Agios Reports Fourth Quarter and Full Year 2019 Financial Results

February 13, 2020

– Fourth Quarter TIBSOVO® Net Revenue of $19.6M and $59.9M for Full Year 2019; Full Year 2020 U.S. TIBSOVO® Net Revenue Expected to be Between $105-115 Million –

– New Clinical Data from Mitapivat in Thalassemia and Sickle Cell Disease to be Submitted for Presentation at the European Hematology Association Annual Congress in June –

– Investigational New Drug Submission for AG-946, a Next Generation PKR Activator, Expected in Q1 –

CAMBRIDGE, Mass., Feb. 13, 2020 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO) today reported business highlights and financial results for the fourth quarter and year ended December 31, 2019. In addition, Agios highlighted key 2020 corporate milestones and data presentations for its clinical development programs.

“On the heels of a busy and productive 2019, I’m more confident than ever in the strength of our team and our ability to make a meaningful impact on the lives of patients through great science, a deep pipeline and differentiated therapies,” said Jackie Fouse, Ph.D., chief executive officer at Agios. “In 2020, our clinical development team is focused on advancing our Phase 3 PK deficiency studies in order to submit a new drug application in 2021, finalizing our pivotal development plan for the PK activation program in thalassemia and establishing proof-of-concept in sickle cell disease. In addition, we are driving enrollment in several Phase 3 studies for our IDH inhibitors in both malignant hematology and solid tumors. Our commercial team is focused on achieving an ambitious revenue target for TIBSOVO® and increasing market development activities in preparation for a potential launch in PK deficiency.”

ANTICIPATED 2020 KEY MILESTONES

Agios expects the following key milestones in 2020:

Hematologic Malignancies

- Deliver full-year U.S. revenue for TIBSOVO® of $105-115 million
- Receive European Medicines Agency CHMP opinion for TIBSOVO® in relapsed or refractory acute myeloid leukemia (AML) with an IDH1 mutation by year-end
- Complete enrollment of the Phase 3 AGILE trial of TIBSOVO® in combination with azacitidine in adult patients with previously untreated IDH1 mutant AML by year-end
- Complete enrollment of the relapsed or refractory myelodysplastic syndrome arm of the TIBSOVO® Phase 1 study of IDH1 mutant advanced hematologic malignancies by year-end

Solid Tumors

- File supplemental new drug application (sNDA) for TIBSOVO® in previously treated IDH1 mutant cholangiocarcinoma by year-end

Rare Genetic Diseases

- Announce topline data for ACTIVATE and ACTIVATE-T pivotal trials for mitapivat in adults with pyruvate kinase (PK) deficiency by year-end
- Submit updated data from the Phase 2 study of mitapivat in thalassemia for presentation at the European Hematology Association (EHA) Congress in June and finalize pivotal development strategy by year-end
- Achieve proof-of-concept for mitapivat in sickle cell disease by mid-2020
- Receive investigational new drug (IND) clearance for AG-946, a next generation PKR activator, and initiate a first-in-human study in healthy volunteers in the first half of 2020

Research

- Achieve at least one new development candidate by year-end

FOURTH QUARTER AND FULL YEAR 2019 FINANCIAL RESULTS

Revenue: Total revenue for the fourth quarter of 2019 was $35.4 million, which includes $12.9 million in collaboration revenue, $19.6 million of net product revenue from sales of TIBSOVO® and $3.0 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene. This compares to $30.0 million for the fourth quarter of 2018, which included $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®. Total revenue for the year ended December 31, 2019 was $117.9 million compared to $94.4 million for the year ended December 31, 2018. The increase in 2019 revenue was primarily

driven by net U.S. sales of TIBSOVO® and were offset by a decline in collaboration revenue due to the recognition of a milestone from Celgene and the upfront payment from CStone in 2018.

Cost of Sales: Cost of sales were $0.3 million for the fourth quarter of 2019 compared to $0.7 million for the fourth quarter of 2018, and $1.3 million for the year ended December 31, 2019 compared to $1.4 million for the comparable period in 2018.

