Agios Reports Third Quarter 2018 Financial Results

November 1, 2018

– Supplemental New Drug Application Submission for Single Agent TIBSOVO® (ivosidenib) in Newly Diagnosed IDH1m AML Patients Not Eligible for Standard Treatment Planned by the End of January 2019 –

– Enrollment for Phase 3 AGILE Trial of Ivosidenib Combination with Azacitidine in Newly Diagnosed AML Patients Not Eligible for Intensive Chemotherapy Expected to Complete in 2020 with Revised Primary Endpoint –

– Mitapivat Pivotal Trials ACTIVATE and ACTIVATE-T in Adults with PK Deficiency Expected to Complete Enrollment in 2019 –

– TIBSOVO® Launch in R/R IDH1m AML On-track; Reported Revenue of $4.5M in First Partial Quarter of Launch –

CAMBRIDGE, Mass., Nov. 01, 2018 (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, 2018.

"On the heels of the third quarter U.S. approval of TIBSOVO®, our first wholly owned precision medicine, we remain focused on executing on our remaining 2018 milestones and continuing to create value from our portfolio," said David Schenkein, M.D., chief executive officer at Agios. "Based on regulatory discussions, we are accelerating our frontline strategy in IDH1m AML with the planned submission of an sNDA expanding TIBSOVO®'s label to newly diagnosed AML patients not eligible for standard treatment and a shorter enrollment timeline for the Phase 3 AGILE trial. In addition, we continue to activate new sites globally for our pivotal PK deficiency program and accrue patients in the dose-escalation portion of the Phase 1 study in MTAP-deleted tumors. We believe these programs coupled with our robust preclinical pipeline, support our next phase of growth toward becoming a fully integrated, sustainable biopharmaceutical company."

THIRD QUARTER & RECENT 2018 HIGHLIGHTS

- Launched TIBSOVO® (ivosidenib) for the treatment of adult patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation as detected by a test approved by the U.S. Food and Drug Administration (FDA).
- Awarded the U.S. Prix Galien Award for Best Pharmaceutical Product of 2018 for IDHIFA® (enasidenib), an isocitrate dehydrogenase-2 (IDH2) inhibitor indicated for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (AML) with an IDH2 mutation as detected by a test approved by the FDA. Each year, the Prix Galien USA Committee recognizes outstanding achievements in improving the human condition through the development of innovative therapies.
- Reached agreement with the FDA to submit a supplemental new drug application (sNDA) for single agent TIBSOVO® in newly diagnosed AML patients with an IDH1 mutation who are not eligible for standard treatment.
- Reached agreement with the FDA that event free survival (EFS) is an acceptable primary endpoint for the Phase 3 AGILE trial of ivosidenib combination with azacitidine in newly diagnosed AML patients with an IDH1 mutation who are ineligible for intensive chemotherapy. Full enrollment for AGILE is now expected to complete in 2020 vs. previous guidance of 2021.
- Received global rights to AG-881, a brain-penetrant, pan-IDH inhibitor that was previously part of a joint worldwide collaboration with Celgene.
- Submitted an investigational new drug (IND) application for AG-636, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH) for the treatment of hematologic malignancies.
- Announced that effective February 1, 2019, CEO David Schenkein, M.D., will transition to the role of executive chairman of the board of directors and Jacqualyn (“Jackie”) Fouse, Ph.D., will succeed Dr. Schenkein as Agios’ next chief executive officer.

KEY UPCOMING MILESTONES

The company expects to achieve the following near-term milestones:

Cancer:

- Submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for TIBSOVO® for the treatment of adult patients with R/R AML with an IDH1 mutation by year end 2018.
- Support, in conjunction with Celgene, the initiation of HOVON 150, an intergroup sponsored, global, registration-enabling Phase 3 trial combining ivosidenib or enasidenib with standard induction and consolidation chemotherapy followed by a maintenance therapy period in frontline AML patients with an IDH1 or IDH2 mutation, respectively, by year end 2018.
- Submit an sNDA to the FDA for TIBSOVO® for the treatment of patients with newly diagnosed AML with an IDH1 mutation who are not eligible for standard therapy by the end of January 2019.

Rare Genetic Diseases:

- Initiate a Phase 2 proof-of-concept trial of mitapivat in thalassemia by year end 2018.

FOURTH QUARTER CLINICAL DATA PRESENTATIONS

- Updated data from Phase 1 trial of AG-881 in solid tumors, including glioma, has been accepted as an oral presentation at the 2018 Society for Neuro-Oncology (SNO) Annual Meeting on November 15-18 in New Orleans.
- Updated data from the ongoing Phase 1 trial of single agent ivosidenib in IDH1m hematologic malignancies in a subset of patients with newly diagnosed AML not eligible for standard therapy has been accepted as an oral presentation at the 2018 American Society of Hematology (ASH) Annual Meeting and Exposition on December 1-4 in San Diego.
- Updated data from the ongoing Phase 1 combination trial of ivosidenib or enasidenib with standard-of-care intensive chemotherapy in patients with newly diagnosed AML with an IDH2 or IDH1 mutation also been accepted as an oral presentation at ASH.
- Updated data in myelodysplastic syndrome (MDS) from the ongoing Phase 1 study of single agent ivosidenib in IDH1m hematologic malignancies has been accepted as a poster presentation at ASH.

