Agios Reports First Quarter 2020 Financial Results and Provides Update on Business Operations and COVID-19 Response

April 30, 2020

– First Quarter TIBSOVO® Net Revenue of $22.7 Million; Company Reiterates 2020 TIBSOVO® Net U.S. Revenue Guidance of $105–115 Million –

– New Clinical Data from Mitapivat in Thalassemia Accepted for Presentation at the European Hematology Association Annual Congress in June –

– Proof-of-Concept Decision for Mitapivat in Sickle Cell Disease on Track for Mid-2020; Data to be Submitted for Presentation at the American Society of Hematology Annual Meeting –

– Cash Conservation Efforts Extend Runway to the End of June 2022 –

CAMBRIDGE, Mass., April 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 first quarter ended March 31, 2020. In addition, Agios provided an update on its response to the global COVID-19 pandemic and the expected impact on its business operations.

“Though there is no modern playbook for a crisis like the COVID-19 pandemic, we moved quickly to reduce the risk of our team’s and communities’ exposure to the virus and took action to enable uninterrupted access to our commercial and clinical medicines for the patients who are counting on us,” said Jackie Fouse, Ph.D., chief executive officer at Agios.

“Despite the challenges we faced in the first quarter in the wake of the COVID-19 pandemic, we made significant clinical and commercial progress, including completing enrollment in the ACTIVATE pivotal trial of mitapivat in PK deficiency, delivering strong TIBSOVO® performance and achieving key U.S. and EU regulatory milestones. We have also made important resource allocation decisions with the goal of delivering on our key business objectives while conserving cash and increasing our financial flexibility,” continued Dr. Fouse. “Through these efforts, we remain on track to achieve our Agios 2025 strategic vision. As we have all worked to adapt to these unexpected and trying circumstances, the teamwork and resilience demonstrated by my colleagues has truly been remarkable and provides confidence that we will come out of these challenging times even stronger than we were before.”

FIRST QUARTER 2020 HIGHLIGHTS & RECENT PROGRESS

- TIBSOVO® net sales increased by 16% and total number of unique prescribers expanded by 25% from the fourth quarter of 2019; this growth was driven largely by uptake in both the newly diagnosed and relapsed and refractory acute myeloid leukemia (AML) segments.
- Enrollment was completed in ACTIVATE, the ongoing pivotal trial of mitapivat in adults with pyruvate kinase (PK) deficiency who do not regularly receive transfusions.
- In March, mitapivat was issued a positive opinion on its application for orphan drug designation by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products for the treatment of adults with PK deficiency.
- In March, Agios received clearance from the U.S. Food and Drug Administration (FDA) for its investigational new drug application for AG-946, a next generation PKR activator.
- Updated data from the Phase 1 study of vorasidenib in non-enhancing low-grade glioma have been accepted for presentation at the 2020 American Society of Clinical Oncology (ASCO) Annual Meeting in May, which is being held virtually.
- Data from the Phase 2 study of mitapivat in thalassemia have been accepted for presentation at the European Hematology Association (EHA) Annual Congress in June, which will be held virtually.

COVID-19 RESPONSE & IMPACT ON BUSINESS OUTLOOK

TIBSOVO®

To date, patient and physician demand for TIBSOVO® (ivosidenib tablets) – an oral therapy for AML – has not been negatively impacted by the COVID-19 pandemic. AML is a serious disease that progresses rapidly if untreated, and in most cases treatment delays are not recommended. Professional oncology guidelines for the treatment of cancer patients during the pandemic favor effective oral options with well tolerated safety profiles.

Based on current inventory levels and a robust supply chain strategy, Agios does not anticipate interruptions to supply of TIBSOVO®, even if extended disruptions to manufacturing operations were to occur as a result of the COVID-19 pandemic.

In addition, Agios has taken steps to ensure new and existing TIBSOVO® patients can access the medication. TIBSOVO® can be delivered directly to patients’ residences to ensure they can receive their medication even if they are homebound for a prolonged period of time. The myAgios ™ patient assistance program has remained a constant resource for patients, including those who have lost health insurance due to unemployment.

Based on these considerations, Agios continues to expect its 2020 TIBSOVO® net U.S. revenue to be between $105 and 115 million.

Clinical Programs

Because hospitals globally have shifted resources to treat patients with COVID-19, Agios expects some delays in its clinical programs based on anticipated challenges related to data collection, access to trial sites and patient enrollment.

In February, Agios established a clinical trial task force to ensure the safety of patients in its clinical trials and to support patients on a case-by-case basis to enable their continued participation in the studies. Where appropriate, Agios has instituted home visits, telemedicine approaches, the use of local laboratories and courier shipments of investigational medicines.

