Agios Reports Business Highlights and Second Quarter 2024 Financial Results

August 1, 2024

– Reported Positive Topline Data from Phase 3 ENERGIZE-T Study; Expect to File sNDA Based on ENERGIZE and ENERGIZE-T Studies Encompassing All Thalassemia Subtypes by End of 2024 –

– Announced $905 Million Purchase Agreement for Vorasidenib Royalty with Royalty Pharma; Agios to Receive a Total of $1.1 Billion in Payments Upon FDA Approval of Vorasidenib –

– Reported Results from Phase 3 ACTIVATE-KidsT Study of Mitapivat in Children with Pyruvate Kinase (PK) Deficiency Who are Regularly Transfused –

– PYRUKYND® (Mitapivat) Net Revenue of $8.6 Million in Q2; Cash, Cash Equivalents and Marketable Securities of $645.3 Million as of June 30, 2024 –

CAMBRIDGE, Mass., Aug. 01, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in cellular metabolism and pyruvate kinase (PK) activation pioneering therapies for rare diseases, today reported business highlights and financial results for the second quarter ended June 30, 2024.

“Based on the positive data generated in the Phase 3 ENERGIZE and ENERGIZE-T studies, mitapivat is the first therapy to demonstrate efficacy in all subtypes of thalassemia, and we look forward to filing for FDA review by the end of the year,” said Brian Goff, chief executive officer at Agios. “This morning, we were pleased to report topline data in the Phase 3 ACTIVATE-KidsT study of mitapivat, which is the first study to report safety and efficacy data in children with PK deficiency. We continue to make significant progress toward our vision of becoming a leading rare disease company with a potential multi-billion-dollar franchise in PK activation. Finally, we were pleased to bolster our cash position through a purchase agreement with Royalty Pharma for our vorasidenib royalty, with Agios now positioned to receive a total of $1.1 billion in payments upon FDA approval of vorasidenib.”

Second Quarter 2024 and Recent Highlights

- *PYRUKYND® Revenues:* Generated $8.6 million in net revenue for the second quarter of 2024, a 5 percent sequential increase from the first quarter of 2024, primarily driven by increased patient demand. A total of 201 unique patients have completed prescription enrollment forms, representing an increase of 7 percent over the first quarter of 2024. A total of 128 patients are on PYRUKYND® therapy, a 7 percent increase from the first quarter of 2024.
- *Thalassemia:*
  - Met the primary and all key secondary endpoints in the Phase 3 ENERGIZE-T study of mitapivat in adults with transfusion-dependent alpha- or beta-thalassemia.
  - Presented positive results from the Phase 3 ENERGIZE study of mitapivat in adults with non-transfusion-dependent thalassemia in a plenary session at the European Hematology Association 2024 (EHA2024) Hybrid Congress.
- *Pediatric PK Deficiency:*
  - Announced topline data from the Phase 3 ACTIVATE-KidsT study of mitapivat in children with PK deficiency who are regularly transfused. Agios plans to present a more detailed analyses of the results at an upcoming medical meeting.
  - Completed enrollment of the Phase 3 ACTIVATE-Kids study of mitapivat in children with PK deficiency who are not regularly transfused. Topline data from this study are expected in 2025.
- *Corporate Development:*
  - Announced a $905 million purchase agreement with Royalty Pharma for Agios’ rights to its vorasidenib royalty. Under the agreement, Agios will receive a payment of $905 million upon approval of vorasidenib by the FDA and Royalty Pharma will receive the entirety of the 15% royalty on annual U.S. net sales of vorasidenib up to $1 billion, and a 12% royalty on annual U.S. net sales greater than $1 billion. Agios retains a 3% royalty on annual U.S. net sales greater than $1 billion. Agios retains rights to a $200 million milestone payment from Servier upon FDA approval of vorasidenib.
  - Entered into a distribution agreement with NewBridge Pharmaceuticals to advance commercialization of PYRUKYND® in the Gulf Cooperation Council (GCC) region. NewBridge, a leading specialty company headquartered in Dubai, will commercialize PYRUKYND® in Bahrain, Kuwait, Oman, Qatar, Saudi Arabia and the United Arab Emirates.