THIRD QUARTER 2018 FINANCIAL RESULTS

Revenue for the quarter ended September 30, 2018 was $15.2 million, which includes $4.5 million of net product revenue from U.S. sales of TIBSOVO®, $8.7 million of collaboration revenue and $2.0 million of royalty revenue from net global sales of IDHIFA® under our collaboration agreements with Celgene. Revenue for the quarter ended September 30, 2017 was $11.4 million and consisted of $10.6 million of collaboration revenue and $0.7 million of royalty revenue under our agreements with Celgene. The year-over-year increase in total revenue for the third quarter was primarily driven by U.S. sales of TIBSOVO® and royalty revenue from U.S. sales of IDHIFA®, offset by a decrease in collaboration revenue recognized during the quarter.

Cost of sales for the quarter ended September 30, 2018 were $0.7 million and relate to manufacturing costs associated with TIBSOVO® sales.

Research and development (R&D) expenses were $82.6 million, including $13.4 million of stock-based compensation expense, for the quarter ended September 30, 2018, compared to $72.9 million, including $7.6 million in stock-based compensation expense, for the comparable period in 2017. The increase in R&D expense was primarily attributable to start-up costs for the mitapivat (AG-348) pivotal program in PK deficiency and IND enabling activities for AG-636, our DHODH inhibitor. R&D expense also increased as a result of ongoing research efforts across our discovery platform programs.

Sales, general and administrative (SG&A) expenses were $31.1 million, including $10.8 million of stock-based compensation expense, for the quarter ended September 30, 2018, compared to $17.5 million, including $4.6 million of stock-based compensation expense, for the quarter ended September 30, 2017. The increase in SG&A expense was primarily attributable to the growth in our U.S. commercial organization to support the launch of TIBSOVO® and personnel costs related to increased headcount.

Net loss for the quarter ended September 30, 2018 was $94.7 million, compared to a net loss of $77.1 million for the quarter ended September 30, 2017.

Cash, cash equivalents and marketable securities as of September 30, 2018 were $878.4 million, compared to $567.8 million as of December 31, 2017. The increase in cash was driven by the net proceeds of $516.2 million from the January follow-on offering, $14.8 million of cost reimbursements and royalty payments under our collaboration agreements with Celgene, $12.0 million under our collaboration agreement with CStone and $29.2 million received from employee stock transactions. This was offset by expenditures to fund operations of $263.0 million during the nine months ended September 30, 2018.

The company expects that its cash, cash equivalents and marketable securities as of September 30, 2018, 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 2018 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 5285068. 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. 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 TIBSOVO® (ivosidenib)

TIBSOVO® (ivosidenib) is an isocitrate dehydrogenase-1 (IDH1) inhibitor indicated for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (AML) with a susceptible IDH1 mutation as detected by an FDA-approved test. Please see full Prescribing Information including

Boxed Warning at TIBSOVO.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 co-development and co-commercialization 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 IDHIFA® (enasidenib), TIBSOVO® (ivosidenib), AG-881, mitapivat, AG-270 and AG-636; the potential benefits of Agios' product candidates; its key milestones for 2018; 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," "believe," "could," "estimate," "expect," "hope," "intend," "may," "milestone," "path," "plan," "possible," "potential," "predict," "prepare," "project," "strategy," "will," "would," 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 collaborator, Celgene, 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 and 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, such as its agreements with Celgene and CStone Pharmaceuticals; 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, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 878,400	$ 567,750
Accounts receivable, net	2,631	-
Collaboration receivable – related party	3,395	2,448
Royalty receivable – related party	1,863	1,222
Inventory	863	-
Total assets	929,079	614,397
Deferred revenue – related party	108,435	163,640
Stockholders' equity	760,213	375,503

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues:				

Product revenue, net	$ 4,465	$ -	$ 4,465	$ -
Collaboration revenue – related party	8,732	10,643	42,478	32,497
Collaboration revenue – other	-	-	12,440	-
Royalty revenue – related party	2,001	715	4,991	715
Total Revenue	15,198	11,358	64,374	33,212
Cost and expenses:				
Cost of sales	695	-	695	-
Research and development, net	82,561	72,917	247,515	215,465
Sales, general and administrative	31,104	17,458	82,287	48,411
Total cost and expenses	114,360	90,375	330,497	263,876
Loss from operations	(99,162)	(79,017)	(266,123)	(230,664)
Interest income	4,498	1,880	11,889	4,279
Net loss	(94,664)	(77,137)	(254,234)	(226,385)
Net loss per share – basic and diluted	(1.63)	(1.59)	(4.45)	(4.94)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	58,033,386	48,459,424	57,158,492	45,851,203

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