The status of each Agios clinical program, along with the expected impact of COVID-19, is outlined below. The ultimate extent of the impact of the COVID-19 pandemic on clinical trial enrollment, continuation and data collection will depend on future developments, which are highly uncertain.

Rare Genetic Diseases

Clinical Trial	Status & Program Updates
ACTIVATE: Ongoing pivotal trial of mitapivat in adults with PK deficiency who do not regularly receive transfusions	• Enrollment is complete with 80 patients. • Topline data are now expected between the end of 2020 and mid-2021 versus previous guidance of the end of 2020; the potential delay is due to anticipated challenges with clinical trial site access after the last patient has completed the study.
ACTIVATE-T: Ongoing pivotal trial of mitapivat in adults with PK deficiency who regularly receive transfusions	• Enrollment is complete with 27 patients. • Topline data are now expected between the end of 2020 and mid-2021 versus previous guidance of the end of 2020; the potential delay is due to anticipated challenges with clinical trial site access after the last patient has completed the study.
Thalassemia Phase 2: Ongoing proof-of-concept trial of mitapivat in alpha- and beta-thalassemia	• Enrollment is complete with 20 patients. • Data on 13 patients will be presented at EHA in June.
Sickle Cell Disease Phase 2: Ongoing proof-of-concept trial of mitapivat in sickle cell disease being run under a Cooperative Research and Development Agreement (CRADA) with the U.S. National Institutes of Health (NIH)	• New enrollment is paused as a result of the COVID-19 pandemic. • The decision on proof-of-concept for mitapivat in sickle cell disease remains on track for mid-2020. • Data are expected to be submitted by NIH for presentation at the American Society of Hematology (ASH) Annual Meeting in December.
AG-946 Phase 1: First-in-human study of next-generation PKR activator in healthy volunteers	• Study initiation is expected in mid-2020.

Hematologic Malignancies

Clinical Trial	Status & Program Updates
TIBSOVO® Phase 1: Relapsed and Refractory AML EU Filing	• The EU regulatory process for TIBSOVO® in relapsed and refractory AML remains on track with a CHMP opinion expected by the end of 2020.
AGILE: Ongoing Phase 3 trial of TIBSOVO® in combination with azacitidine in frontline AML	• Enrollment completion is now expected in 2021 versus previous guidance of the end of 2020; this delay is due to COVID-19 related delays in site start-up activities and enrollment interruptions.
HOVON150/AMLSG29: Ongoing intergroup-sponsored Phase 3 trial of TIBSOVO® or IDHIFA® (enasidenib) in combination with standard induction and consolidation chemotherapy in frontline AML	• Enrollment has slowed as a result of COVID-19 related delays in site start-up activities and enrollment interruptions. • Agios intends to provide an update on expected timing of enrollment completion when site activation is complete and patient enrollment has returned to expected levels.

Myelodysplastic Syndrome (MDS): Ongoing expansion arm of the Phase 1 trial of TIBSOVO® in hematologic malignancies	• Enrollment completion is now expected in 2021 versus previous guidance of the end of 2020; this delay is due to COVID-19 related delays in site start-up activities and enrollment interruptions.
AG-636 Phase 1: First-in-human dose-escalation trial of DHODH inhibitor in lymphoma	• As a result of limited enrollment in the study, Agios will stop in-house development of AG-636 and evaluate partnering options.

Solid Tumors

Clinical Trial	Status & Program Updates
ClarIDHy: Ongoing Phase 3 trial of TIBSOVO® in previously treated cholangiocarcinoma	• Agios still expects mature overall survival data from the study in mid-2020, but anticipates delays in collecting the data from trial sites and executing the data cleaning process. • Agios now expects to file a supplemental new drug application (sNDA) for TIBSOVO® in previously treated cholangiocarcinoma between the end of 2020 and mid-2021 versus previous guidance of the end of 2020.
INDIGO: Ongoing Phase 3 trial of vorasidenib in low-grade glioma	• Enrollment has slowed as a result of COVID-19 related delays in site start-up activities and enrollment interruptions. • Agios intends to provide an update on expected timing of enrollment completion when site activation is complete and patient enrollment has returned to expected levels.
AG-270 Phase 1: Ongoing dose-escalation arms evaluating AG-270 in combination with taxanes in non-small cell lung cancer and pancreatic cancer	• Enrollment has slowed as a result of COVID-19 related delays at trial sites. • A go/no-go decision is still expected no later than 2022.

