Agios Reports Fourth Quarter and Full Year 2018 Financial Results

February 14, 2019

– Strong Launch of TIBSOVO® Continues with Net Revenue of $9.4M for the Fourth Quarter and $13.8M for Full Year 2018 –

– Phase 3 ClarIDHy Trial of TIBSOVO® in Second Line or Later Cholangiocarcinoma Fully Enrolled –

– AG-270 Preclinical Data Accepted for Presentation at AACR; Data from Perioperative Study of TIBSOVO® and Vorasidenib in Low Grade Glioma Submitted for Presentation at ASCO –

– Dr. Jackie Fouse Assumed Role as Chief Executive Officer on Feb. 1, 2019 –

CAMBRIDGE, Mass., Feb. 14, 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 fourth quarter and year ended December 31, 2018. In addition, Agios highlighted select 2019 corporate milestones and data presentations for its clinical development programs.

"I'm excited to join the Agios team on the heels of a transformational year for the company. During 2018, we launched our first wholly owned oncology medicine, expanded our clinical programs across both oncology and rare genetic diseases, and continued to advance our robust research pipeline," said Jackie Fouse, Ph.D. "We start 2019 with a strong foundation on which to build and with the opportunity to make a meaningful impact on patients' lives and our business. Our objectives for this year focus on broadening the potential of our IDH inhibitors in AML and solid tumors, advancing mitapivat and AG-270 through clinical development, and remaining steadfast in our pursuit of great science."

KEY UPCOMING MILESTONES

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

Cancer:

- Potential FDA approval of the supplemental new drug application (sNDA) for single agent TIBSOVO® (ivosidenib) for the treatment of patients with newly diagnosed AML with an IDH1 mutation who are not eligible for standard therapy and subsequent launch in this indication in the U.S.
- 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 (AG-881) in low-grade glioma with an IDH1 mutation by year-end.
- Determine recommended dose of AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor, in methylthioadenosine phosphorylase (MTAP)-deleted tumors; initiate expansion arms, including a single-agent arm in a variety of MTAP-deleted cancers and a combination arm in a solid tumor in the first half of 2019.
- Initiate a 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 approximately 20 regularly transfused patients
  - 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 KEY 2019 DATA PRESENTATIONS

- Updated data from the ongoing Phase 1 combination trial of TIBSOVO® with azacitidine in patients with newly diagnosed AML with an IDH1 mutation to be presented at the 17th International Symposium on Acute Leukemias taking place February 24-27, 2019 in Munich.
- Preclinical data for AG-270 accepted for presentation at the American Association for Cancer Research (AACR) meeting taking place March 29-April 3, 2019 in Atlanta.
- Data from the perioperative 'window' trial with TIBSOVO® and vorasidenib in IDHm low-grade glioma submitted for presentation at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting taking place May 31-June 4, 2019 in Chicago.
- 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.

FOURTH QUARTER 2018 HIGHLIGHTS & RECENT PROGRESS

- Submitted an sNDA to the FDA for TIBSOVO® for the treatment of patients with newly diagnosed AML with an IDH1 mutation who are not eligible for standard therapy.
- Submitted and received validation for a Marketing Authorization Application to the European Medicines Agency for TIBSOVO® for the treatment of adult patients with R/R AML with an IDH1 mutation.
- Completed enrollment in the Phase 3 ClarIDHy study of TIBSOVO® in IDH1 mutant second line or later cholangiocarcinoma.
- Initiated a Phase 2 proof-of-concept trial of mitapivat in thalassemia.
- Received FDA clearance of an IND application for AG-636, a DHODH inhibitor.

FOURTH QUARTER AND FULL YEAR 2018 FINANCIAL RESULTS

Revenue: Total revenue for the fourth quarter of 2018 was $30.0 million, which includes $18.4 million in collaboration revenue, $9.4 million of net product revenue from U.S. sales of TIBSOVO® and $2.2 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene. This compares to $9.8 million for the fourth quarter of 2017, which included $8.6 million in collaboration revenue and $1.2 million in royalty revenue from net global sales of IDHIFA®. Total revenue was $94.4 million for the year ended December 31, 2018 compared to $43.0 million for the year ended December 31, 2017. The increases in revenue are primarily driven by net U.S. sales of TIBSOVO®, additional collaboration revenue and royalty revenue from net U.S. sales of IDHIFA®.

Cost of Sales: We began U.S. sales of TIBSOVO® in the third quarter of 2018. Cost of sales were $0.7 million for the fourth quarter of 2018, and $1.4 million for the year ended December 31, 2018.

Research and Development (R&D) Expenses: R&D expenses were $93.8 million for the fourth quarter of 2018 compared to $77.2 million for the fourth quarter of 2017, and $341.3 million for the year ended December 31, 2018 compared to $292.7 million for the comparable period in 2017. The increase in R&D expense was primarily attributable to start-up costs for the mitapivat pivotal program in PK deficiency and Phase 2 study in thalassemia and IND enabling activities for AG-636, our DHODH inhibitor. R&D expense also increased as a result of ongoing research efforts across our discovery platform programs.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $31.9 million for the fourth quarter of 2018 compared to $22.7 million for the fourth quarter of 2017, and $114.1 million for the year ended December 31, 2018 compared to $71.1 million for the year ended December 31, 2017. 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 $91.8 million for the fourth quarter of 2018 compared to $88.3 million for the fourth quarter of 2017, and $346.0 million for the year ended December 31, 2018 was compared to a net loss of $314.7 million for the year ended December 31, 2017.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of December 31, 2018 were $805.4 million compared to $567.8 million as of December 31, 2017. The change in cash was primarily driven by the net proceeds of $516.2 million from the January follow-on offering. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2018, 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 fourth quarter and full year 2018 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 9886713. 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. 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 (AG-881), 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 the U.S. FDA, the EMA or 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 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.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	December 31, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 805,421	$ 567,750
Accounts receivable, net	5,076	-
Collaboration receivable – related party	2,462	2,448
Royalty receivable – related party	2,234	1,222
Inventory	869	-
Total assets	858,457	614,397
Deferred revenue – related party	92,519	163,640
Stockholders' equity	687,537	375,503

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

	Three Months Ended December 31,		Years Ended December 31,	
	2018	2017	2018	2017
Revenues:				
Product revenue, net	$ 9,376	$ -	$ 13,841	$ -
Collaboration revenue – related party	18,183	8,577	60,661	41,074
Collaboration revenue – other	230	-	12,670	-
Royalty revenue – related party	2,224	1,222	7,215	1,937
Total Revenue	30,013	9,799	94,387	43,011
Cost and expenses:				
Cost of sales	702	-	1,397	-
Research and development, net	93,809	77,216	341,324	292,681
Selling, general and administrative	31,858	22,713	114,145	71,124

Total cost and expenses	126,369	99,929	456,866	363,805
Loss from operations	(96,356)	(90,130)	(362,479)	(320,794)
Interest income	4,562	1,845	16,451	6,124
Net loss	$ (91,794)	$ (88,285)	$ (346,028)	$ (314,670)
Net loss per share – basic and diluted	$ (1.58)	$ (1.81)	$ (6.03)	$ (6.75)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	58,189,254	48,772,901	57,418,300	46,587,631

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