Agios Reports Business Highlights and First Quarter 2019 Financial Results

May 2, 2019

– Commercial Organization Launch Ready Ahead of June 21, 2019 PDUFA Action Date for Single Agent TIBSOVO® for IDH1m Newly Diagnosed AML –

– Updated Data from Phase 1 Studies of TIBSOVO® in Newly Diagnosed AML and First Data from Perioperative Study of TIBSOVO® and Vorasidenib Accepted for Presentation at ASCO –

– Broad Clinical Development Plan for Mitapivat Progressing with Pyruvate Kinase Deficiency Pivotal Studies Enrolling, First Patient Dosed in Phase 2 Thalassemia Study and NIH-Sponsored Sickle Cell Study to Initiate in 2019 –

– $9.1M First Quarter Net Revenue for TIBSOVO®; March 31, 2019 Cash, Cash Equivalents and Marketable Securities was $707.8M; Provides Runway Through at Least the End of 2020 –

CAMBRIDGE, Mass., May 02, 2019 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today reported business highlights and financial results for the first quarter ended March 31, 2019.

"We continued to deliver significant progress across our oncology and rare genetic disease programs during the first quarter. Importantly, our U.S. commercial team is driving TIBSOVO® toward solid performance for its first full year on the market, and we are on track to achieve our internal forecast," said Jackie Fouse, Ph.D., chief executive officer at Agios. "We made progress on expansion opportunities for TIBSOVO ® across the frontline AML setting. FDA accepted our sNDA in newly diagnosed AML, the HOVON/AMLSG Phase 3 intensive chemotherapy combination study initiated, and we received Breakthrough Therapy Designation for the combination of TIBSOVO® and azacitadine. We also advanced our mitapivat program. Our two pivotal trials in pyruvate kinase deficiency remain on track to complete enrollment this year, we have dosed the first patient in our Phase 2 thalassemia study, and we now expect a NIH-sponsored study in sickle cell disease to initiate this year."

FIRST QUARTER 2019 HIGHLIGHTS & RECENT PROGRESS

- Received FDA acceptance and Priority Review for the supplemental new drug application (sNDA) for single agent TIBSOVO® for the treatment of patients with newly diagnosed acute myeloid leukemia (AML) with an isocitrate dehydrogenase-1 (IDH1) mutation who are not eligible for standard therapy. The Prescription Drug User Fee Act (PDUFA) action date was set for June 21, 2019.
- Presented updated data from the ongoing Phase 1 combination trial of TIBSOVO® with azacitidine in patients with newly diagnosed AML with an IDH1 mutation at the 17th International Symposium on Acute Leukemias.
- Received Breakthrough Therapy designation from FDA for TIBSOVO® in combination with azacitidine for the treatment of newly diagnosed AML patients with an IDH1 mutation who are ≥75 years old or who have comorbidities that preclude use of intensive induction chemotherapy.
- Supported the cooperative groups HOVON and AMLSG on the initiation of the Phase 3 randomized, placebo-controlled study of TIBSOVO® or IDHIFA® in combination with induction therapy and consolidation therapy followed by maintenance therapy in patients with newly diagnosed AML with an IDH1 mutation.
- Presented preclinical data for AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor, at the American Association for Cancer Research meeting.
- Dosed the first patient in a Phase 2 proof-of-concept study for mitapivat in thalassemia.
- Announced two newly created commercial leadership roles to support the commercialization of the company's medicines in the U.S. and Europe. Darrin Miles, who has been with the company since 2015, most recently as vice president, oncology program leadership, was promoted to senior vice president, U.S. commercial and global marketing. In addition, a search is underway for the role of senior vice president, international.

KEY UPCOMING MILESTONES

The company plans to achieve the following key milestones in the remainder of 2019:

Cancer:

- Potential U.S. approval and launch of single agent TIBSOVO® for newly diagnosed AML with an IDH1 mutation not eligible for standard therapy by June 21, 2019.
- Submit an sNDA to the FDA for TIBSOVO® for second line or later IDH1 mutant cholangiocarcinoma by year-end.
- Initiate a registration-enabling Phase 3 study of vorasidenib in low-grade glioma with an IDH1 mutation by year-end.
- Determine recommended dose of AG-270 in methylthioadenosine phosphorylase (MTAP)-deleted tumors; initiate expansion

arms, including a single-agent arm in a variety of MTAP-deleted tumors and two combination arms combining AG-270 and standard-of-care in non-small cell lung cancer and pancreatic ductal adenocarcinoma in the third quarter.
- Begin dosing patients in the Phase 1 dose-escalation trial of AG-636, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH), in lymphoma in the first half of 2019.

Rare Genetic Diseases:

- Complete enrollment in two global pivotal trials for mitapivat in adults with pyruvate kinase (PK) deficiency by year-end 2019:
  - ACTIVATE-T: A single-arm trial of up to 40 regularly transfused patients; enrollment was increased from 20 to 40 based on demand
  - ACTIVATE: A 1:1 randomized, placebo-controlled trial of 80 patients who do not receive regular transfusions
- Achieve proof-of-concept for mitapivat in thalassemia in the second half of 2019.

