



AgiOS Pharmaceuticals Reports First Quarter 2014 Financial Results

May 8, 2014

AG-221 Showed Promising Clinical Activity in AML; Additional Data to be Presented at EHA

Three First-in-Class Compounds now in Clinical Development

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism (IEMs), today reported business highlights and financial results for the first quarter ended March 31, 2014.

"In the first quarter, we made significant progress in the development and advancement of all our programs," said David Schenkein, M.D., chief executive officer of Agios. "We presented preliminary data for our lead product AG-221 at AACR last month that demonstrated the potential for a paradigm shift in the current treatment of acute myelogenous leukemia. These preliminary data showed that AG-221 had promising clinical activity with a favorable safety profile, establishing the first clinical proof of concept for an IDH2 mutant inhibitor. We also recently initiated two clinical trials of the IDH1 mutant inhibitor AG-120, and the first clinical study for our third program, AG-348, targeting pyruvate kinase deficiency."

Recent Business Highlights

Cancer Metabolism: IDH Mutant Inhibitors in Collaboration with Celgene

- In April, during the clinical trial symposium at the American Association of Cancer Research (AACR) Annual Meeting 2014, investigators presented preliminary data from the first two cohorts in the ongoing Phase 1 study of AG-221 in 10 patients with advanced IDH2 mutant positive hematologic malignancies. Initial data showed that AG-221 was well tolerated when dosed at 30 mg and 50 mg twice daily in 28 day continuous cycles. Promising efficacy was observed with six of seven evaluable patients having objective responses, including three complete remissions and two complete remissions with incomplete platelet recovery. Additional information on the preliminary data is available in [a press release](#) issued by Agios on April 6, 2014.
- In March, Agios announced initiation of two Phase 1 studies of AG-120, one in advanced hematologic malignancies and one in advanced solid tumors, including glioma. These multi-center, open-label, dose-escalation studies are evaluating the safety, pharmacokinetics, pharmacodynamics and clinical activity of AG-120 in patients whose advanced cancer harbor an IDH1 mutation.

IEMs: Wholly Owned Asset Targeting PK Deficiency

- In April, Agios announced dose administration of AG-348, a first-in-class pyruvate kinase (PK) R activator, in a Phase 1 healthy volunteer study. The single-center, randomized, double-blind, placebo-controlled clinical trial is assessing the safety and tolerability, as well as pharmacokinetics and pharmacodynamics, of AG-348 through a single ascending dose escalation in healthy adults. This will be the first of two healthy volunteer studies designed to enable a proof of concept study in patients with pyruvate kinase deficiency.
- A natural history study of PK deficiency is also ongoing. Natural history studies represent an important tool for understanding clinical characteristics and disease progression and help support the design of future clinical trials.

Upcoming Milestones

- Agios continues to dose escalate its Phase 1 study of AG-221, and expects to initiate expansion cohorts by the end of 2014.
- A late-breaker abstract on an update from the dose-escalation portion of the ongoing Phase 1 study of AG-221 has been accepted for oral presentation at the 19th Congress of the European Hematology Association (EHA) in Milan, Italy, June 12-15, 2014.
- Enrollment remains on track for the AG-120 clinical trials, and the company anticipates providing updates on both trials at medical conferences in 2015.
- Agios expects to start a multiple-ascending dose escalation Phase 1 clinical trial of AG-348 in healthy volunteers in mid-2014, and to report data for the healthy volunteer trials of AG-348 and interim data from the natural history study at a medical conference in 2015.

First Quarter 2014 Financial Results

Cash, cash equivalents and marketable securities as of March 31, 2014 were \$167.3 million, compared to \$193.9 million as of December 31, 2013. Subsequent to March 31, 2014, the company completed a public offering, with the underwriters' option to purchase additional shares expected to close today, that will add approximately \$95 million in net proceeds, after deducting underwriting discounts and estimated offering expenses, to this cash position.

Total revenue was \$8.4 million for the first quarter of 2014, compared to \$6.3 million for the comparable period in 2013. Total revenue is primarily comprised of amortization of deferred revenue from payments received in previous periods from Agios' collaboration agreement with Celgene. The increase in total revenue in the first quarter of 2014 was related to Celgene's December 2013 election to extend the discovery phase of the collaboration agreement through April 2015. The related \$20 million extension payment is expected to be received in May 2014.

Research and development (R&D) expenses were \$17.4 million, including \$1.1 million of stock-based compensation expense, in the first quarter of 2014, compared to \$11.5 million, including \$0.3 million in stock-based compensation expense, for the comparable period in 2013. The increase in R&D expense was due to increased spending on the on-going clinical activities for AG-221, and increased spending as the company prepared to initiate clinical development of AG-120 and AG-348 programs.

