Agios Reports Business Highlights and Third Quarter 2020 Financial Results

November 5, 2020

– Third Quarter TIBSOVO® Net Revenue of $31.7 Million; Company Narrows 2020 TIBSOVO® Net U.S. Revenue Guidance to $113–115 Million –

– Topline Data from the Mitapivat Phase 3 ACTIVATE Trial in Non-Transfusion Dependent PK Deficiency Expected by Year-End; Topline Phase 3 ACTIVATE-T Data Expected in Q1 2021 –

– Reported Positive Overall Survival Trend in ClarIDHy Phase 3 Trial of TIBSOVO® in Advanced Cholangiocarcinoma; Company Plans to Submit Supplemental New Drug Application in Q1 2021 –

CAMBRIDGE, Mass., Nov. 05, 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 third quarter ended September 30, 2020.

"In the third quarter, we made significant strides toward executing against our key 2020 priorities and getting our medicines to patients who need them," said Jackie Fouse, Ph.D., chief executive officer at Agios. "Most notably, we generated mature overall survival data from our Phase 3 ClarIDHy study of TIBSOVO® in previously treated cholangiocarcinoma, prepared for topline data readouts from our two global Phase 3 studies of mitapivat in pyruvate kinase deficiency and advanced our pivotal plans for mitapivat in thalassemia and sickle cell disease. We look forward to building on these achievements with a catalyst-rich fourth quarter and 2021."

THIRD QUARTER 2020 & RECENT HIGHLIGHTS

- TIBSOVO® (ivosidenib tablets) net sales of $31.7 million, an increase of 15% from the second quarter of 2020.
- Reported topline mature overall survival results from ClarIDHy Phase 3 study of TIBSOVO® in previously treated IDH1-mutant cholangiocarcinoma patients; submitted final data for presentation at the virtual American Society of Clinical Oncology Gastrointestinal Cancers Symposium (ASCO GI).
- Withdrew European Marketing Authorization Application (MAA) for TIBSOVO® in IDH1-mutant relapsed or refractory acute myeloid leukemia (AML) as a result of feedback from European Medicine Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP).
- Initiated Phase 1 healthy volunteers study of AG-946, a next-generation, orally available, small molecule activator of the metabolic enzyme pyruvate kinase-R (PKR).
- Appointed Jonathan Biller as chief financial officer, head of legal and corporate affairs.

KEY UPCOMING MILESTONES

Rare Genetic Diseases

- Report topline data from ACTIVATE, the company's global pivotal trial for mitapivat in adults with pyruvate kinase (PK) deficiency who do not receive regular transfusions, by the end of 2020.
- Report topline data from ACTIVATE-T, the company's global pivotal trial for mitapivat in adults with pyruvate kinase (PK) deficiency who receive regular transfusions, in Q1 2021.
- Finalize 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.
- Finalize pivotal development plan for mitapivat in sickle cell disease in the first half of 2021.

Hematologic Malignancies and Solid Tumors

- Deliver full-year 2020 U.S. revenue for TIBSOVO® of $113-115 million; the company had previously provided guidance of $105-115 million.
- Submit a supplemental new drug application for TIBSOVO® in previously treated cholangiocarcinoma in Q1 2021.

Research

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

UPCOMING INVESTOR EVENTS

- **November 19, 2020**: Agios will host a webinar focused on its pyruvate kinase-R (PKR) activation clinical programs, including Agios' leadership in elucidating the PKR activation mechanism, the commercial opportunity for each program and the company's preparations for its first potential launch in a rare genetic disease, expected in 2022.
- **December 8, 2020**: Agios will host a webinar to share updated data from the Phase 1 trial of mitapivat in sickle cell

disease, which is being conducted in collaboration with the National Institutes of Health (NIH), as well as an overview of the company's Phase 3 development plans for mitapivat in patients with thalassemia.

THIRD QUARTER 2020 FINANCIAL RESULTS

Revenue: Total revenue for the third quarter of 2020 was $34.7 million, which includes $31.7 million of net product revenue from sales of TIBSOVO®, $2.3 million in collaboration revenue and $0.7 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 $26.0 million for the third quarter of 2019. Total revenue increased 33% from the same period last year, driven by an 82% increase in TIBSOVO® net product revenue and offset by a decrease in collaboration revenue due to completion of the research and development services performance obligation with Celgene in the second quarter, as well as an adjustment in sales reserves taken in the third quarter by Bristol Myers Squibb.

Cost of Sales: Cost of sales were $0.6 million for the third quarter of 2020 compared to $0.4 million for the third quarter of 2019.

Research and Development (R&D) Expenses: R&D expenses were $89.6 million for the third quarter of 2020 compared to $101.7 million for the third quarter of 2019. The decrease in R&D expense was primarily attributable to a decrease in TIBSOVO® clinical development costs, including winding down the ClarIDHy Phase 3 study.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $34.8 million for the third quarter of 2020 compared to $33.0 million for the third quarter of 2019. The increase in SG&A expense was primarily attributable to increased workforce expenses, offset by a decrease in external spending due to COVID-19 and cost savings initiatives.

Net Loss: Net loss was $99.0 million for the third quarter of 2020 compared to $106.2 million for the third quarter of 2019.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of September 30, 2020 were $722.4 million, compared to $540.5 million as of September 30, 2019. The company expects that its cash, cash equivalents and marketable securities as of September 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 to 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 third 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 7578909. 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, 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)

	September 30, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	$ 722,428	$ 717,806
Accounts receivable, net	18,989	8,952
Collaboration receivable – related party	2,334	1,539
Royalty receivable – related party	—	2,900
Inventory	11,371	7,331
Total assets	908,449	890,741
Deferred revenue – related party	—	61,513
Stockholders' equity	480,452	640,528

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(In thousands, except share and per share data)	2020	2019	2020	2019
Revenues:				
Product revenue, net	$ 31,716	$ 17,422	$ 81,971	$ 40,287
Collaboration revenue – related party	1,206	5,516	67,038	32,414
Collaboration revenue – other	1,101	420	2,786	2,202
Royalty revenue – related party	683	2,666	7,356	7,569
Total revenue	34,706	26,024	159,151	82,472
Cost and expenses:				
Cost of sales	638	393	1,846	1,030
Research and development	89,555	101,672	271,728	304,646
Selling, general and administrative	34,840	33,019	109,292	97,200
Total cost and expenses	125,033	135,084	382,866	402,876
Loss from operations	(90,327)	(109,060)	(223,715)	(320,404)
Interest income, net	1,115	2,887	5,820	11,282
Non-cash interest expense for the sale of future revenue	(9,767)	—	(11,818)	—
Net loss	$ (98,979)	$ (106,173)	$ (229,713)	$ (309,122)
Net loss per share – basic and diluted	$ (1.43)	$ (1.81)	$ (3.33)	$ (5.27)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	69,144,061	58,803,534	68,905,853	58,661,607

Contacts

Investors:
Holly Manning, 617-844-6630
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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