



## AgiOS Reports Second Quarter 2018 Financial Results

August 2, 2018

- TIBSOVO® Launch Underway Post FDA Approval for IDH1m R/R AML; Second Medicine Approved from Agios' Discovery Platform in 12 Months –
- Entered License Agreement with CStone Pharmaceuticals to Develop and Commercialize Ivosidenib in Greater China –
- Mitapivat (AG-348) Pivotal Program (ACTIVATE and ACTIVATE-T) Initiated for Pyruvate Kinase Deficiency –
- Company in Strong Financial Position with Q2 2018 Ending Cash, Cash Equivalents and Marketable Securities of \$937 Million –

CAMBRIDGE, Mass., Aug. 02, 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 second quarter ended June 30, 2018. In addition, Agios highlighted select corporate milestones and clinical data from its development programs.

"The first half of 2018 has been productive across all aspects of our business, culminating in the recent approval and launch of our second internally discovered medicine," said David Schenkein, M.D., chief executive officer at Agios. "This achievement sets us well on the path to becoming a sustainable, multiproduct company with a thriving research engine on track to submit its 7<sup>th</sup> IND and a broad clinical development program with multiple trials planned or underway to expand our oncology and rare genetic disease portfolios."

### SECOND QUARTER 2018 HIGHLIGHTS & RECENT PROGRESS

- Received full approval from the U.S. Food and Drug Administration (FDA) on July 20, 2018 for TIBSOVO® (ivosidenib) for the treatment of patients with relapsed or refractory AML (R/R AML) with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation as detected by an FDA approved test.
- Entered into an exclusive license agreement with CStone Pharmaceuticals to develop and commercialize ivosidenib in Greater China, resulting in a \$12 million upfront payment and the potential for \$412 million in development and commercial milestones.
- Initiated ACTIVATE, a global, placebo-controlled, pivotal trial for mitapivat (AG-348) in approximately 80 adults with PK deficiency who do not receive regular blood transfusions. A second pivotal trial (ACTIVATE-T) in PK deficiency patients who receive regular blood transfusions is ongoing.
- Presented new and updated data from the IDH programs at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago. Links to the data presentations, including updated data from the Phase 1 trial combining ivosidenib and azacitidine in the frontline AML setting can be found [here](#).
- Secured publication of the ivosidenib Phase 1 data in patients with IDH1m advanced hematological malignancies in the *New England Journal of Medicine*.
- Supported publication of the results from the Pyruvate Kinase Deficiency Natural History Study in the journal *Blood*.
- Disclosed active research programs in three rare genetic diseases: phenylketonuria, erythroid porphyria and Friedreich's ataxia, as part of a preclinical pipeline update at the company's Investor Day in May.

### KEY UPCOMING MILESTONES

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

#### Cancer:

- 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 conjunction 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.

#### Rare Genetic Diseases:

- Initiate a Phase 2 proof of concept trial of mitapivat (AG-348) in thalassemia in the fourth quarter of 2018.

#### Research:

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

### EXPECTED FOURTH QUARTER CLINICAL DATA PRESENTATIONS

- 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 has been submitted to the 2018 American Society of Hematology (ASH) Annual Meeting and Exposition on December 1-4 in San Diego.
- Updated data in untreated AML from the ongoing Phase 1 study of ivosidenib in IDH1m hematologic malignancies has been submitted to ASH.
- Updated data in myelodysplastic syndrome (MDS) from the ongoing Phase 1 study of ivosidenib in IDH1m hematologic malignancies has been submitted to ASH.

## SECOND QUARTER 2018 FINANCIAL RESULTS

Revenue for the quarter ended June 30, 2018 was \$40.4 million, which includes \$26.4 million of collaboration revenue and \$1.6 million of royalty revenue from net U.S. sales of IDHIFA<sup>®</sup> under our collaboration agreements with Celgene, and \$12.4 million of collaboration revenue under our agreement with CStone. Revenue for the quarter ended June 30, 2017 was \$11.3 million and consisted solely of collaboration revenue under our agreements with Celgene. The year over year increase in collaboration revenue for the second quarter was primarily driven by the \$15.0 million milestone related to Celgene's filing of an MAA to the EMA for IDHIFA<sup>®</sup> and \$12.4 million related to the delivery of the license under the CStone Agreement.

