Agios Reports Business Highlights and Third Quarter 2023 Financial Results

November 2, 2023

– First Patient Dosed in Phase 3 Portion of the RISE UP Pivotal Study of Mitapivat in Sickle Cell Disease –

– Completed Enrollment in Phase 3 ACTIVATE-KidsT Pediatric Study of Mitapivat in PK Deficiency; ACTIVATE-Kids Study Achieves >50% Enrollment –

– On Track for Data Readouts in Two Phase 3 Trials of Mitapivat in Thalassemia Next Year and Topline Data for AG-946 in LR-MDS by Year-end 2023 –

– U.S. PYRUKYND® (mitapivat) Net Revenue of $7.4 Million in Q3; Cash, Cash Equivalents and Marketable Securities of $872.4 Million as of September 30, 2023 –

CAMBRIDGE, Mass., Nov. 02, 2023 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today reported business highlights and financial results for the third quarter ended September 30, 2023.

“Agios is approaching a catalyst-rich period, with three mid-to-late-stage data readouts expected by the end of next year, and a total of six by the end of 2025,” said Brian Goff, chief executive officer at Agios. “We are excited to report dosing of the first patient in the Phase 3 portion of the pivotal RISE UP study of our leading PK activator, mitapivat, in sickle cell disease and look forward to sharing more detailed data from the positive Phase 2 portion of RISE UP at an upcoming medical meeting. We look forward to future data readouts across our industry-leading pipeline of PK activators, including the Phase 2a study of AG-946 in lower-risk MDS by the end of this year and both Phase 3 studies of mitapivat in thalassemia next year.”

Third Quarter 2023 & Recent Highlights

- *PYRUKYND® U.S. Launch:* Generated $7.4 million in U.S. net revenue for the third quarter of 2023, a 10 percent increase over the second quarter of 2023. A total of 160 unique patients have completed prescription enrollment forms, representing an increase of 9 percent over the second quarter of 2023. A total of 100 patients are on PYRUKYND® therapy.
- *Sickle Cell Disease:* Dosed first patient in the Phase 3 portion of the RISE UP pivotal study of mitapivat.
- *Pediatric PK Deficiency:* Completed enrollment in the Phase 3 ACTIVATE-kidsT study of mitapivat in regularly transfused pediatric patients with PK deficiency. Achieved goal of >50% enrollment in Phase 3 ACTIVATE-kids study.

Key Upcoming Milestones & Priorities

Agios expects to execute on the following additional key milestones and priorities in the coming months:

- **Sickle Cell Disease:** Present data from the positive Phase 2 portion of the RISE UP study of mitapivat at an upcoming medical meeting. Advance patient enrollment in the Phase 3 portion of RISE UP.
- **Lower-risk Myelodysplastic Syndromes (LR-MDS):** Announce topline data from the Phase 2a study of novel PK activator AG-946 by year-end 2023.
- **Thalassemia:** Announce topline data from the two Phase 3 studies of mitapivat in non-transfusion-dependent and transfusion-dependent thalassemia in the first and second halves of 2024, respectively.
- **Pediatric PK Deficiency:** Complete enrollment in the Phase 3 ACTIVATE-kids study of mitapivat in non-regularly transfused pediatric PK deficiency next year.
- **Pipeline:** File investigational new drug (IND) application for phenylalanine hydroxylase (PAH) stabilizer for the treatment of phenylketonuria (PKU) by year-end 2023.
- **Data Presentations:** Present broad set of clinical and translational data at the 65th American Society of Hematology (ASH) Annual Meeting & Exposition; abstracts will be available at 9 a.m. ET today.

Third Quarter 2023 Financial Results

Revenue: Net U.S. product revenue from sales of PYRUKYND® for the third quarter of 2023 was $7.4 million, compared to $3.5 million for the third quarter of 2022.

Cost of Sales: Cost of sales for the third quarter of 2023 was $0.6 million.

Research and Development (R&D) Expenses: R&D expenses were $81.8 million for the third quarter of 2023, compared to $65.0 million for the third quarter of 2022. The year-over-year increase was primarily driven by the $17.5 million upfront payment to Alnylam for the TMPRSS6 asset.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $25.8 million for the third quarter of 2023 compared to $29.1 million for the third quarter of 2022. The year-over-year decrease was primarily attributable to lower stock-based compensation expense and reduced professional fees.

Net Loss: Net loss was $91.3 million for the third quarter of 2023 compared to $81.7 million for the third quarter of 2022.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of September 30, 2023, were $872.4 million compared to $1.1 billion as of December 31, 2022. Agios expects that its cash, cash equivalents and marketable securities together with anticipated product revenue, interest income and vorasidenib milestone will enable the company to fund its operating expenses and capital expenditures at least into 2026. This does not include potential royalties from vorasidenib, commercializing mitapivat outside of the U.S. through one or more partnerships, or other potential strategic business or financial agreements.

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 2023 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 the pioneering leader in PK activation and is dedicated to developing and delivering transformative therapies for patients living with rare 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 deep scientific expertise in classical hematology and leadership in the field of cellular metabolism and rare hematologic diseases, 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 is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera, and a preclinical PAH stabilizer as a potential treatment for phenylketonuria (PKU). 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 the potential benefits of PYRUKYND® (mitapivat), AG-946, TMPRSS6 siRNA and Agios’ PAH stabilizer; Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and its PAH stabilizer; Agios’ strategic vision and goals, including its key milestones for 2023; 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. For example, there can be no guarantee that any product candidate Agios 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, without limitation: risks and uncertainties related to the impact of pandemics or other public health emergencies to 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 current or future approved products, and launching, marketing and selling current or 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, 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 establish and maintain key collaborations; uncertainty regarding any milestone or royalty payments related to the sale of its oncology business or its in-licensing of TMPRSS6 siRNA, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios’ cash and cash equivalents; 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)

	September 30, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 872,390	$ 1,096,993
Accounts receivable, net	1,176	2,206
Inventory	17,274	8,492
Total assets	1,007,258	1,238,718
Stockholders' equity	886,843	1,100,814

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Revenues:				
Product revenue, net	$ 7,399	$ 3,516	$ 19,720	$ 7,430
Milestone revenue	—	—	—	2,500
Total revenue	7,399	3,516	19,720	9,930
Operating expenses:				
Cost of sales	$ 633	$ 517	$ 2,295	$ 1,291
Research and development	81,841	64,966	218,037	209,612
Selling, general and administrative	25,822	29,123	84,598	88,902
Total operating expenses	108,296	94,606	304,930	299,805
Loss from operations	(100,897)	(91,090)	(285,210)	(289,875)
Royalty income from gain on sale of oncology business	—	4,443	—	9,851
Interest income, net	8,375	3,818	24,720	6,305
Other income, net	1,198	1,082	4,342	5,392
Net loss	$ (91,324)	$ (81,747)	$ (256,148)	$ (268,327)
Net loss per share - basic and diluted	$ (1.64)	$ (1.49)	$ (4.61)	$ (4.90)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	55,803,663	54,844,579	55,559,766	54,734,301

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