Agios Reports Second Quarter 2026 Financial Results and Provides Business Update

- Mitapivat (PYRUKYND® and AQVESME™) delivered worldwide net revenues of $44.7 million in the second quarter of 2026, compared to $12.5 million in the second quarter of 2025
- Strong U.S. commercial launch of AQVESME in thalassemia, with 442 cumulative prescriptions written as of June 30, 2026
- sNDA for mitapivat granted FDA Priority Review in sickle cell disease with PDUFA goal date of November 1, 2026
- Diversified and expanded late-stage pipeline with licensing of cevidoplenib for immune thrombocytopenia and advancement of AG-236 into Phase 2/3 development for polycythemia vera
- $964.8 million in cash, cash equivalents, and marketable securities as of June 30, 2026; well capitalized to support commercial execution and pipeline advancement

CAMBRIDGE, Mass., July 30, 2026 – Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a commercial-stage biopharmaceutical company focused on delivering innovative medicines for patients with rare diseases, today announced financial results and updates for the second quarter ended June 30, 2026.

“Our second-quarter performance reflects continued execution across the key priorities that will drive sustainable growth for Agios: strong commercial momentum, pipeline diversification, and strategic portfolio discipline,” said Brian Goff, Chief Executive Officer, Agios. “We are encouraged by the ongoing U.S. commercial launch of AQVESME in thalassemia, which continues to see robust engagement from both physicians and patients. We also progressed mitapivat toward a potential new indication in sickle cell disease, highlighted by the FDA granting Priority Review for our sNDA. Beyond these milestones, we strengthened our hematology pipeline with the licensing of cevidoplenib and advancement of AG-236 into Phase 2/3 development, while maintaining disciplined capital allocation. Together, these achievements underscore our ability to deliver meaningful innovation for patients and long-term shareholder value.”

Second Quarter 2026 and Recent Corporate Highlights

- **Mitapivat (PYRUKYND® and AQVESME™) Commercial Performance and Update –**
  - $40.9 million in U.S. net revenue and $3.8 million in ex-U.S. net revenue in the second quarter of 2026.
    - U.S. net revenue was driven by the U.S. commercial launch of AQVESME (mitapivat) in thalassemia in late January 2026.
    - Ex-U.S. net revenue reflected anticipated demand for PYRUKYND (mitapivat) in Europe following approval for thalassemia in May 2026, as well as continued, consistent early demand in Gulf Cooperation Council (GCC) countries.

- As of June 30, 2026, 442 cumulative AQVESME prescriptions for thalassemia have been written by Risk Evaluation and Mitigation Strategy (REMS)-certified U.S. physicians.

- **Business Development** –
  - Agios announced an agreement with Oscotec to license the exclusive global rights to cevidoplenib, a highly-selective, next-generation, oral spleen tyrosine kinase (SYK) inhibitor for immune thrombocytopenia (ITP). The addition of cevidoplenib diversifies Agios' rare hematology portfolio and represents an opportunity to unlock up to $1.0 billion in peak U.S. sales potential in this indication.
  - Agios expects to advance cevidoplenib into Phase 3 development for ITP in the first half of 2028, following completion of additional chemistry, manufacturing, and controls (CMC) development work.

Research and Development (R&D) Highlights

- **Mitapivat (pyruvate kinase [PK] activator)**
  - **Thalassemia –**
    - The European Commission (EC) granted marketing authorization for PYRUKYND in adults for the treatment of anemia associated with transfusion-dependent and non-transfusion-dependent alpha- or beta-thalassemia, with an orphan medicinal product designation. With this decision, PYRUKYND is the only medicine approved in all European Union (EU) member states for this broad patient population.
    - Mitapivat is now approved for adults with thalassemia in the U.S., Saudi Arabia, United Arab Emirates, and EU.

  - **Sickle Cell Disease –**
    - The U.S. Food and Drug Administration (FDA) accepted Agios' supplemental New Drug Application (sNDA) for mitapivat in sickle cell disease with a Priority Review. The Prescription Drug User Fee Act (PDUFA) goal date for this sNDA, submitted under the FDA's accelerated approval pathway, is November 1, 2026.
    - Additionally, Agios dosed the first patient in the REIGNITE Phase 3 trial, the confirmatory clinical trial required to be conducted under the accelerated approval pathway. This global trial is designed to demonstrate the clinical benefit of mitapivat on reducing transfusion burden in patients with sickle cell disease aged 12 years or older.
    - Agios also filed for regulatory approval of mitapivat for sickle cell disease in Saudi Arabia.

- **AG-236 (siRNA targeting TMPRSS6)**
  - **Polycythemia Vera –**

        - Results from Agios' Phase 1 trial of AG-236 in healthy volunteers demonstrated sustained hepcidin control and effects on iron regulation biomarkers without the need for titration, supporting iron pathway modulation that can potentially address excess red blood cell production in polycythemia vera.
        - The data also indicate the potential for an up to every-six-month dosing schedule.
        - Based on these results, Agios will advance AG-236 into a Phase 2/3 development program in polycythemia vera, with initiation of the Phase 2 portion expected in the second half of 2026.

- **AG-181 (phenylalanine hydroxylase [PAH] stabilizer)**
    - **Phenylketonuria (PKU) –**
        - Agios dosed the first patient in the Phase 1b trial evaluating the safety and tolerability of AG-181 in adults with PKU. Data from this trial are expected in the second half of 2026.
- **Tebapivat (PK activator)**
    - **Lower-Risk Myelodysplastic Syndromes (LR-MDS) –**
        - Agios announced that it will not advance tebapivat in LR-MDS following results from the company's Phase 2b trial. While tebapivat demonstrated evidence of biological activity, it did not demonstrate clinical benefit in a sufficient proportion of patients or any patient subgroup to meet the company's predefined threshold for advancement in LR-MDS.

