# Agios Reports Business Highlights and First Quarter 2022 Financial Results

May 5, 2022

*– Received U.S. FDA Approval for PYRUKYND®, the First Therapy for the Treatment of Hemolytic Anemia in Adults with Pyruvate Kinase (PK) deficiency and Agios' First Genetically Defined Disease Medicine; U.S. Launch Underway –*

*– PYRUKYND® Pivotal Studies in Thalassemia and Sickle Cell Disease Open and Enrolling; Start-Up Activities Ongoing for Pediatric PK Deficiency Program –*

*– Completed Healthy Volunteer Cohorts and Initiated Sickle Cell Disease Cohort of AG-946 Phase 1 Study; On Track to Initiate Phase 2a Study in Myelodysplastic Syndrome by Year-End –*

*– Cash, Cash Equivalents and Marketable Securities $1.2 Billion as of March 31, 2022 –*

CAMBRIDGE, Mass., May 05, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism pioneering therapies for genetically defined diseases, today reported business highlights and financial results for the first quarter ended March 31, 2022.

"The U.S. approval of PYRUKYND® in adults with PK deficiency was Agios' first step toward changing treatment paradigms for people with genetically defined diseases, starting with developing the first disease-modifying therapy for a rare, debilitating, lifelong hemolytic anemia," said Jackie Fouse, Ph.D., chief executive officer at Agios. "We are now poised to expand our impact for many more patients, including adults with PK deficiency in the EU, pediatric PK deficiency patients and people living with thalassemia, sickle cell disease and low- to intermediate-risk myelodysplastic syndrome. I am tremendously proud of our team's incredible work on the U.S. launch of PYRUKYND®, ongoing regulatory interactions in the EU, five pivotal clinical trials planned or underway, the expansion of our PK activation portfolio with AG-946 and our innovative research engine. We continue to execute in each of these arenas, motivated and inspired by our connections to patients."

**First Quarter 2022 & Recent Highlights**

- Received approval from the U.S. Food and Drug Administration (FDA) for PYRUKYND®, the first therapy for the treatment of hemolytic anemia in adults with PK deficiency and Agios' first genetically defined disease medicine.
- Executed commercial launch of PYRUKYND® and generated approximately $0.8 million in net U.S. revenue for the first partial quarter following launch.
- Published results from the ACTIVATE Phase 3 clinical study evaluating PYRUKYND® in adults with PK deficiency who do not receive regular transfusions in the *New England Journal of Medicine*.
- Completed the single ascending dose and multiple ascending dose healthy volunteer cohorts of the Phase 1 study of novel PK activator AG-946 and identified doses for the Phase 1 sickle cell disease cohort and Phase 2a study in low- to intermediate risk myelodysplastic syndrome (MDS).
- Initiated the sickle cell disease cohort of the Phase 1 study of AG-946.
- Published 2022 Environmental, Social and Governance (ESG) Report disclosing ESG initiatives and metrics aligned with the United Nations Sustainable Development Goals (UN SDGs) and the standards for the Biotechnology and Pharmaceuticals industry set by the Sustainability Accounting Standards Board (SASB).

**Key Upcoming Milestones & Priorities**

Agios expects to execute on the following key milestones and priorities in 2022:

- **Adult PK Deficiency:** Receive European Medicines Agency (EMA) regulatory decision for PYRUKYND® in adults with PK deficiency by year-end.
- **Pediatric PK Deficiency:** Initiate Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® in not regularly transfused and regularly transfused pediatric patients with PK deficiency, respectively, in mid-2022.
- **Thalassemia:** Enroll a meaningful portion of patients in the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® in not regularly transfused and regularly transfused adults with thalassemia, respectively, by year-end.
- **Sickle Cell Disease:** Complete enrollment in the Phase 2 portion of the RISE UP study of PYRUKYND® in sickle cell disease by year-end.
- **Myelodysplastic Syndrome:** Initiate Phase 2a study of AG-946 in adults with low- to intermediate-risk MDS by year-end.

*Data Presentations*

- Submitted new clinical and translational data to the European Hematology Association (EHA) congress, to be held June 9-12 in Vienna and virtually, including:
    - New patient-reported outcomes (PRO) data from ACTIVATE Phase 3 study of PYRUKYND®

- Data demonstrating the normalization of hemoglobin levels with long-term treatment of PYRUKYND® in adults with PK deficiency
- Additional PK deficiency comorbidities and complications data from the PEAK registry

- Continue to publish clinical and translational data supporting the utility of PK activators across key disease areas and elucidating the burden of disease for PK deficiency, thalassemia and sickle cell disease.

**First Quarter 2022 Financial Results**

The financial results discussion compares Agios' continuing operations. All periods have been adjusted to exclude discontinued operations related to the divested oncology business.

