Agios Reports Business Highlights and Second Quarter 2020 Financial Results

July 30, 2020

– Second Quarter TIBSOVO® Net Revenue of $27.6 Million; Company Reiterates 2020 TIBSOVO® Net U.S. Revenue Guidance of $105–115 Million –

– Significant Clinical Progress for First-in-Class PKR Activator Mitapivat: Established Proof-of-Concept in Sickle Cell Disease; Presented Phase 2 Data in α- and β-Thalassemia; Pivotal Programs to be Initiated in 2021 –

– $255 Million Sale of IDHIFA® Royalty Extends Cash Runway, Inclusive of Thalassemia and Sickle Cell Disease Pivotal Development, through the End of 2022 –

CAMBRIDGE, Mass., July 30, 2020 (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 second quarter ended June 30, 2020.

"The second quarter was a productive and important time at Agios, as we accomplished several key 2020 objectives across our three focus areas of malignant hematology, solid tumors and rare genetic diseases. In particular, we made significant progress on our mitapivat clinical programs, including achieving proof-of-concept in sickle cell disease and planning for our pivotal development programs in both thalassemia and sickle cell disease," said Jackie Fouse, Ph.D., chief executive officer at Agios. "For the remainder of 2020, we are focused on the completion of our pivotal trials ACTIVATE and ACTIVATE-T for mitapivat in pyruvate kinase deficiency and securing regulatory feedback on the pivotal programs in both thalassemia and sickle cell disease to enable their initiation next year, the submission of a supplemental new drug application for TIBSOVO® in cholangiocarcinoma in the first quarter of 2021, driving enrollment in our ongoing clinical trials and continued strong commercial execution."

SECOND QUARTER 2020 HIGHLIGHTS

Rare Genetic Diseases

- Established clinical proof-of-concept for mitapivat in sickle cell disease based on a preliminary analysis of data on eight patients from the Phase 1 study being conducted in collaboration with the National Institutes of Health (NIH). Seven of eight (88%) evaluable patients experienced a hemoglobin increase, with five of eight patients (63%) achieving a hemoglobin increase of ≥1.0 g/dL from baseline. Additionally, the data showed improvements in associated markers of sickling as well as a safety profile consistent with previously reported mitapivat data or expected in the context of sickle cell disease.
- Presented data on 13 patients from the Phase 2 study of mitapivat in non-transfusion-dependent α- and β-thalassemia at the European Hematology Association (EHA) Annual Congress in June. Treatment with mitapivat induced a hemoglobin increase of ≥1.0 g/dL in 12 of 13 (92%) evaluable patients, including four of four (100%) α-thalassemia patients. Additionally, the data showed improvements in associated markers of hemolysis and erythropoiesis as well as a safety profile consistent with previously reported mitapivat data.
- Received Orphan Drug Designation from the Food and Drug Administration (FDA) for mitapivat in thalassemia.

Hematologic Malignancies and Solid Tumors

- TIBSOVO® net sales of $27.6 million, an increase of 22% from the first quarter of 2020; expanded total number of unique prescribers by 15% from the first quarter of 2020.
- Published data from the Phase 3 ClarIDHy study of TIBSOVO® in *The Lancet Oncology*. As a result of this publication, the National Comprehensive Cancer Network (NCCN) guidelines were updated to recommend treatment with TIBSOVO® for patients with advanced IDH1-mutant cholangiocarcinoma.
- Presented updated data from the Phase 1 dose-escalation study of vorasidenib in IDH-mutant non-enhancing glioma at the American Society of Clinical Oncology (ASCO) Annual Meeting in May. In the study, vorasidenib demonstrated prolonged disease control and encouraging preliminary activity, as well as a favorable safety profile consistent with previously reported data.

Corporate

- Completed a $255 million purchase agreement with Royalty Pharma for IDHIFA® (enasidenib) royalty rights and outstanding regulatory milestone payments.

KEY UPCOMING MILESTONES

Rare Genetic Diseases

- Report data from ACTIVATE and ACTIVATE-T, the company's two global pivotal trials for mitapivat in adults with pyruvate

kinase (PK) deficiency, between the end of 2020 and mid-2021.
- Finalize robust pivotal development plan for mitapivat in thalassemia, including both α-and β-thalassemia, as well as transfusion dependent and non-transfusion dependent patient populations, by the end of 2020.
- Initiate first-in-human study in healthy volunteers for AG-946, a next-generation PKR activator, in Q3 2020.

Hematologic Malignancies and Solid Tumors

- Deliver full-year 2020 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 the end of 2020.
- Report mature overall survival data from ClarIDHy Phase 3 study in Q3 2020; if data are supportive, file supplemental new drug application (sNDA) for TIBSOVO® in previously treated IDH1-mutant cholangiocarcinoma in Q1 2021.