General and administrative (G&A) expenses were \$3.3 million, including \$0.4 million of stock-based compensation expense, in the first quarter of 2014, compared to \$1.9 million, including \$0.1 million of stock-based compensation expense, for the comparable period in 2013. The increase in G&A expense was largely due to increased headcount and other professional expenses to support public company operations.

Net loss for the first quarter of 2014 was \$12.2 million, compared to net loss of \$7.2 million for the comparable period in 2013.

"Following the close of our recent equity offering, we believe we're well positioned financially to fund our current development and research programs," said Glenn Goddard, senior vice president of finance at Agios. "Our company is excited about the future and developing medicines that could have a major impact on the treatment of serious diseases."

Financial Guidance for the Full Year 2014

Agios expects to end 2014 with more than \$200 million of cash, cash equivalents and marketable securities. The company believes this cash position will be sufficient to fund its operating expenses and capital expenditure requirements through mid-2017.

Conference Call Information

Agios will host a conference call and live webcast with slides today at 8:30 a.m. EDT to discuss the first quarter 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 32998186. 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 Acute Myelogenous Leukemia (AML)

AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults. Undifferentiated blast cells proliferate in the bone marrow rather than mature into normal blood cells. AML incidence significantly increases with age. Less than 10 percent of U.S. patients are eligible for bone marrow transplant, and the vast majority of patients do not respond to chemotherapy and progress to relapsed / refractory AML. AML prevalence is estimated to be approximately 115,000 to 160,000 patients worldwide, with approximately 20 percent of patients carrying an IDH mutation. The five-year survival rate for AML is approximately 20 to 25 percent.

About IDH Mutations and Cancer

IDH1 and IDH2 are two metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies. The prevalence of IDH is expected to evolve as genomic analysis of tumors increase. Agios' research revealed the potential of IDH1 and IDH2 mutations as novel therapeutic targets in cancer, which may lead to clinical benefit for the subset of cancer patients whose tumors carry them. Patients carry either an IDH1 or IDH2 mutation, but not both.

Agios is developing two oral, first-in-class IDH mutant inhibitors: AG-221 is an IDH2 mutant inhibitor and AG-120 is an IDH1 mutant inhibitor. AG-221 is currently being evaluated in a Phase 1 dose-escalation study in patients with advanced hematologic malignancies. AG-120 is currently being evaluated in two Phase 1 trials, one in hematologic malignancies and another in solid tumors. Both compounds were discovered and developed in the laboratory of Agios.

About PK Deficiency

Pyruvate kinase (PK) deficiency, a rare, inherited hemolytic anemia, is caused by mutations that affect the activity of the metabolic enzyme pyruvate kinase-R (PK-R), the form of pyruvate kinase that is present in red blood cell counts. The current standard of care for PK deficiency is supportive, including blood transfusions, splenectomy, chelation therapy to address iron overload and/or interventions for other treatment- and disease-related morbidities. Currently, there is no approved therapy to treat the underlying cause of PK deficiency. AG-348 is a first-in-class orally available, potent, selective small molecule activator of PK-R, which, when mutated, leads to PK deficiency. AG-348 was discovered in the laboratory of Agios, and the company retains worldwide development and commercialization rights.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and inborn errors of metabolism (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, including 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; its expectations regarding the closing of the sale of additional shares in connection with its recent equity financing, and the benefit of its strategic plans and focus. 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; the satisfaction of customary closing conditions in connection with the closing of the sale of additional shares in connection with its recent equity offering, 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, 2013 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 (Unaudited)
(in thousands)

	March 31, 2014	December 31, 2013
Cash and cash equivalents, and marketable securities	\$ 167,274	\$ 193,894
Total assets	181,912	201,205
Deferred revenue	51,371	57,639
Stockholders' equity	121,343	131,482

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

	Three Months Ended March 31, 2014	2013
Total revenue	\$ 8,411	\$ 6,268
Operating expenses:		
Research and development	17,407	11,462
General and administrative	3,288	1,852
Total operating expenses	20,695	13,314
Loss from operations	(12,284)	(7,046)
Interest income	36	8
Loss before provision for income taxes	(12,248)	(7,038)
Provision for income taxes	—	190
Net loss	<u>\$ (12,248)</u>	<u>\$ (7,228)</u>
Cumulative preferred stock dividends	—	(1,797)
Net loss applicable to common stockholders	<u>\$ (12,248)</u>	<u>\$ (9,025)</u>

Net loss per share applicable to common stockholders – basic and diluted	<u>\$ (0.39)</u>	<u>\$ (2.47)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders – basic and diluted	<u>31,395</u>	<u>3,658</u>

Source: Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals, Inc.

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