*Revenue:* Net U.S. product revenue from sales of PYRUKYND® for the first quarter of 2022 was $0.8 million. This revenue reflects the first partial quarter of PYRUKYND® launch, following FDA approval on February 17, 2022.

*Cost of Sales*: Cost of sales for the first quarter of 2022 were $0.3 million.

*Non-Operating Income:* Non-operating income included approximately $2.7 million from TIBSOVO® (ivosidenib) royalties for the first quarter of 2022.

*Research and Development (R&D) Expenses:* R&D expenses were $70.1 million for the first quarter of 2022 compared to $57.7 million for the first quarter of 2021. The year-over-year increase in R&D was driven primarily by start-up costs for the PYRUKYND® pivotal studies in thalassemia and sickle cell disease and planned increases in research activities, offset by closeouts of the ACTIVATE and ACTIVATE-T studies.

*Selling, General and Administrative (SG&A) Expenses:* SG&A expenses were $31.5 million for the first quarter of 2022 compared to $33.6 million for the first quarter of 2021. The year-over-year decrease in SG&A expenses was primarily attributable to lower workforce expenses.

*Net Loss from Continuing Operations:* Net loss from continuing operations was $94.8 million for the first quarter of 2022 compared to a net loss of $90.9 million for the first quarter of 2021.

*Cash Position and Guidance:* Cash, cash equivalents and marketable securities as of March 31, 2022, were $1.2 billion compared to $2.4 billion as of March 31, 2021. The year-over-year decrease is attributable to operating expenses and 16.2 million shares of common stock that the company repurchased for $802.5 million during the second through fourth quarters of 2021. Agios expects that its cash, cash equivalents and marketable securities will enable the company to execute its operating plan through major catalysts and to cash-flow positivity without the need to raise additional equity.

**Conference Call Information**
Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss first quarter 2022 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 refer to conference ID 5738266. 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 a biopharmaceutical company that is fueled by connections. The Agios team cultivates strong bonds with patient communities, healthcare professionals, partners and colleagues to discover, develop and deliver therapies for genetically defined diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational medicines with active and planned programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has multiple investigational therapies in preclinical development and an industry-leading research team with unmatched expertise in cellular metabolism and genetics. 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 the preclinical, clinical and commercial advancement of its drug development programs, including PYRUKYND® (mitapivat) and AG-946; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2022; its financial guidance regarding the period in which it will have capital available to fund its operations; and the potential benefits of Agios' 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. 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, without limitation risks and uncertainties related to: the failure of Agios to receive milestone or royalty payments related to the sale of its oncology business, the uncertainty of the timing of any receipt of any such payments, and the uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; the impact of the COVID-19 pandemic on Agios' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of future approved products, and launching, marketing and selling future approved products; 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, including with respect to the regulatory submissions for PYRUKYND® (mitapivat), 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 and 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 establish and maintain 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, or SEC, including the risks and uncertainties set forth under the heading Risk Factors in our filings with the SEC. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this press release are made only as of

the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

**Consolidated Balance Sheet Data**
**(in thousands)**
**(Unaudited)**

| | March 31, 2022 | December 31, 2021 |
|---|---|---|
| Cash, cash equivalents, and marketable securities | 1,179,365 | 1,286,393 |
| Accounts receivable, net | 540 | - |
| Inventory | 2,485 | - |
| Total assets | 1,335,859 | 1,437,736 |
| Stockholders' equity | 1,207,453 | 1,291,975 |

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2022 | 2021 |
| Product revenue, net | $ 832 | $ - |
| Total revenue | 832 | - |
| Cost and expenses: | | |
| Cost of sales | 339 | - |
| Research and development | 70,123 | 57,667 |
| Selling, general and administrative | 31,515 | 33,550 |
| Total cost and expenses | 101,977 | 91,217 |
| Loss from operations | (101,145) | (91,217) |
| Royalty income from gain on sale of oncology business | 2,704 | - |
| Interest income, net | 694 | 340 |
| Other income, net | 2,973 | - |
| Net loss from continuing operations | (94,774) | (90,877) |
| Net income from discontinued operations, net of tax | - | 1,965,202 |
| Net (loss) income | $ (94,774) | $ 1,874,325 |
| Net loss from continuing operations per share - basic and diluted | $ (1.74) | $ (1.31) |
| Net income from discontinued operations per share - basic and diluted | - | 28.26 |
| Net (loss) income per share - basic and diluted | (1.74) | 26.95 |
| Weighted-average number of common shares used in computing net loss per share from continuing operations, net income from discontinued operations and net (loss) income per share – basic and diluted | 54,555,467 | 69,543,510 |

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