Agios Reports Business Highlights and Third Quarter 2022 Financial Results

November 3, 2022

– U.S. PYRUKYND® (mitapivat) Launch Provides Capability Building Platform to Support Expected Future Product Growth and Expansion; Net Revenue of $3.5 Million in Q3 –

– Received Positive CHMP Opinion for PYRUKYND® in Adults with Pyruvate Kinase (PK) Deficiency –

– On Track to Meet Year-end Enrollment Targets in PYRUKYND® Thalassemia and Sickle Cell Disease Pivotal Programs; First PK Activator Trials in Pediatric PK Deficiency and Myelodysplastic Syndromes (MDS) Continue to Enroll –

– Strengthened Company Leadership with Appointments of Cecilia Jones as Chief Financial Officer and Rahul Ballal and Cynthia Smith as Board Members –

– $1.0 Billion of Cash, Cash Equivalents and Marketable Securities as of September 30, 2022; Subsequently Received $131.8 Million from Sale of TIBSOVO® (ivosidenib tablets) Royalties –

CAMBRIDGE, Mass., Nov. 03, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare and genetically defined diseases, today reported business highlights and financial results for the third quarter ended September 30, 2022.

“Agios is on the cusp of changing the treatment landscape for people with rare and genetically defined diseases, with the potential of PYRUKYND ® to make a positive impact across multiple underserved diseases. I joined the company in August because of its differentiated portfolio, top-notch team and genuine dedication to patients, and my excitement for the future of Agios has only grown over the past few months,” said Brian Goff, chief executive officer at Agios. “We are continuing to build our capabilities and connections in rare and genetically defined diseases through our U.S. launch of PYRUKYND®, and we expect our learnings to support anticipated future expansion in related diseases where development efforts are ongoing. We will build on the tremendous accomplishments we’ve achieved in 2022 as we close out the year with a focus on continuing to execute the launch, enrolling our thalassemia and sickle cell disease pivotal trials and securing the approval of PYRUKYND® for PK deficiency in the EU and Great Britain. I am honored to lead this team as we together drive long-term growth and value for patients, shareholders and all our stakeholders.”

Third Quarter 2022 & Recent Highlights

- Continued to execute U.S. launch of PYRUKYND®, generating $3.5 million in U.S. net revenue for the third quarter of 2022, the second full quarter following FDA approval. A total of 84 unique patients have completed prescription enrollment forms, representing an increase of 64 percent over the second quarter. A total of 56 patients are on PYRUKYND® therapy, representing a 51 percent increase over the second quarter. Increased patient demand was partially offset by modest inventory build in the prior quarters of launch.
- Received positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), recommending the granting of a marketing authorization for PYRUKYND® for the treatment of PK deficiency in adult patients.
- Completed Medicines and Healthcare Products Regulatory Agency (MHRA) filing for the approval of PYRUKYND® as a treatment for adults with PK deficiency in Great Britain.
- Continued to enroll patients across five PYRUKYND® pivotal studies in thalassemia, sickle cell disease and pediatric PK deficiency.
- Initiated Phase 2a study of novel PK activator AG-946 in adults with lower-risk MDS.
- Published PYRUKYND® data in top-tier medical journals, including Phase 2 thalassemia data in *The Lancet* and Phase 3 ACTIVATE-T data in *The Lancet Haematology*.
- Appointed Cecilia Jones as Agios’ chief financial officer, effective Sept. 26, 2022.
- Appointed Rahul Ballal, Ph.D., chief executive officer of Imara, and Cynthia Smith, former chief commercial officer of ZS Pharma, to Agios’ board of directors.
- Completed the sale of royalty rights on U.S. net sales of Servier’s TIBSOVO ® to Sagard Healthcare Partners for a one-time payment of $131.8 million.

Key Upcoming Milestones & Priorities

Agios expects to execute on the following key milestones and priorities by the end of 2022:

- **Adult PK Deficiency:** Receive EU and Great Britain regulatory decisions for PYRUKYND® in adults with PK deficiency.
- **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.

- **Sickle Cell Disease:** Complete enrollment in the Phase 2 portion of the RISE UP study of PYRUKYND® in adults with sickle cell disease.
- **Data Presentations:** Present broad set of clinical and translational data at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition; abstracts will be available at 9 a.m. ET today.

Third 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 third quarter of 2022 was $3.5 million. This revenue reflects the second full quarter of PYRUKYND® launch, following FDA approval on February 17, 2022.

Cost of Sales: Cost of sales for the third quarter of 2022 was $0.5 million.

Non-Operating Income: Non-operating income included approximately $4.4 million from TIBSOVO® royalties for the third quarter of 2022. TIBSOVO® royalty income will cease in 2022 due to the sale of these royalty rights to Sagard Healthcare Partners.

Research and Development (R&D) Expenses: R&D expenses were $65.0 million for the third quarter of 2022 compared to $64.0 million for the third quarter of 2021.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $29.1 million for the third quarter of 2022 compared to $27.2 million for the third quarter of 2021. The year-over-year increase in SG&A expenses was primarily attributable to an increase in workforce-related expenses.

Net Loss from Continuing Operations: Net loss from continuing operations was $81.7 million for the third quarter of 2022 compared to a net loss of $84.3 million for the third quarter of 2021.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of September 30, 2022, were $1.0 billion compared to $1.4 billion as of September 30, 2021. This cash position does not include the receipt of a one-time payment of $131.8 million associated with the sale of royalty rights on U.S. net sales of Servier's TIBSOVO ®. 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 third 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 rare and 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 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 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)

	September 30, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 1,026,032	$ 1,286,393
Accounts receivable, net	1,818	—
Inventory	5,176	—
Total assets	1,180,320	1,437,736
Stockholders' equity	1,050,170	1,291,975

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Revenues:				
Product revenue, net	$ 3,516	$ —	$ 7,430	$ —
Milestone revenue	—	—	2,500	—
Total revenue	3,516	—	9,930	—
Cost and expenses:				
Cost of sales	$ 517	$ —	$ 1,291	$ —
Research and development	64,966	64,000	209,612	183,674
Selling, general and administrative	29,123	27,152	88,902	89,917
Total cost and expenses	94,606	91,152	299,805	273,591
Loss from operations	(91,090)	(91,152)	(289,875)	(273,591)
Royalty income from gain on sale of oncology business	4,443	1,996	9,851	3,996
Interest income, net	3,818	256	6,305	504
Other income, net	1,082	4,641	5,392	11,165
Net loss from continuing operations	(81,747)	(84,259)	(268,327)	(257,926)
Net (loss) income from discontinued operations, net of tax	—	(4,507)	—	1,957,268
Net (loss) income	$ (81,747)	$ (88,766)	$ (268,327)	$ 1,699,342
Net loss from continuing operations per share - basic and diluted	$ (1.49)	$ (1.48)	$ (4.90)	$ (4.13)
Net (loss) income from discontinued operations per share - basic and diluted	$ —	$ (0.08)	$ —	$ 31.31
Net (loss) income per share - basic and diluted	$ (1.49)	$ (1.56)	$ (4.90)	$ 27.19
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	54,844,579	57,048,175	54,734,301	62,503,087

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