preclinical data for each of its lead programs at the annual American Society for Hematology (ASH) meeting in December 2013.
- In early 2014, Agios anticipates submitting an IND and initiating Phase 1 clinical trials for AG-120 in patients with advanced solid and hematological malignancies that carry an IDH1 mutation.
- Agios anticipates submitting an IND and initiating Phase 1 clinical trials for AG-348 in 2014, including normal healthy volunteers and patients with PK deficiency.

Third Quarter 2013 Financial Results & Financial Guidance

- **Cash Position:** Cash, cash equivalents and marketable securities as of September 30, 2013 were $208.4 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 $6.3 million in each of the third quarters of 2013 and 2012 and $18.8 million in each of the nine months ended September 30, 2013 and 2012. Collaboration revenue is primarily comprised of deferred revenue from payments received in previous periods from Agios' collaboration agreement with Celgene.
- **R&D Expenses:** Research and development expenses were $14.8 million in the third quarter of 2013 and $39.2 million in the nine months ended September 30, 2013, compared to $9.8 million and $29.8 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, in addition to IND-enabling activities for the company's AG-120 and AG-348 programs.
- **G&A Expenses:** General and administrative expenses were $2.5 million in the third quarter of 2013 and $6.2 million in the nine months ended September 30, 2013, compared to $1.6 million and $5.5 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 $11.2 million for the third quarter of 2013 and $27.0 million for the nine months ended September 30, 2013, compared to net loss of $4.6 million and $14.8 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 at least the fourth quarter of 2016.

Conference Call Information

Agios will host a conference call and live audio webcast today at 8:30 a.m. EST to discuss the quarter 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 92532816. 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; 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 June 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)

	September 30, 2013	December 31, 2012
Cash, cash equivalents, and marketable securities	$ 208,368	$ 127,976
Total assets	219,069	137,008
Deferred revenue	63,907	82,711
Preferred stock	-	115,922
Stockholders' equity (deficit)	142,595	(72,024)

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Total revenue	$ 6,268	$ 6,268	$ 18,804	$ 18,824
Operating expenses:				
Research and development	14,803	9,798	39,223	29,812
General and administrative	2,534	1,580	6,222	5,510
Total operating expenses	17,337	11,378	45,445	35,322
Loss from operations	(11,069)	(5,110)	(26,641)	(16,498)
Interest income	13	13	26	61
Provision (benefit) for income taxes	121	(452)	410	(1,648)
Net loss	($11,177)	($4,645)	($27,025)	($14,789)
Cumulative preferred stock dividends	567	1,798	4,162	5,393
Net loss applicable to common stockholders	($10,610)	($2,847)	($22,863)	($9,396)
Net loss per share applicable to common stockholders:				
Basic and diluted	($0.47)	($0.83)	($2.26)	($2.81)
Weighted average shares outstanding:				
Basic and diluted	22,744	3,439	10,112	3,348

Source: Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals, Inc.
Glenn Goddard
SVP Finance
investors@agios.com
or
Media Contact:
Dan Budwick, 973-271-6085
dan@purecommunicationsinc.com