Agios Announces “Agios 2025” Strategic Vision and Highlights 2020 Milestones

January 12, 2020

– In 2025, Company Expects to Have 4 Marketed Products in at Least 8 Indications, at Least 6 Molecules in Clinical Development and a Broad Research Pipeline –

– Broadening Opportunities for PKR Activation: Topline Data from Pivotal Studies of Mitapivat in PK Deficiency Expected by Year-end 2020 and Clinical Data for PKR Activation in Thalassemia and Sickle Cell Disease Expected Mid-Year 2020 –

– Expanding Utility of IDH Inhibition: Registration-enabling Phase 1 Expansion Underway for TIBSOVO® in MDS; Phase 3 INDIGO Study of Vorasidenib in Low-Grade Glioma Enrolling; TIBSOVO® sNDA Filing for Cholangiocarcinoma Expected by Year-end 2020 –

– Financial Highlights Include 2020 TIBSOVO® Net U.S. Revenue Guidance of $105–115 Million and Cash Runway Through at Least the End of 2021 –

SAN FRANCISCO, Jan. 12, 2020 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO) today announced its “Agios 2025” six-year strategic vision focused on creating and commercializing differentiated medicines to treat hematologic malignancies, solid tumors and rare genetic diseases. Under this plan, by the end of 2025, the company expects to have four marketed products across at least eight indications, at least six molecules in clinical development and be cash-flow positive. Agios will present at the 38th Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 13 at 7:30 a.m. PT (10:30 a.m. ET), and a live webcast will be available at investor.agios.com.

“We are entering an exciting new chapter for Agios as we advance our first rare genetic disease program across three opportunities in PK deficiency, thalassemia and sickle cell disease and continue our work to expand the benefit of IDH inhibitors to solid tumors as well as to additional indications in hematologic malignancies,” said Jackie Fouse, Ph.D., chief executive officer of Agios. “We will realize our 2025 vision by continuing to leverage our unmatched expertise in cellular metabolism, early translational research and our passionately patient-focused team, the same attributes that enabled us to discover, develop and market two targeted oncology medications in just 10 years. With both near- and long-term value drivers, Agios enters 2020 with strong momentum that will continue as we work toward achieving our 2025 strategic vision.”

“AGIOS 2025” STRATEGIC VISION

The “Agios 2025” strategic vision delineates the company’s view for growth over the next six years 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

ANTICIPATED 2020 KEY MILESTONES

Agios announced today that it expects to achieve the following key milestones in 2020:

Hematologic Malignancies

- Deliver full-year U.S. revenue for TIBSOVO® of $105-115 million
- Receive European Medicines Agency CHMP opinion for TIBSOVO® in relapsed or refractory acute myeloid leukemia (AML) with an IDH1 mutation by year-end
- Complete enrollment of Phase 3 AGILE trial of TIBSOVO® in combination with azacitidine in adult patients with previously untreated IDH1 mutant AML by year-end
- Complete enrollment of the relapsed or refractory myelodysplastic syndrome arm of the TIBSOVO® Phase 1 study of IDH1 mutant advanced hematologic malignancies by year-end

Solid Tumors

- File supplemental new drug application (sNDA) for TIBSOVO® in previously treated IDH1 mutant cholangiocarcinoma by year-end

Rare Genetic Diseases

- Announce topline data for ACTIVATE and ACTIVATE-T pivotal trials for mitapivat in adults with pyruvate kinase (PK) deficiency by year-end
- Submit updated data from the Phase 2 study of mitapivat in thalassemia for presentation at the European Hematology Association (EHA) Congress and finalize pivotal development strategy by year-end

- Achieve proof of concept for mitapivat in sickle cell disease by mid-2020
- Receive investigational new drug (IND) clearance for AG-946, a next generation PKR activator, and initiate first-in-human study in healthy volunteers in the first half of 2020

Research

- Achieve at least one new development candidate by year-end

RECENT MILESTONES

The company also provided an update on the following 2019 key milestones:

- Completed enrollment of ACTIVATE-T, a single-arm trial evaluating mitapivat in regularly transfused adults with PK deficiency
- Expect to complete enrollment in ACTIVATE, a 1:1 randomized, placebo-controlled trial in adult PK deficiency patients who do not receive regular transfusions, in the first quarter of 2020
- Initiated the registration-enabling Phase 3 INDIGO study of vorasidenib in patients with Grade 2 non-enhancing glioma with an IDH mutation

2019 Year-End Cash and Guidance
Agios ended 2019 with approximately $718 million of cash, cash equivalents and marketable securities. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2019, together with anticipated product and royalty revenue, interest income and expense reimbursements under our collaboration agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its planned operating expenses and capital expenditure requirements through at least the end of 2021.

Presentation at 38th Annual J.P. Morgan Healthcare Conference
Agios will webcast its corporate presentation and break out session from the 38th Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 13, 2020 at 7:30 a.m. PT (10:30 a.m. ET). A live webcast of the presentation can be accessed under "Events & Presentations" in the Investors section of the company's website at agios.com. A replay of the webcast will be archived on the Agios website for at least two weeks following the presentation.

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), IDHIFA® (enasidenib), mitapivat, vorasidenib, AG-270 and AG-636; the potential benefits of Agios' product candidates; Agios's strategic vision and goals for 2025; its key milestones for 2020; its estimates regarding its balance of cash, cash equivalents and marketable securities for the year ended December 31, 2019; 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," "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: 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.

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Source: Agios Pharmaceuticals, Inc.