Research

- Agios remains on track to name a new development candidate by the end of the year.
- Agios conducted a prioritization exercise and made the decision to pause certain research programs, including its program in Friedreich's Ataxia.

Cash Conservation Actions

In order to conserve cash while supporting the execution of critical business objectives during this period of uncertainty, Agios has made decisions to cease in-house development of AG-636; delay select research programs, including the Friedreich's Ataxia program, and longer-term clinical studies; limit staff hiring and significantly reduce contract workforce; and pause certain infrastructure projects. Additional savings are anticipated across the business as a result of reduced spending levels that will occur naturally due to the COVID-19 pandemic, such as travel expenses and clinical trial spend.

FIRST QUARTER 2020 FINANCIAL RESULTS

Revenue: Total revenue for the first quarter of 2020 was $87.1 million, which includes $22.7 million of net product revenue from sales of TIBSOVO®, $61.1 million in collaboration revenue and $3.3 million in royalty revenue from net global sales of IDHIFA® under the collaboration agreement with Celgene. This compares to revenue of $30.2 million for the first quarter of 2019.

The year-over-year increase in total revenue was primarily due to a $42.2 million increase in collaboration revenue. As previously announced, Celgene declined to extend the metabolic immuno-oncology research collaboration with Agios. In addition, Celgene declined its option to designate one undisclosed research program for continued development and opt-in right. As a result, Agios recognized the majority of the deferred revenue during the first quarter of 2020. The remainder of the deferred revenue balance will be recognized in the second quarter of 2020 through the expiration of the research term in mid-May. The revenue increase was also driven by an increase of $13.5 million of net sales of TIBSOVO®.

Cost of Sales: Cost of sales were $0.5 million for the first quarter of 2020 compared to $0.3 million for the first quarter of 2019.

Research and Development (R&D) Expenses: R&D expenses were $91.3 million for the first quarter of 2020 compared to $95.6 million for the first quarter of 2019. The decrease in R&D expenses was primarily due to the recognition of milestones for the initiation of the HOVON150/AMLSG29 study in the first quarter of 2019 and decreased activity for the ClarIDHy study and the Phase 1 hematologic malignancy study of TIBSOVO®.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $38.5 million for the first quarter of 2020 compared to $31.8 million

for the first quarter of 2019. The increase in SG&A expenses were driven by higher personnel costs, including stock-based compensation expense, related to our workforce.

Net Loss: Net loss was $40.3 million for the first quarter of 2020 compared to $93.1 million for the first quarter of 2019.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of March 31, 2020 were $613.1 million compared to $707.8 million as of March 31, 2019.

As a result of the company's cash conservation actions, the company expects that its cash, cash equivalents and marketable securities as of March 31, 2020, 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 June 2022.

CONFERENCE CALL INFORMATION

Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss first 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 referring to conference ID 1151878. 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 2025" Strategic Vision
The "Agios 2025" strategic vision delineates the company's view for growth with established and expanding franchises focused on treating hematologic malignancies, solid tumors and rare genetic diseases. As part of this vision, Agios expects to achieve the following milestones by the end of 2025:

- **4 marketed medicines** discovered and developed at Agios
- Approvals in **8+ indications** spanning hematologic malignancies, solid tumors and rare genetic diseases
- **6+ molecules in the clinic** generated by the company's internal research discovery engine
- **Cash-flow positive** within the six-year timeframe

About Agios/Celgene Collaboration
IDHIFA® (enasidenib) is 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.

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.

Condensed Consolidated Balance Sheet Data
(in thousands)

(Unaudited)

	March 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 613,124	$ 717,806
Accounts receivable, net	11,813	8,952
Collaboration receivable – related party and other	4,610	3,467
Royalty receivable – related party	3,300	2,900
Inventory	9,778	7,331
Total assets	799,738	890,741
Deferred revenue – related party	4,748	61,513
Stockholders' equity	625,299	640,528

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

	Three Months Ended March 31,	
	2020	2019
Revenues:		
Product revenue, net	$ 22,674	$ 9,138
Collaboration revenue – related party and other	61,090	18,889
Royalty revenue – related party	3,334	2,200
Total Revenue	87,098	30,227
Cost and expenses:		
Cost of sales	533	334
Research and development, net	91,256	95,585
Selling, general and administrative	38,501	31,791
Total cost and expenses	130,290	127,710
Loss from operations	(43,192)	(97,483)
Interest income	2,936	4,405
Net loss	$ (40,256)	$ (93,078)
Net loss per share – basic and diluted	$ (0.59)	$ (1.59)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	68,608,279	58,453,918

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

Source: Agios Pharmaceuticals, Inc.