Agios Provides Business Update on Discovery Research Strategy and Pipeline, Progress on Clinical Programs, Commercial Launch Preparations and Reports First Quarter 2018 Financial Results at Investor Day

May 4, 2018

– Commercial Infrastructure In Place Ahead of August 21, 2018 PDUFA Action Date for TIBSOVO® (Ivosidenib) for IDH1m R/R AML –

– Clinical Portfolio Advancing with Four Compounds in Development and Five Pivotal Trials Ongoing or Planned Across Three Distinct Disease Areas –

– Drug Discovery Platform Poised to Deliver New Research Programs with Next IND Expected in Q4 2018; Three Rare Genetic Disease Programs Unveiled –

– Company in a Strong Financial Position to Launch TIBSOVO® (Ivosidenib) and Execute Research and Clinical Plans with Q1 2018 Ending Cash, Cash Equivalents and Marketable Securities of $995M –

CAMBRIDGE, Mass., May 04, 2018 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, is hosting an Investor Day in New York City today. During the event, the company will provide a comprehensive business update and report financial results for the first quarter ended March 31, 2018. The presentations will highlight how Agios' drug discovery platform and broad clinical portfolio set Agios on the path to become a sustainable, multi-product biopharmaceutical company. The event will be webcast today starting at 8:00 a.m. ET at investor.agios.com.

"As we prepare to launch our second Agios-discovered and first wholly owned medicine later this year, we continue to invest in our productive drug discovery engine and advance a robust pipeline of first-in-class medicines," said David Schenkein, M.D., chief executive officer at Agios. "Our first quarter progress against that objective was highlighted by the NDA acceptance of TIBSOVO® in IDH1m relapsed or refractory AML and multiple clinical trial initiations, including dosing the first patient with our MAT2A inhibitor AG-270 and the start of our AG-348 pivotal program in PK deficiency."

HIGHLIGHTS FROM INVESTOR DAY PRESENTATIONS

- Communicated a robust research pipeline consisting of nine advanced drug discovery programs against novel targets across oncology, rare genetic diseases and metabolic immuno-oncology with the potential to deliver multiple INDs over the next 24 months.
- Expanded rare genetic disease portfolio:
  - The company disclosed active research programs in three rare genetic diseases: phenylketonuria (PKU), erythroid porphyria and Friedreich's ataxia
  - The most advanced research program is in PKU, where Agios has developed a novel approach to stabilize the mutant phenylalanine hydroxylase (PAH) protein and has demonstrated significantly decreased blood phenylalanine levels in a severe pre-clinical model of the disease. PKU is an autosomal recessive disease caused by mutations in the PAH gene affecting approximately 16,000 patients in the U.S.[1]
- Updated clinical milestones to advance the development of isocitrate dehydrogenase (IDH) 1 inhibitors in solid tumors:
  - Glioma pivotal development strategy expected to be finalized by year-end 2018
  - Completion of enrollment of ClarIDHy, a global, registration-enabling randomized Phase 3 study for ivosidenib in IDH1m positive advanced cholangiocarcinoma, accelerated to the first half of 2019
- Announced acceptance of the following presentations at 2018 American Society of Clinical Oncology (ASCO) Annual Meeting:
  - Updated data from the expansion phase of the ongoing Phase 1 study of ivosidenib in IDH1m relapsed or refractory (R/R) acute myeloid leukemia (AML)
  - Updated data from the ongoing Phase 1/2 combination trial of enasidenib or ivosidenib with VIDAZA® in patients with newly diagnosed AML with an IDH2 or IDH1 mutation ineligible for intensive chemotherapy
  - First clinical data from the Phase 1 study of AG-881 in advanced IDHm positive solid tumors, including glioma
- Completed commercial infrastructure build, including the deployment of an expanded sales force, to successfully launch TIBSOVO® (ivosidenib) within 48 hours of potential FDA approval.

FIRST QUARTER 2018 HIGHLIGHTS & RECENT PROGRESS

- Initiated ACTIVATE-T, a single-arm pivotal trial for AG-348, in adult pyruvate kinase (PK) deficiency patients who receive regular blood transfusions.
- Initiated PEAK, a global registry, for adult and pediatric patients with PK deficiency.
- Initiated a perioperative 'window' trial with ivosidenib and AG-881 in IDHm low-grade glioma to further investigate their effects on brain tumor tissue.

- Initiated a Phase 1 dose-escalation trial for AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor, in patients with methylthioadenosine phosphorylase (MTAP)-deleted tumors.
- Announced FDA acceptance, priority review and a Prescription Drug User Fee Act (PDUFA) action date of August 21, 2018 for the new drug application (NDA) for TIBSOVO® (ivosidenib) for the treatment of patients with R/R AML with an IDH1 mutation.
- Completed an underwritten public offering of 8,152,986 shares of common stock at the offering price of $67.00 per share, resulting in proceeds to the company, net of underwriting discounts and commissions, of approximately $516.2 million.

