



## AgiOS Reports Fourth Quarter and Full Year 2017 Financial Results

February 14, 2018

*– All 2017 Key Milestones Achieved, Including First U.S. Product Approval from Agios' Discovery Platform and NDA Submission for Second Product Candidate, Ivosidenib, for IDH1m R/R AML –*

*– 2018 Priorities Focus on Expanding Clinical and Research Programs to Drive Long-Term Value –*

*– 2017 Year-End Cash, Cash Equivalents and Marketable Securities was \$568M; January Follow-on Offering of \$516M Extends Cash Runway Through at Least the End of 2020 –*

CAMBRIDGE, Mass., Feb. 14, 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 fourth quarter and year ended December 31, 2017. In addition, Agios highlighted select 2018 corporate milestones and data presentations for its clinical development programs.

"2017 was an extraordinary year for Agios with the U.S. approval of IDHIFA<sup>®</sup>, our first internally discovered and developed drug, the NDA submission for our wholly owned medicine ivosidenib and our sixth IND submission since the company's inception," said David Schenkein, M.D., chief executive officer at Agios. "It was a data-rich year where we set the stage for building long-term value across our cancer and rare disease portfolios. Execution in 2018 will be equally critical as we ready our organization for the potential approval and launch of ivosidenib, initiate our pivotal program for AG-348 in pyruvate kinase deficiency and advance our robust discovery portfolio."

### KEY UPCOMING MILESTONES

The company plans to achieve the following key milestones in 2018:

#### Cancer:

- Potential approval and commercialization of ivosidenib in the United States for relapsed/refractory (R/R) acute myeloid leukemia (AML) with an isocitrate dehydrogenase-1 (IDH1) mutation in the third quarter of 2018.
- Potential submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for ivosidenib for IDH1m R/R AML in the fourth quarter of 2018.
- Support, in conjunction with Celgene, the initiation of 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.
- Initiate a perioperative 'window' trial with ivosidenib and AG-881 in low-grade glioma in the first quarter of 2018 to further investigate their effects on brain tumor tissue.
- Initiate 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 in the first quarter of 2018.

#### Rare Genetic Diseases:

- Initiate two global pivotal trials for AG-348 in pyruvate kinase (PK) deficiency in the first half of 2018:
  - ACTIVATE-T: A single arm trial of approximately 20 regularly transfused patients is expected to initiate in the first quarter of 2018.
  - ACTIVATE: A placebo-controlled trial of approximately 80 patients who do not receive regular transfusions is expected to initiate in the second quarter of 2018.
- Initiate a global registry, known as PEAK, for adult and pediatric patients with PK deficiency in the first 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, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH) for the treatment of hematologic malignancies in the fourth quarter of 2018.

### ANTICIPATED KEY 2018 DATA PRESENTATIONS

- Updated data from the expansion phase of the ongoing Phase 1 study of ivosidenib in IDH1m R/R AML has been submitted to the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting.
- Updated data from the ongoing Phase 1/2 combination trial of enasidenib or ivosidenib with VIDAZA<sup>®</sup> in patients with newly diagnosed AML with an IDH2 or IDH1 mutation ineligible for intensive chemotherapy has been submitted to ASCO.

- First clinical data from the Phase 1 study of AG-881 in advanced IDHm positive solid tumors, including glioma, has been submitted to ASCO.
- 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 be submitted to the 2018 American Society of Hematology (ASH) Annual Meeting and Exposition.

#### **FOURTH QUARTER 2017 HIGHLIGHTS & RECENT PROGRESS**

- Completed an underwritten public offering in January of 8,152,986 shares of common stock at the offering price of \$67.00 per share, resulting in proceeds, net of underwriting discounts and commissions, of approximately \$516.2 million.
- Submitted a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for ivosidenib for the treatment of patients with R/R AML with an IDH1 mutation.
- Received FDA clearance of an IND application for AG-270, a MAT2A inhibitor, for the treatment of MTAP-deleted tumors.
- Presented new and updated data from the IDH and PKR programs at the 2017 American Society of Hematology Annual Meeting and Exposition (ASH):
  - First data from the expansion phase of the ongoing Phase 1 trial of ivosidenib in IDH1m R/R AML and advanced hematologic malignancies
  - First 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 IDH1 or IDH2 mutation
  - First data from the ongoing Phase 1/2 combination trial of ivosidenib or enasidenib with VIDAZA® in patients with newly diagnosed AML with an IDH1 or IDH2 mutation ineligible for intensive chemotherapy
  - Updated data from the AG-348 Phase 2 DRIVE PK study in PK deficiency
- Appointed Jacquelyn “Jackie” Fouse, Ph.D., former president and chief financial officer of Celgene, to Agios’ board of directors.
- Presented updated data from the glioma expansion cohort of the ongoing Phase 1 trial of ivosidenib in advanced IDH1m positive solid tumors at the 2017 Society for NeuroOncology Annual Meeting.