Key Upcoming Milestones & Priorities

Agios expects to execute on the following additional key milestones and priorities by the end of 2024:

- *Thalassemia:* File sNDA for mitapivat in thalassemia based on the positive results from the Phase 3 ENERGIZE and ENERGIZE-T trials (year-end).
- *Sickle Cell Disease:* Complete enrollment in the Phase 3 portion of the RISE UP study of mitapivat (year-end).
- *Lower-risk Myelodysplastic Syndromes:* Dose first patient in Phase 2b study of tebapivat (AG-946) (mid-year).
- *Other:* Potential approval of Servier's vorasidenib for the treatment of IDH-mutant diffuse glioma. The FDA has assigned a PDUFA action date of August 20, 2024.

Second Quarter 2024 Financial Results

Revenue: Net product revenue from sales of PYRUKYND® for the second quarter of 2024 was $8.6 million, compared to $6.7 million for the second quarter of 2023.

Cost of Sales: Cost of sales for the second quarter of 2024 was $1.5 million.

Research and Development (R&D) Expenses: R&D expenses were $77.4 million for the second quarter of 2024, compared to $68.9 million for the second quarter of 2023. The year-over-year increase was primarily attributable to an increase in costs associated with the in-licensed siRNA TMPRSS6 program for polycythemia vera.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $35.5 million for the second quarter of 2024 compared to $30.4 million for the second quarter of 2023. The year-over-year increase was primarily attributable to an increase in commercial-related activities as we prepare for the potential approval of PYRUKYND® in thalassemia.

Net Loss: Net loss was $96.1 million for the second quarter of 2024 compared to $83.8 million for the second quarter of 2023.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of June 30, 2024, were $645.3 million compared to $806.4 million as of December 31, 2023. Agios expects that its cash, cash equivalents and marketable securities together with anticipated product revenue, interest income and payments upon FDA approval of vorasidenib, will provide the financial independence to prepare for potential PYRUKYND® launches in thalassemia and sickle cell disease, and to opportunistically expand our pipeline through both internally and externally discovered assets.

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 2024 financial results and recent business highlights. 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, MDS-associated anemia and phenylketonuria (PKU). In addition to its clinical pipeline, Agios is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera. 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), tebapivat (AG-946), TMPRSS6 siRNA and AG-181, Agios' PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 (tebapivat) and AG-181; the potential FDA approval of vorasidenib; Agios' use of proceeds from the transaction with Royalty Pharma; potential U.S. net sales of vorasidenib and potential future royalty payments; Agios' strategic vision and goals, including its key milestones for 2024; 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)

	June 30, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 645,296	$ 806,363
Accounts receivable, net	3,762	2,810
Inventory	23,937	19,076
Total assets	773,063	937,118
Stockholders' equity	660,510	811,019

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenues:				
Product revenue, net	$ 8,615	$ 6,712	$ 16,804	$ 12,321
Total revenue	8,615	6,712	16,804	12,321
Operating expenses:				
Cost of sales	$ 1,495	$ 1,108	$ 2,122	$ 1,662
Research and development	77,401	68,895	146,021	136,196
Selling, general and administrative	35,536	30,409	66,550	58,776
Total operating expenses	114,432	100,412	214,693	196,634
Loss from operations	(105,817)	(93,700)	(197,889)	(184,313)
Interest income, net	8,120	8,254	17,009	16,345
Other income, net	1,579	1,640	3,213	3,144
Net loss	$ (96,118)	$ (83,806)	$ (177,667)	$ (164,824)
Net loss per share - basic and diluted	$ (1.69)	$ (1.51)	$ (3.14)	$ (2.97)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	56,802,546	55,604,330	56,593,011	55,435,796

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