    - **Sickle Cell Disease –**
        - Agios announced that it will not advance tebapivat in sickle cell disease following results from the company's Phase 2 trial. The data further reinforced PK activation as a clinically validated mechanism in sickle cell disease; however, they did not demonstrate a sufficiently differentiated profile relative to other PK activators to justify continued development of tebapivat in this indication.

Second Quarter 2026 Financial Results

For the quarter ended June 30, 2026, net loss was $100.7 million, compared to net loss of $112.0 million for the quarter ended June 30, 2025.

- **Net product revenue from U.S. sales** of mitapivat (PYRUKYND and AQVESME) for the second quarter of 2026 was $40.9 million, compared to $12.2 million for the second quarter of 2025.

- **Net product revenue from ex-U.S. sales** of mitapivat (PYRUKYND) for the second quarter of 2026 was $3.8 million, compared to $0.3 million for the second quarter of 2025.

- **Cost of sales** for the second quarter of 2026 was $3.0 million.
- **Research and Development (R&D) expenses** were $100.8 million for the second quarter of 2026, compared to $91.9 million for the second quarter of 2025, driven primarily by the $25.0 million up-front payment associated with the agreement with Oscotec to license cevidoplenib.
- **Selling, General and Administrative (SG&A) expenses** were $51.5 million for the second quarter of 2026, compared to $45.9 million for the second quarter of 2025, due to an increase in activities related to the U.S. commercial launch of AQVESME in thalassemia.
- **Cash, cash equivalents and marketable securities** were $964.8 million as of June 30, 2026, compared to $1.2 billion as of December 31, 2025. Agios expects that its cash, cash equivalents and marketable securities, together with anticipated product revenue and interest income, will provide the financial independence to execute the U.S. commercial launch of AQVESME in thalassemia, prepare for the potential U.S. commercial launch of mitapivat in sickle cell disease, advance the company's existing clinical programs, and opportunistically expand its pipeline through both internally- and externally-discovered assets.

Second Quarter 2026 Conference Call Information
Agios will host a conference call and live webcast today, July 30, 2026, at 8:00 a.m. ET to discuss the company's second quarter 2026 financial results and recent business highlights. The live webcast will be accessible on the Investors section of the company's website (www.agios.com) under the "Events & Presentations" tab. A replay of the webcast will be available on the company's website approximately two hours after the event.

About Agios: Fueled by Connections to Transform Rare Diseases™
At Agios, our vision is to redefine the future of rare disease treatment. Fueled by connections, we build trusted partnerships with communities – collaborating to develop and deliver innovative medicines that have the potential to transform lives. With a foundation in hematology, we combine biological expertise with real-world insights to advance a growing pipeline of rare disease medicines that reflect the priorities of the people we serve. Agios is a commercial-stage biopharmaceutical company headquartered in Cambridge, Massachusetts. To learn more, visit www.agios.com and follow us on LinkedIn and X.

Available Information about Agios
To achieve broad dissemination, Agios may disclose information to the public through a variety of disclosure channels including press releases, SEC filings, and public conference calls and webcasts. Some of the information distributed through these disclosure channels may be considered material information. Investors and others should note that Agios plans to use its website (www.agios.com) as a distribution channel to announce and give notice of Agios' upcoming events and presentations (including, but not limited to, presentations at medical or healthcare

conferences). Such information, which may be deemed material, will be available on the Investors section of the company’s website under the “Events & Presentations” tab. In addition, you may sign up to automatically receive email alerts about Agios’ upcoming events and presentations (“Calendar Alerts”) by visiting the “Email Alerts” option under the “IR Resources” tab of the Investors section of the company’s website and submitting your email address.

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), AQVESME™ (mitapivat), tebapivat, AG-236, AG-181 and cevidoplenib; Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including mitapivat, tebapivat, AG-236, AG-181 and cevidoplenib; Agios’ expectations for the review of marketing applications for mitapivat by regulatory agencies, including the FDA and European Commission; Agios’ strategic vision and goals; 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 royalty payments related to the sale of its oncology business or any milestone or royalty payments related to its in-licensing of AG-236 and cevidoplenib, 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, 2026	December 31, 2025
Cash, cash equivalents, and marketable securities	$ 964,830	$ 1,164,438
Accounts receivable, net	20,112	10,577
Inventory*	35,752	32,920
Total assets	1,108,424	1,297,225
Stockholders' equity	1,026,021	1,193,114

*June 30, 2026 balance includes $5.6 million of long-term inventory included in other non-current assets within our condensed consolidated balance sheets.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenues:				
Product revenue, net	$ 44,745	$ 12,455	$ 65,491	$ 21,181
Total revenue	44,745	12,455	65,491	21,181
Operating expenses:				
Cost of sales	$ 2,996	$ 1,702	$ 4,315	$ 2,787
Research and development	100,754	91,940	181,902	164,683
Selling, general and administrative	51,547	45,869	99,851	87,396
Total operating expenses	155,297	139,511	286,068	254,866
Loss from operations	(110,552)	(127,056)	(220,577)	(233,685)
Interest income, net	9,730	14,513	20,525	30,600
Other income, net	119	523	238	1,776
Net loss	$ (100,703)	$ (112,020)	$ (199,814)	$ (201,309)
Net loss per share - basic and diluted	$ (1.69)	$ (1.93)	$ (3.38)	$ (3.49)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	59,488,871	57,932,576	59,137,508	57,697,193

Contacts:
Investor Contact
Morgan Sanford, Vice President, Investor Relations
Agios Pharmaceuticals
morgan.sanford@agios.com

Media Contact
Eamonn Nolan, Senior Director, Corporate Communications
Agios Pharmaceuticals
eamonn.nolan@agios.com