#### **FULL YEAR 2017 FINANCIAL RESULTS**

Cash, cash equivalents and marketable securities as of December 31, 2017 were \$567.8 million, compared to \$573.6 million as of December 31, 2016. The decrease in cash was driven by expenditures to fund operations of \$306.8 million during the year ended December 31, 2017. These expenditures were offset by an increase in cash driven by net proceeds of \$270.2 million from the April follow on offering, \$17.0 million of cost reimbursements under our collaboration agreements with Celgene and \$14.2 million received from employee stock transactions.

Revenue for the year ended December 31, 2017 was \$43.0 million, which includes \$41.1 million of collaboration revenue and \$1.9 million of royalty revenue from net sales of IDHIFA®. Revenue for the year ended December 31, 2016 was \$69.9 million, which included a \$25.0 million milestone payment related to the initiation of the Phase 3 IDHENTIFY trial with IDHIFA® under the 2010 Agreement.

Research and development (R&D) expenses were \$292.7 million, including \$30.8 million of stock-based compensation expense, for the year ended December 31, 2017, compared to \$220.2 million, including \$25.4 million in stock-based compensation expense, for the year ended December 31, 2016. The increase in R&D expense was primarily attributable to the ivosidenib program, including manufacturing and regulatory activities to prepare the NDA submission, start-up costs for the Phase 3 AGILE clinical trial, and on-going site activation and patient enrollment of the Phase 3 ClarIDHy clinical trial. R&D expense also increased compared to the prior year due to IND enabling activities for AG-270.

General and administrative (G&A) expenses were \$71.1 million, including \$17.0 million of stock-based compensation expense, for the year ended December 31, 2017, compared to \$50.7 million, including \$16.7 million of stock-based compensation expense, for the year ended December 31, 2016. The increase in G&A expense was primarily attributable to an increase of \$21.1 million to support our growing commercial organization for the launch of IDHIFA® and the potential launch of ivosidenib in 2018.

Net loss for the year ended December 31, 2017 was \$314.7 million, compared to a net loss of \$198.5 million for the year ended December 31, 2016.

#### **CASH GUIDANCE**

In January, Agios completed an underwritten public offering of 8,152,986 shares of common stock, which includes the full exercise of the underwriters’ option to purchase an additional 1,063,433 shares, at the offering price of \$67.00 per share, resulting in proceeds, net of underwriting discounts and commissions, of approximately \$516.2 million.

The company expects that its cash, cash equivalents and marketable securities as of December 31, 2017, together with the net proceeds from the recent financing, 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.

#### **CONFERENCE CALL INFORMATION**

Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss fourth quarter and full year 2017 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 3198522. 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 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](http://www.agios.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 payments assuming achievement of certain milestones, 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<sup>®</sup>, ivosidenib, AG-881, AG-348 and AG-270; 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," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope," "strategy," "milestone," "will," 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)

|                                                  | December 31, 2017 | December 31, 2016 |
|--------------------------------------------------|-------------------|-------------------|
| Cash, cash equivalents and marketable securities | \$ 567,750        | \$ 573,564        |
| Collaboration receivable – related party         | 2,448             | 4,886             |
| Royalty receivable – related party               | 1,222             | —                 |
| Total assets                                     | 614,397           | 619,094           |
| Deferred revenue – related party                 | 163,640           | 190,210           |
| Stockholders' equity                             | 375,503           | 358,591           |

## Consolidated Statements of Operations Data

(in thousands, except share and per share data)

(Unaudited)

|                                       | Three Months Ended<br>December 31, |           | Years<br>Ended December 31, |           |
|---------------------------------------|------------------------------------|-----------|-----------------------------|-----------|
|                                       | 2017                               | 2016      | 2017                        | 2016      |
| Collaboration revenue – related party | \$ 8,577                           | \$ 22,648 | \$ 41,074                   | \$ 69,892 |
| Royalty revenue – related party       | 1,222                              | —         | 1,937                       | —         |

|                                                                                                   |             |             |              |              |
|---------------------------------------------------------------------------------------------------|-------------|-------------|--------------|--------------|
| Total Revenue                                                                                     | 9,799       | 22,648      | 43,011       | 69,892       |
| Operating expenses:                                                                               |             |             |              |              |
| Research and development, net                                                                     | 77,216      | 64,678      | 292,681      | 220,163      |
| General and administrative                                                                        | 22,713      | 15,379      | 71,124       | 50,714       |
| Total operating expenses                                                                          | 99,929      | 80,057      | 363,805      | 270,877      |
| Loss from operations                                                                              | (90,130)    | (57,409)    | (320,794)    | (200,985)    |
| Interest income                                                                                   | 1,845       | 923         | 6,124        | 2,514        |
| Net loss                                                                                          | \$ (88,285) | \$ (56,486) | \$ (314,670) | \$ (198,471) |
| Net loss per share – basic and diluted                                                            | \$ (1.81)   | \$ (1.34)   | \$ (6.75)    | \$ (5.07)    |
| Weighted-average number of common shares used in computing net loss per share – basic and diluted | 48,772,901  | 42,110,541  | 46,587,631   | 39,126,400   |

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Source: Agios Pharmaceuticals, Inc.