Agios Reports Business Highlights and Second Quarter 2022 Financial Results

August 4, 2022

– Generated $3.1 Million in Net U.S. Revenue for First Full Quarter of PYRUKYND® (mitapivat) Sales –

– Brian Goff to Become Chief Executive Officer at Agios on August 8, 2022, Leveraging 30+ Years of Biopharma Experience, Including Significant Expertise in Rare Disease and Hematology; Current CEO Jackie Fouse to Become Chair of the Board –

– Actively Enrolling Patients in Five PYRUKYND® Pivotal Studies Across Thalassemia, Sickle Cell Disease and Pediatric Pyruvate Kinase (PK) Deficiency –

– $1.1 Billion of Cash, Cash Equivalents and Marketable Securities as of June 30, 2022 –

CAMBRIDGE, Mass., Aug. 04, 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 second quarter ended June 30, 2022.

“As we look ahead to the next chapter for the company, Agios is operating from a position of strength. We are executing our commercial launch of PYRUKYND®, the first therapy for a rare, debilitating, lifelong blood disorder; we also have five pivotal trials underway, multiple early-stage studies planned or ongoing, a promising preclinical pipeline and a strong balance sheet providing optionality for the future growth of the business,” said Jackie Fouse, Ph.D., chief executive officer at Agios. “As I reflect on my legacy as CEO of Agios, I am proud of the bold and strategic decisions we have made to maximize and accelerate our impact for people with genetically defined diseases. I am tremendously grateful to the Agios team for their unwavering resiliency in the face of unprecedented challenges and heartfelt dedication to patients and each other. I look forward to my new role at Agios as board chair and to supporting Brian as he leverages his expertise to expand Agios’ genetically defined disease capabilities, foster the company’s differentiated culture and drive our next phase of impact.”

Second Quarter 2022 & Recent Highlights

- Continued to execute U.S. launch of PYRUKYND®, generating $3.1 million in U.S. net revenue for the second quarter of 2022, the first full quarter following FDA approval.
- Initiated Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® in pediatric patients with PK deficiency who are not regularly transfused and who are regularly transfused, respectively.
- Announced that effective August 8, 2022, Dr. Fouse will transition to the role of chair of Agios’ board of directors and Brian Goff will assume the role of Agios’ chief executive officer and member of the board of directors.
- Evolved Agios’ research approach to focus on advancing the company’s existing validated preclinical programs and in-licensing or acquiring well-characterized, high-potential assets.
- Expanded role of Sarah Gheuens, M.D., Ph.D., to chief medical officer and head of research and development, incorporating research and discovery sciences in addition to her existing chief medical officer responsibilities.
- Presented clinical and translational data at the 2022 European Hematology Association (EHA) Congress, including new data supporting the potential benefits of PYRUKYND® treatment in adults with PK deficiency.

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.
- **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:** 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.

Second 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 second quarter of 2022 was $3.1 million. This revenue reflects the first full quarter of PYRUKYND® launch, following FDA approval on February 17, 2022. In addition, Agios recognized revenue of $2.5 million dollars in the

second quarter of 2022 as an up-front payment associated with the licensing of intellectual property for the company's Friedreich's Ataxia preclinical program.

Cost of Sales: Cost of sales for the second quarter of 2022 was $0.4 million.

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

Research and Development (R&D) Expenses: R&D expenses were $74.5 million for the second quarter of 2022 compared to $62.0 million for the second quarter of 2021. The year-over-year increase in R&D was driven primarily by increased headcount and workforce-related expenses, planned increased activity associated with the PAH preclinical program, start-up costs for the AG-946 Phase 2a MDS study, increased spend for the AG-946 Phase 1 trial, and start-up costs for the PYRUKYND pivotal studies in sickle cell disease and pediatric PK deficiency.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $28.3 million for the second quarter of 2022 compared to $29.2 million for the second quarter of 2021. The year-over-year decrease in SG&A expenses was primarily attributable to the completion of the reimbursable transition services Agios provided to Servier, which concluded in the first quarter of 2022, related to the sale of the oncology business.

Net Loss from Continuing Operations: Net loss from continuing operations was $91.8 million for the second quarter of 2022 compared to a net loss of $82.8 million for the second quarter of 2021.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of June 30, 2022, were $1.1 billion compared to $1.7 billion as of June 30, 2021. The year-over-year decrease is attributable to operating expenses and 5.7 million shares of common stock that the company repurchased for $273.4 million during the third and 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 second quarter 2022 financial results and recent business activities. 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 expected benefits of Agios' chief executive officer succession plan; 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 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; 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; 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)

	June 30, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$1,100,154	$1,286,393

Accounts receivable, net	1,598	—
Inventory	4,060	—
Total assets	1,252,467	1,437,736
Stockholders' equity	1,124,070	1,291,975

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
Revenues:				
Product revenue, net	$3,082	$ —	$3,914	$ —
Milestone revenue	2,500	—	2,500	—
Total revenue	5,582	—	6,414	—
Cost and expenses:				
Cost of sales	$435	$ —	$774	$ —
Research and development	74,523	62,007	144,646	119,674
Selling, general and administrative	28,264	29,215	59,779	62,765
Total cost and expenses	103,222	91,222	205,199	182,439
Loss from operations	(97,640)	(91,222)	(198,785)	(182,439)
Royalty income from gain on sale of oncology business	2,704	2,000	5,408	2,000
Interest income (expense), net	1,793	(92)	2,487	248
Other income, net	1,337	6,524	4,310	6,524
Net loss from continuing operations	(91,806)	(82,790)	(186,580)	(173,667)
Net (loss) income from discontinued operations, net of tax	—	(3,427)	—	1,961,775
Net (loss) income	$(91,806)	$(86,217)	$(186,580)	$1,788,108
Net loss from continuing operations per share - basic and diluted	$(1.68)	$(1.36)	$(3.41)	$(2.66)
Net (loss) income from discontinued operations per share - basic and diluted	$ —	$(0.06)	$ —	$30.05
Net (loss) income per share - basic and diluted	$(1.68)	$(1.41)	$(3.41)	$27.39
Weighted-average number of common shares used in computing net loss per share from continuing operations, net (loss) income from discontinued operations and net (loss) income per share – basic and diluted	54,799,680	61,066,977	54,678,249	65,281,827

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