



AgiOS Reports Business Highlights and Second Quarter 2023 Financial Results

August 3, 2023

- *Announced Positive Results from Phase 2 Portion of the RISE UP Pivotal Study of Mitapivat in Sickle Cell Disease; On Track to Enroll First Patient in Phase 3 Portion of the Study in Q4 2023*
- *Completed Enrollment in Phase 3 ENERGIZE and ENERGIZE-T Studies of Mitapivat in Thalassemia and Phase 2a Study of AG-946 in Lower-Risk MDS*
- *Announced Exclusive Worldwide License Agreement with Alnylam Pharmaceuticals to License Alnylam's Novel siRNA for the Potential Treatment of Polycythemia Vera*
- *U.S. PYRUKYND® (mitapivat) Net Revenue of \$6.7 Million in Q2; \$947 Million of Cash, Cash Equivalents and Marketable Securities as of June 30, 2023*

CAMBRIDGE, Mass., Aug. 03, 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 second quarter ended June 30, 2023.

"Since our last quarterly update, Agios has made tremendous progress executing across our industry-leading pipeline of PK activators, and today we are further expanding our portfolio beyond PK activation through focused business development," said Brian Goff, chief executive officer at Agios. "We announced positive data from the Phase 2 portion of the RISE UP study of mitapivat in sickle cell disease, completed enrollment in three clinical studies, licensed a compelling preclinical program from Alnylam, and continued to strengthen our commercial capabilities to support future anticipated launches. We look forward to the readout of the Phase 2a study of AG-946 in lower-risk MDS by the end of this year and the readouts of the Phase 3 studies of mitapivat in thalassemia next year."

Second Quarter 2023 & Recent Highlights

- **PYRUKYND® U.S. Launch:** Generated \$6.7 million in U.S. net revenue for the second quarter of 2023, a 20 percent increase over the first quarter of 2023. A total of 147 unique patients have completed prescription enrollment forms, representing an increase of 16 percent over the first quarter of 2023. A total of 99 patients are on PYRUKYND® therapy, representing an 11 percent increase over the first quarter of 2023.
- **Sickle Cell Disease:** Announced positive results from the Phase 2 portion of the RISE UP pivotal study of mitapivat in sickle cell disease.
- **Thalassemia:** Completed enrollment of the Phase 3 ENERGIZE and ENERGIZE-T studies of mitapivat in not regularly transfused and regularly transfused adults with thalassemia, respectively.
- **Lower-Risk Myelodysplastic Syndromes (LR-MDS):** Completed enrollment of the Phase 2a study of AG-946 in LR-MDS.
- **Business Development:** Announced an exclusive worldwide license agreement with Alnylam Pharmaceuticals for a novel siRNA for the potential treatment of polycythemia vera.
- **Leadership:** [Appointed](#) Catherine Owens to the board of directors. Kaye Foster assumed the role of lead independent director.
- **Other:** Data from Servier's Phase 3 trial of vorasidenib in patients with residual or recurrent IDH mutant low-grade glioma were presented during the plenary session at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting and published in the *New England Journal of Medicine*. As part of the divestiture of Agios' oncology business to Servier, Agios retains rights to a potential \$200 million milestone upon FDA approval of vorasidenib and 15% royalties on potential U.S. net sales.

Key Upcoming Milestones & Priorities

AgiOS expects to execute on the following additional key milestones and priorities by the end of 2023:

- **Pediatric PK Deficiency:** Enroll more than half of patients in the Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of mitapivat.
- **Sickle Cell Disease:** Enroll first patient in Phase 3 portion of RISE UP study of mitapivat, with the 100 mg dose selected from the successful Phase 2 portion.

- **Lower-risk Myelodysplastic Syndromes (LR-MDS):** Announce data from the Phase 2a study of novel PK activator AG-946.
- **Pipeline:** File investigational new drug (IND) application for phenylalanine hydroxylase (PAH) stabilizer for the treatment of phenylketonuria (PKU).

Second Quarter 2023 Financial Results

Revenue: Net U.S. product revenue from sales of PYRUKYND[®] for the second quarter of 2023 was \$6.7 million, compared to \$3.1 million for the second quarter of 2022. PYRUKYND[®] received FDA approval on February 17, 2022.

Cost of Sales: Cost of sales for the second quarter of 2023 was \$1.1 million.

Research and Development (R&D) Expenses: R&D expenses were \$68.9 million for the second quarter of 2023 compared to \$74.5 million for the second quarter of 2022. The year-over-year decrease was primarily driven by a decrease in workforce related expenses as a result of reduced headcount related to the evolution of our research organization.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were \$30.4 million for the second quarter of 2023 compared to \$28.3 million for the second quarter of 2022. The year-over-year increase was primarily attributable to an increase in stock-based compensation expense.

Net Loss: Net loss was \$83.8 million for the second quarter of 2023 compared to \$91.8 million for the second quarter of 2022.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of June 30, 2023, were \$946.9 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 second 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 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 has TMPRSS6 siRNA as a potential treatment for polycythemia vera, a PAH stabilizer in preclinical development as a potential treatment for phenylketonuria (PKU), and deep scientific expertise in classical hematology. 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 its 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 the COVID-19 pandemic 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 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 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. 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.

(Unaudited)

	June 30, 2023		December 31, 2022	
Cash, cash equivalents, and marketable securities	\$	946,923	\$	1,096,993
Accounts receivable, net		2,251		2,206
Inventory		15,671		8,492
Total assets		1,085,153		1,238,718
Stockholders' equity		964,236		1,100,814

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Revenues:				
Product revenue, net	\$ 6,712	\$ 3,082	\$ 12,321	\$ 3,914
Milestone revenue	—	2,500	—	2,500
Total revenue	6,712	5,582	12,321	6,414
Operating expenses:				
Cost of sales	\$ 1,108	\$ 435	\$ 1,662	\$ 774
Research and development	68,895	74,523	136,196	144,646
Selling, general and administrative	30,409	28,264	58,776	59,779
Total operating expenses	100,412	103,222	196,634	205,199
Loss from operations	(93,700)	(97,640)	(184,313)	(198,785)
Royalty income from gain on sale of oncology business	—	2,704	—	5,408
Interest income, net	8,254	1,793	16,345	2,487
Other income, net	1,640	1,337	3,144	4,310
Net loss	\$ (83,806)	\$ (91,806)	\$ (164,824)	\$ (186,580)
Net loss per share - basic and diluted	\$ (1.51)	\$ (1.68)	\$ (2.97)	\$ (3.41)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	55,604,330	54,799,680	55,435,796	54,678,249

Contacts:

Investor Contact

Chris Taylor, VP Investor Relations and Corporate Communications
Agius Pharmaceuticals
IR@agios.com

Media Contact

Dan Budwick
1AB Media
dan@1abmedia.com



Source: Agios Pharmaceuticals, Inc.