ANTICIPATED 2019 DATA PRESENTATIONS

- The following abstracts have been accepted for presentation at 2019 American Society of Clinical Oncology (ASCO) Annual Meeting:
  - Updated data from a Phase 1 study of single agent TIBSOVO® in IDH1 mutant newly diagnosed AML ineligible for standard therapies.
  - Updated data from the Phase 1 combination study of TIBSOVO® and azacitidine in newly diagnosed AML with an IDH1 mutation.
  - Results from the first cohort in a Phase 1 perioperative study of TIBSOVO® and vorasidenib in recurrent IDH1 mutant low-grade glioma.
- Topline data from the Phase 3 ClarIDHy study of TIBSOVO® in IDH1 mutant second line or later cholangiocarcinoma to be reported in the first half and full data to be presented in the second half of 2019.
- Data from the dose-escalation portion of the ongoing Phase 1 study of AG-270 in patients with MTAP-deleted tumors expected in the second half of 2019.

FIRST QUARTER 2019 FINANCIAL RESULTS

Revenue: Total revenue for the first quarter of 2019 was $30.2 million, which includes $17.9 million in collaboration revenue and $2.2 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene, and $9.1 million of net product revenue from U.S. sales of TIBSOVO®. This compares to revenue of $8.8 million for the first quarter of 2018, which included $7.3 million in collaboration revenue and $1.4 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene.

Cost of Sales: We began U.S. sales of TIBSOVO® in the third quarter of 2018. Cost of sales were $0.3 million for the first quarter of 2019.

Research and Development (R&D) Expenses: R&D expenses were $95.6 million for the first quarter of 2019 compared to $78.2 million for the first quarter of 2018. The increase in R&D expense was primarily attributable to clinical trial activity related to TIBSOVO® frontline trials, the mitapivat pivotal program in PK deficiency and Phase 2 study in thalassemia, and start-up activities for AG-636.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $31.8 million for the first quarter of 2019 compared to $24.6 million for the first quarter of 2018. The increase in SG&A expense was primarily attributable to costs to support commercialization of TIBSOVO® and personnel costs related to increased headcount.

Net Loss: Net loss was $93.1 million for the first quarter of 2019 compared to $90.8 million for the first quarter of 2018.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of March 31, 2019 were $707.8 million compared to $805.4 million as of December 31, 2018. The net decrease of $97.6 million in cash position was primarily driven by net expenditures to fund operations, including a onetime cash expense of $19.2 million for bonus payouts during the first quarter. The company expects that its cash, cash equivalents and marketable securities as of March 31, 2019, together with anticipated product and royalty revenue, anticipated interest income, and anticipated expense reimbursements under our collaboration and license agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through at least the end of 2020.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss first quarter 2019 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 referring to conference ID 9573049. The live webcast can be accessed under “Events & Presentations” in the Investors 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 diseases through scientific leadership in the field of cellular metabolism and adjacent areas of biology. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies 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

IDHIFA® (enasidenib) and AG-270 are part of our collaboration with Celgene Corporation. Under the terms of our 2010 collaboration agreement focused on cancer metabolism, Celgene has worldwide development and commercialization rights for IDHIFA®. Agios continues to conduct certain clinical development activities within the IDHIFA® development program and is eligible to receive reimbursement for those development activities and up to $80 million in remaining milestone payments, and royalties on any net sales. Celgene and Agios are currently co-commercializing IDHIFA® in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts. AG-270 is part of a 2016 global research collaboration agreement with Celgene focused on metabolic immuno-oncology. Celgene has the option to participate in a worldwide 50/50 cost and profit share with Agios, under which Agios is eligible for up to $169 million in clinical and regulatory milestone payments for the program.

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' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including TIBSOVO® (ivosidenib), IDHIFA® (enasidenib), vorasidenib, mitapivat, AG-270 and AG-636; the potential benefits of Agios' product candidates; its key milestones for 2019; its plans regarding future data presentations; its financial guidance regarding the period in which it will have capital available to fund its operations; and the potential benefit of its strategic plans and focus. The words "anticipate," "expect," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," 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 or its collaborators 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 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 CStone Pharmaceuticals; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. 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.

Condensed Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	March 31, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 707,791	$ 805,421
Accounts receivable, net	4,355	5,076
Collaboration receivable – related party	2,319	2,462
Royalty receivable – related party	2,200	2,234
Inventory	2,328	869
Total assets	822,131	858,457
Deferred revenue – related party	77,128	92,519
Stockholders' equity	620,257	687,537

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

	Three Months Ended March 31,	
	2019	2018
Revenues:		
Product revenue, net	$ 9,138	$ -
Collaboration revenue – related party	17,919	7,345
Collaboration revenue – other	970	-

Royalty revenue – related party	2,200	1,417
Total Revenue	30,227	8,762
Cost and expenses:		
Cost of sales	334	-
Research and development, net	95,585	78,224
Selling, general and administrative	31,791	24,550
Total cost and expenses	127,710	102,774
Loss from operations	(97,483)	(94,012)
Interest income	4,405	3,187
Net loss	$ (93,078)	$ (90,825)
Net loss per share – basic and diluted	$ (1.59)	$ (1.63)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	58,453,918	55,694,603

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