Agios Reports Business Highlights and Third Quarter 2019 Financial Results

October 31, 2019

– TIBSOVO® Net Revenue of $17.4 Million; 27% Growth from Q2 2019 –

– Reported Positive Phase 3 ClarIDHy Results for TIBSOVO® in Previously Treated IDH1 Mutant Cholangiocarcinoma; sNDA Submission Planned by Year-End –

– Presented Data from Single Agent Dose-Escalaton Arm of the Phase 1 Study of AG-270 in MTAP Deleted Tumors; Combination Arms with Taxanes in Non-Small Cell Lung Cancer and Pancreatic Cancer Initiated –

– Published Updated DRIVE PK Data for Mitapivat in PK Deficiency in New England Journal of Medicine*; Updated Data from Extension Phase to be Presented at American Society of Hematology Annual Meeting –*

CAMBRIDGE, Mass., Oct. 31, 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 third quarter ended September 30, 2019.

“The third quarter was marked by strong commercial execution, now a full year since the U.S. launch of TIBSOVO®, and several meaningful clinical updates spanning our early and late stage oncology programs that advance our strategy,” said Jackie Fouse, Ph.D., chief executive officer at Agios. “As we look ahead to the remainder of 2019, we are focused on achieving our remaining key milestones, including completing enrollment in our mitapivat PK deficiency pivotal program, establishing proof of concept for mitapivat in thalassemia, initiating our pivotal trial of vorasidenib in low-grade glioma and submission of the TIBSOVO® supplemental new drug application for IDH1 mutant cholangiocarcinoma. These milestones are critical steps toward realizing the value-creation potential for both our oncology and rare genetic disease portfolios in 2020 and beyond.”

THIRD QUARTER 2019 HIGHLIGHTS & RECENT PROGRESS

- Presented data from the single agent dose-escalation portion of the ongoing Phase 1 study of AG-270 in patients with methylthioadenosine phosphorylase (MTAP)-deleted tumors at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in October. The data demonstrated that AG-270 induces reductions in the biomarkers of methionine adenosyltransferase 2A (MAT2A) inhibition, notably plasma concentrations of S-adenosylmethionine (SAM) and tumor levels of symmetrically demethylated arginine (SDMA), at well tolerated doses.
- Presented results from the Phase 3 ClarIDHy study of TIBSOVO® in previously treated isocitrate dehydrogenase-1 (IDH1) mutant cholangiocarcinoma at the European Society for Medical Oncology Congress in September, demonstrating significant improvement in progression free survival compared to placebo.
- Initiated two combination arms for the Phase 1 study of AG-270 in MTAP-deleted tumors, one evaluating AG-270 in combination with docetaxel in MTAP-deleted second-line non-small cell lung cancer and another in combination with nab-paclitaxel and gemcitabine in MTAP-deleted first or second-line pancreatic ductal adenocarcinoma.
- Published new data from the core and extension phases of the DRIVE PK Phase 2 study of mitapivat in adults with pyruvate kinase (PK) deficiency in the September 5, 2019 issue of the *New England Journal of Medicine*, demonstrating sustained increases in hemoglobin for up to 35 months.

KEY UPCOMING MILESTONES

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

Oncology:

- Submit a supplemental new drug application to the FDA for TIBSOVO® for previously treated IDH1 mutant cholangiocarcinoma by year-end.
- Initiate the registration-enabling Phase 3 INDIGO study of vorasidenib in Grade 2 non-enhancing glioma with an IDH mutation by year-end. The study will evaluate 366 patients in 1:1 double-blind randomization to either 50 mg of vorasidenib once daily or placebo. The primary endpoint is progression free survival.

Rare Genetic Diseases:

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

REMAINING 2019 DATA PRESENTATIONS

- Updated data from the perioperative study of TIBSOVO® and vorasidenib in low-grade glioma have been accepted for presentation at the Society for Neuro-Oncology Annual Meeting taking place in Phoenix from November 22-24, 2019.
- Data from the IDH and PKR programs have been accepted for presentation at the American Society of Hematology Annual Meeting taking place in Orlando, Fla. from December 7-10, 2019, including new data from the extension phase of the Phase 2 DRIVE PK study of mitapivat in adults with PK deficiency and important translational data from the Phase 1 combination study of TIBSOVO® and azacitidine in frontline acute myeloid leukemia (AML).

THIRD QUARTER 2019 FINANCIAL RESULTS

Revenue: Total revenue for the third quarter of 2019 was $26.0 million, which includes $17.4 million of net product revenue from U.S. sales of TIBSOVO®, $5.5 million in collaboration revenue and $2.7 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene. This compares to total revenue of $15.2 million for the third quarter of 2018.

Cost of Sales: Cost of sales were $0.4 million for the third quarter of 2019.

Research and Development (R&D) Expenses: R&D expenses were $101.7 million for the third quarter of 2019 compared to $82.6 million for the third quarter of 2018. The increase in R&D expense was primarily attributable to start-up costs for the Phase 3 INDIGO study of vorasidenib in IDH mutated low-grade glioma, the mitapivat pivotal program in PK deficiency and Phase 2 study in thalassemia, and costs of the ongoing Phase 3 TIBSOVO® combination trials in the frontline AML setting.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $33.0 million for the third quarter of 2019 compared to $31.1 million for the third quarter of 2018.

Net Loss: Net loss was $106.2 million for the third quarter of 2019 compared to $94.7 million for the third quarter of 2018.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of September 30, 2019 were $540.5 million compared to $805.4 million as of December 31, 2018. The net decrease of $264.9 million in cash position was primarily driven by net expenditures to fund operations. The company expects that its cash, cash equivalents and marketable securities as of September 30, 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 third 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 refer to conference ID 5996044. 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®, IDHIFA®, 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 inthis 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; 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)

	September 30, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 540,476	$ 805,421
Accounts receivable, net	7,106	5,076
Collaboration receivable – related party	1,838	2,462
Royalty receivable – related party	2,600	2,234
Inventory	5,849	869
Total assets	698,616	858,457
Deferred revenue – related party	66,670	92,519
Stockholders' equity	448,291	687,537

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

	Three Months Ended September 30		Nine Months Ended September 30	
	2019	2018	2019	2018
Revenues:				
Product revenue, net	$ 17,422	$ 4,465	$ 40,287	$ 4,465
Collaboration revenue – related party	5,516	8,732	32,414	42,478
Collaboration revenue – other	420	-	2,202	12,440
Royalty revenue – related party	2,666	2,001	7,569	4,991
Total Revenue	26,024	15,198	82,472	64,374
Cost and expenses:				
Cost of sales	393	695	1,030	695
Research and development, net	101,672	82,561	304,646	247,515
Selling, general and administrative	33,019	31,104	97,200	82,287
Total cost and expenses	135,084	114,360	402,876	330,497
Loss from operations	(109,060)	(99,162)	(320,404)	(266,123)
Interest income	2,887	4,498	11,282	11,889
Net loss	$ (106,173)	$ (94,664)	$ (309,122)	$ (254,234)
Net loss per share – basic and diluted	$ (1.81)	$ (1.63)	$ (5.27)	$ (4.45)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	58,803,534	58,033,386	58,661,607	57,158,492

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Source: Agios Pharmaceuticals, Inc.