Research and development (R&D) expenses were \$86.7 million, including \$9.7 million of stock-based compensation expense, for the quarter ended June 30, 2018, compared to \$79.8 million, including \$8.2 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, including the initiation of the ACTIVATE-T trial. R&D expense also increased as a result of IND enabling activities for AG-636, our DHODH inhibitor.

General and administrative (G&A) expenses were \$26.6 million, including \$6.8 million of stock-based compensation expense, for the quarter ended June 30, 2018, compared to \$16.1 million, including \$4.0 million of stock-based compensation expense, for the quarter ended June 30, 2017. The increase in G&A expense was primarily attributable to the growth in our U.S. commercial organization to support the launch of TIBSOVO<sup>®</sup>.

Net loss for the quarter ended June 30, 2018 was \$68.7 million, compared to a net loss of \$83.1 million for the quarter ended June 30, 2017.

Cash, cash equivalents and marketable securities as of June 30, 2018 were \$936.6 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, \$8.9 million of cost reimbursements under our collaboration agreements with Celgene and \$22.0 million received from employee stock transactions. This was offset by expenditures to fund operations of \$178.1 million during the six months ended June 30, 2018.

The company expects that its cash, cash equivalents and marketable securities as of June 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 second 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 1497883. The live webcast can be accessed under "Events & Presentations" in the Investors section of the company's website at [www.agios.com](http://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](http://www.agios.com).

### About TIBSOVO<sup>®</sup> (ivosidenib)

TIBSOVO<sup>®</sup> (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. For more information, visit [TIBSOVO.com](http://TIBSOVO.com).

### About Agios/Celgene Collaboration

IDHIFA<sup>®</sup> (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<sup>®</sup> (enasidenib). Agios continues to conduct certain clinical development activities within the IDHIFA<sup>®</sup> (enasidenib) development program and is eligible to receive reimbursement for those development activities and up to \$95 million in remaining milestone payments, and royalties on any net sales. Celgene and Agios are currently co-commercializing IDHIFA<sup>®</sup> (enasidenib) 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. The program focused on MTAP (methylthioadenosine phosphorylase)-deleted cancers 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-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 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)

	<u>June 30,</u> <u>2018</u>	<u>December 31,</u> <u>2017</u>
Cash, cash equivalents and marketable securities	\$ 936,629	\$ 567,750
Collaboration receivable – related party	19,326	2,448
Royalty receivable – related party	1,573	1,222
Total assets	998,235	614,397
Deferred revenue – related party	113,540	163,640
Stockholders' equity	823,142	375,503

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
Collaboration revenue – related party	\$ 26,401	\$ 11,346	\$ 33,746	\$ 21,854
Collaboration revenue – other	12,440	-	12,440	-
Royalty revenue – related party	1,573	-	2,990	-
Total Revenue	40,414	11,346	49,176	21,854
Operating expenses:				
Research and development, net	86,730	79,816	164,954	142,548
General and administrative	26,633	16,130	51,183	30,953
Total operating expenses	113,363	95,946	216,137	173,501
Loss from operations	(72,949)	(84,600)	(166,961)	(151,647)
Interest income	4,204	1,518	7,391	2,399
Net loss	(68,745)	(83,082)	(159,570)	(149,248)
Net loss per share – basic and diluted	(1.19)	(1.78)	(2.81)	(3.35)

Weighted-average number of common shares used in computing net loss per share – basic and diluted	<u>57,721,786</u>	<u>46,745,760</u>	<u>56,713,795</u>	<u>44,525,478</u>
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Source: Agios Pharmaceuticals, Inc.