Research and Development (R&D) Expenses: R&D expenses were $106.2 million for the fourth quarter of 2019 compared to $93.8 million for the fourth quarter of 2018 and $410.9 million for the year ended December 31, 2019 compared to $341.3 million for the comparable period in 2018. The increase in R&D expense was primarily attributable to clinical trial activity for mitapivat in PK deficiency and thalassemia; start-up costs for the vorasidenib Phase 3 INDIGO study in low-grade glioma, including required clinical pharmacology studies and companion diagnostic development; and ongoing enrollment in the TIBSOVO® Phase 3 AGILE and HOVON frontline AML combination studies. 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 $34.8 million for the fourth quarter of 2019 compared to $31.9 million for the fourth quarter of 2018, and $132.0 million for the year ended December 31, 2019 compared to $114.1 million for the year ended December 31, 2018. The increase in SG&A expense was primarily attributable to increased investment in marketing activities in preparation for the potential launch of mitapivat and personnel costs related to increased headcount to support growing operations.

Net Loss: Net loss was $102.4 million for the fourth quarter of 2019 compared to $91.8 million for the fourth quarter of 2018, and $411.5 million for the year ended December 31, 2019 compared to a net loss of $346.0 million for the year ended December 31, 2018.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of December 31, 2019 were $717.8 million compared to $805.4 million as of December 31, 2018. The change in cash was primarily driven by expenditures to fund operations of $464.4 million offset by the net proceeds of $277.2 million from the November follow-on offering and cash inflows of $99.3 million from product sales, stock option exercises, royalty revenue, and collaboration reimbursements and milestones. The company expects that its cash, cash equivalents and marketable securities as of December 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 collaboration-related payments, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through at least the end of 2021.

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 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 4195413. 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/Celgene Collaboration

IDHIFA® (enasidenib) and AG-270 are part of our collaboration with Celgene Corporation, a wholly owned subsidiary of Bristol-Myers Squibb Company. 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.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat malignant hematology, solid tumors and rare genetic diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across these three therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or preclinical development. For more information, please visit the company's 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’ 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), mitapivat, vorasidenib, AG-270, AG-636 and AG-946; the potential benefits of Agios' product candidates; its key milestones and guidance for 2020; 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,” “goal,” “hope,” “milestone,” “plan,” “potential,” “possible,” “strategy,” “will,” “vision,” 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; 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, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 717,806	$ 805,421
Accounts receivable, net	8,952	5,076
Collaboration receivable – related party	1,539	2,462
Royalty receivable – related party	2,900	2,234
Inventory	7,331	869
Total assets	890,741	858,457
Deferred revenue – related party	61,513	92,519
Stockholders' equity	640,528	687,537

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

	Three Months Ended December 31,		Years Ended December 31,	
	2019	2018	2019	2018
Revenues:				
Product revenue, net	$ 19,564	$ 9,376	$ 59,851	$ 13,841
Collaboration revenue – related party	6,843	18,183	39,257	60,661
Collaboration revenue – other	6,060	230	8,262	12,670
Royalty revenue – related party	2,973	2,224	10,542	7,215
Total Revenue	35,440	30,013	117,912	94,387
Cost and expenses:				
Cost of sales	287	702	1,317	1,397
Research and development, net	106,248	93,809	410,894	341,324
Selling, general and administrative	34,834	31,858	132,034	114,145
Total cost and expenses	141,369	126,369	544,245	456,866
Loss from operations	(105,929)	(96,356)	(426,333)	(362,479)
Interest income	3,579	4,562	14,861	16,451
Net loss	$ (102,350)	$ (91,794)	$ (411,472)	$ (346,028)
Net loss per share – basic and diluted	$ (1.60)	$ (1.58)	$ (6.86)	$ (6.03)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	63,949,870	58,189,254	59,994,539	57,418,300

Contacts

Investors:
Holly Manning, 617-844-6630
Associate Director, Investor Relations
Holly.Manning@agios.com

Media:
Jessica Rennekamp, 857-209-3286

Associate Director, Corporate Communications
Jessica.Rennekamp@agios.com

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