Agios Reports Business Highlights and First Quarter 2024 Financial Results

May 2, 2024

– Topline Data Readout from the Phase 3 ENERGIZE-T Study of Mitapivat in Adults with Transfusion-Dependent Alpha- or Beta-Thalassemia now expected in Q2 2024 –

– Announced Positive Results from the Phase 3 ENERGIZE Study of Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia in January –

– Phase 3 Readouts from Mitapivat RISE UP Study in Sickle Cell Disease, ACTIVATE-KIDS and ACTIVATE KIDS-T in Pediatric PK Deficiency, All Expected by End of 2025 –

– PYRUKYND® (Mitapivat) Net Revenue of $8.2 Million in Q1; Cash, Cash Equivalents and Marketable Securities of $714.3 Million as of March 31, 2024 –

CAMBRIDGE, Mass., May 02, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in cellular metabolism and PK activation pioneering therapies for rare diseases, today reported business highlights and financial results for the first quarter ended March 31, 2024.

"We were delighted to report positive data from the Phase 3 ENERGIZE study of mitapivat in non-transfusion-dependent thalassemia and look forward to announcing topline data from the Phase 3 ENERGIZE-T study in transfusion-dependent thalassemia in the second quarter of this year," said Brian Goff, chief executive officer at Agios. "Mitapivat has the potential to become the first therapy approved for all thalassemia subtypes, and our commercial organization is actively preparing for a potential launch next year. Beyond thalassemia, we look forward to the RISE UP Phase 3 readout in sickle cell disease next year, with potential for approval in 2026, as we progress toward our vision of becoming a leading rare disease company with a potential multi-billion-dollar franchise in PK activation."

First Quarter 2024 and Recent Highlights

- *PYRUKYND® Revenues:* Generated $8.2 million in net revenue for the first quarter of 2024, a 15 percent sequential increase from the fourth quarter of 2023, primarily driven by increased patient demand. A total of 188 unique patients have completed prescription enrollment forms, representing an increase of 6 percent over the fourth quarter of 2023. A total of 120 patients are on PYRUKYND® therapy, a 10 percent increase from the fourth quarter of 2023.
- *Thalassemia:* Announced positive results from the Phase 3 ENERGIZE study of mitapivat in adults with non-transfusion-dependent alpha- or beta-thalassemia.
- *Earlier-Stage Pipeline:* Dosed the first participants in the Phase 1 study of AG-181 for the treatment of phenylketonuria (PKU).
- *Environment, Social, and Governance (ESG):* Published 2024 ESG Report, which provides corporate sustainability disclosures for the period Jan. 1, 2023 to Dec. 31, 2023.
- *Other:* Servier announced FDA filing acceptance and priority review for a new drug application (NDA) for vorasidenib for the treatment of IDH-mutant diffuse glioma. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) action date of August 20, 2024. 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 2024:

- *Thalassemia:* Two key milestones for the year, including reporting topline data from the Phase 3 ENERGIZE-T study of mitapivat in transfusion-dependent thalassemia (Q2) and filing for FDA approval of mitapivat in thalassemia (year-end).
- *Sickle Cell Disease:* Complete enrollment in the Phase 3 portion of the RISE UP study of mitapivat (year-end).
- *Pediatric PK Deficiency:* Complete enrollment in the Phase 3 ACTIVATE-kids study of mitapivat (mid-year). Report topline data from Phase 3 ACTIVATE kids-T study (now mid-year).
- *Lower-risk Myelodysplastic Syndromes:* Dose first patient in Phase 2b study of 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. Agios retains certain economic rights, as described above.

First Quarter 2024 Financial Results

Revenue: Net product revenue from sales of PYRUKYND® for the first quarter of 2024 was $8.2 million, compared to $5.6 million for the first quarter of 2023.

Cost of Sales: Cost of sales for the first quarter of 2024 was $0.6 million.

Research and Development (R&D) Expenses: R&D expenses were $68.6 million for the first quarter of 2024, compared to $67.3 million for the first quarter of 2023. The year-over-year comparison reflects an increase in process development expenses, offset by a decrease in workforce-related expenditures.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $31.0 million for the first quarter of 2024 compared to $28.4 million for the first 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 $81.5 million for the first quarter of 2024 compared to $81.0 million for the first quarter of 2023.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of March 31, 2024, were $714.3 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 vorasidenib milestone will enable the company to fund its operating expenses and capital expenditures at least into 2026. This does not include cash inflows, which could extend runway beyond 2026, including potential royalties or monetization of 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 first 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), 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 and AG-181, its PAH stabilizer; 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)

	March 31, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 714,292	$ 806,363
Accounts receivable, net	3,453	2,810
Inventory	23,070	19,076

Total assets	849,709	937,118
Stockholders' equity	743,922	811,019

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

	Three Months Ended March 31,	
	2024	**2023**
Revenues:		
Product revenue, net	$ 8,189	$ 5,609
Total revenue	8,189	5,609
Operating expenses:		
Cost of sales	$ 627	$ 554
Research and development	68,620	67,301
Selling, general and administrative	31,014	28,367
Total operating expenses	100,261	96,222
Loss from operations	(92,072)	(90,613)
Interest income, net	8,889	8,091
Other income, net	1,634	1,504
Net loss	$ (81,549)	$ (81,018)
Net loss per share - basic and diluted	$ (1.45)	$ (1.47)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	56,383,475	55,265,390

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