UPCOMING 2018 MILESTONES & EXPECTED DATA PRESENTATIONS

The company expects to achieve the following additional milestones in 2018:

Cancer:

- Potential approval and commercialization of TIBSOVO® (ivosidenib) in the United States for R/R AML with an IDH1 mutation in the third quarter of 2018.
- Submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for TIBSOVO® (ivosidenib) for the treatment of patients with R/R AML and an IDH1 mutation in the fourth quarter of 2018.
- Support, in collaboration with Celgene, the initiation of HO150, an intergroup sponsored, global, registration-enabling Phase 3 trial combining ivosidenib or enasidenib with standard induction and consolidation chemotherapy in frontline AML patients with an IDH1 or IDH2 mutation in the fourth quarter of 2018.
- Present updated data from the ongoing Phase 1 combination trial of enasidenib or ivosidenib with standard-of-care intensive chemotherapy in patients with newly diagnosed AML with an IDH2 or IDH1 mutation to the 2018 American Society of Hematology (ASH) Annual Meeting and Exposition.

Rare Genetic Diseases:

- Initiate ACTIVATE, a global, placebo-controlled, pivotal trial for AG-348 in approximately 80 adults with PK deficiency who do not receive regular blood transfusions in the second quarter of 2018.
- Initiate a Phase 2 proof of concept trial of AG-348 in thalassemia in the fourth quarter of 2018.

Research:

- Submit an investigational new drug (IND) application for our newest development candidate, AG-636, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH) for the treatment of hematologic malignancies in the fourth quarter of 2018.

FIRST QUARTER 2018 FINANCIAL RESULTS & CASH GUIDANCE

Revenue for the quarter ended March 31, 2018 was $8.8 million, which includes $7.4 million of collaboration revenue and $1.4 million of royalty revenue from net sales of IDHIFA®. Revenue for the quarter ended March 31, 2017 was $10.5 million and consisted solely of collaboration revenue. The decrease in collaboration revenue recognized for the quarter ended March 31, 2018 compared to the comparable period in 2017 was primarily driven by adoption of the new revenue recognition standard.

Research and development (R&D) expenses were $78.2 million, including $8.6 million of stock-based compensation expense, for the quarter ended March 31, 2018, compared to $62.7 million, including $7.0 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 AG-348 pivotal program in PK deficiency, including the initiation of the ACTIVATE-T trial. R&D expense also increased as a result of the initiation of a Phase 1 dose-escalation study of AG-270, our first-in-class MAT2A inhibitor, and IND enabling activities for AG-636, our DHODH inhibitor.

General and administrative (G&A) expenses were $24.6 million, including $5.9 million of stock-based compensation expense, for the quarter ended March 31, 2018, compared to $14.8 million, including $3.7 million of stock-based compensation expense, for the quarter ended March 31, 2017. The increase in G&A expense was primarily attributable to the growth in our U.S. commercial organization in order to support the expected launch of TIBSOVO® (ivosidenib) in the third quarter of 2018.

Net loss for the quarter ended March 31, 2018 was $90.8 million, compared to a net loss of $66.2 million for the quarter ended March 31, 2017.

Cash, cash equivalents and marketable securities as of March 31, 2018 were $994.7 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, $4.4 million of cost reimbursements under our collaboration agreements with Celgene and $12.3 million received from employee stock transactions. This was offset by expenditures to fund operations of $104.8 million during the quarter ended March 31, 2018.

The company expects that its cash, cash equivalents and marketable securities as of March 31, 2018, together with the anticipated product and royalty revenue, anticipated interest income, and anticipated expense reimbursements, 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.

WEBCAST INFORMATION

The live webcast from today’s event 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 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 an approved oncology precision medicine 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 Agios/Celgene Collaboration

IDHIFA® (enasidenib) and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation focused on cancer metabolism. Under the terms of the 2010 collaboration agreement, 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 $95 million in remaining payments assuming achievement of certain milestones, 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. For AG-881, the companies have a joint worldwide development and 50/50 profit share collaboration, and Agios is eligible to receive regulatory milestone payments of up to $70 million. AG-270 is part of a 2016 global research collaboration agreement with Celgene. Through Phase 1 dose escalation, Celgene has the option, for a fee of at least $30 million, to participate in a worldwide cost and profit share with Agios. Upon exercise of the option the parties will share all development costs, subject to specified exceptions, and any profits on net sales and Agios will be 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®, TIBSOVO® (ivosidenib), AG-881, AG-348, 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 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)

	March 31, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 994,747	$ 567,750
Collaboration receivable – related party	3,512	2,448
Royalty receivable – related party	1,417	1,222
Total assets	1,040,126	614,397
Deferred revenue – related party	121,043	163,640
Stockholders' equity	865,594	375,503

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

	Three Months Ended March

	31,	
	2018	2017
Collaboration revenue – related party	$ 7,345	$ 10,508
Royalty revenue – related party	1,417	—
Total Revenue	8,762	10,508
Operating expenses:		
Research and development, net	78,224	62,732
General and administrative	24,550	14,823
Total operating expenses	102,774	77,555
Loss from operations	(94,012)	(67,047)
Interest income	3,187	881
Net loss	$ (90,825)	$ (66,166)
Net loss per share – basic and diluted	$ (1.63)	$ (1.56)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	55,694,603	42,280,525

[1] National PKU Alliance, www.npkua.org

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