Research

- Achieve at least one new development candidate by year-end 2020.

SECOND QUARTER 2020 FINANCIAL RESULTS

Revenue: Total revenue for the second quarter of 2020 was $37.3 million, which includes $27.6 million of net product revenue from sales of TIBSOVO®, $6.4 million in collaboration revenue and $3.3 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene, now a wholly owned subsidiary of Bristol Myers Squibb. This compares to revenue of $26.2 million for the second quarter of 2019. TIBSOVO® net product revenue increased 101% from the same period last year.

Cost of Sales: Cost of sales were $0.7 million for the second quarter of 2020 compared to $0.3 million for the second quarter of 2019.

Research and Development (R&D) Expenses: R&D expenses were $90.9 million for the second quarter of 2020 compared to $107.4 million for the second quarter of 2019. The decrease in R&D expense was primarily attributable to milestone payments related to AG-636 and an undisclosed early-stage research program in the second quarter of 2019, winding down the ClarIDHy Phase 3 study of TIBSOVO® and HOVON startup expenses incurred in the second quarter of 2019, and lower spend across ongoing TIBSOVO® clinical studies as a result of slowed enrollment and reduced activities due to the COVID-19 pandemic.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $36.0 million for the second quarter of 2020 compared to $32.4 million for the second quarter of 2019. The increase in SG&A expense was primarily attributable to the initial gated infrastructure build of the company's European operations offset by reduced travel and industry engagement given restrictions in place to combat the COVID-19 pandemic.

Net Loss: Net loss was $90.5 million for the second quarter of 2020 compared to $109.9 million for the second quarter of 2019.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of June 30, 2020 were $794 million, including the amount received under the Royalty Pharma agreement, compared to $624 million as of June 30, 2019. The company expects that its cash, cash equivalents and marketable securities as of June 30, 2020, together with anticipated product 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, including its pivotal development programs for mitapivat in thalassemia and sickle cell disease, through the end of 2022.

CONFERENCE CALL INFORMATION
Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss its second quarter 2020 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 2955575. 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 the Agios/Celgene Collaboration
In 2010, Agios and Celgene Corporation, now a wholly owned subsidiary of Bristol Myers Squibb, entered into a collaboration agreement focused on cancer metabolism. Under the terms of the agreement, Celgene has worldwide development and commercialization rights for IDHIFA® (enasidenib). Celgene and Agios are currently co-commercializing IDHIFA® in the U.S., and Agios continues to conduct certain clinical development activities within the IDHIFA® development program. Agios is eligible to receive a $25 million payment upon achievement of a specified ex-U.S. commercial milestone event, as well as reimbursement for costs incurred for its co-commercialization efforts and development activities.

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 tablets), IDHIFA® (enasidenib), mitapivat, vorasidenib, AG-270, 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 benefits 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, without limitation: risks and uncertainties related to the impact of the COVID-19 pandemic to Agios' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; 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)

	June 30, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	$ 794,413	$ 717,806
Accounts receivable, net	12,023	8,952
Collaboration receivable – related party	2,537	1,539
Royalty receivable – related party	1,650	2,900
Inventory	11,231	7,331
Total assets	976,141	890,741
Deferred revenue – related party	—	61,513
Stockholders' equity	558,465	640,528

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Product revenue, net	$ 27,581	$ 13,727	$ 50,255	$ 22,865
Collaboration revenue – related party	5,735	8,979	65,832	26,898
Collaboration revenue – other	692	812	1,685	1,782
Royalty revenue – related party	3,339	2,703	6,673	4,903
Total revenue	37,347	26,221	124,445	56,448
Cost and expenses:				
Cost of sales	675	303	1,208	637
Research and development	90,917	107,389	182,173	202,974
Selling, general and administrative	35,951	32,390	74,452	64,181
Total cost and expenses	127,543	140,082	257,833	267,792
Loss from operations	(90,196)	(113,861)	(133,388)	(211,344)
Interest income, net	1,769	3,990	4,705	8,395
Non-cash interest expense for the sale of future revenue	(2,051)	—	(2,051)	—
Net loss	$ (90,478)	$ (109,871)	$ (130,734)	$ (202,949)
Net loss per share – basic and diluted	$ (1.31)	$ (1.87)	$ (1.90)	$ (3.46)

Weighted-average number of common shares used in computing net loss per share – basic and diluted	68,958,091	58,722,244	68,784,109	58,589,167

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Source: Agios Pharmaceuticals, Inc.