Agios Reports Business Highlights and Second Quarter 2021 Financial Results

July 29, 2021

– Completed Regulatory Submissions for Mitapivat for the Treatment of Adults with Pyruvate Kinase (PK) Deficiency in the U.S. and EU –

– Presented Data from ACTIVATE and ACTIVATE-T Phase 3 Studies and Thalassemia Phase 2 Study at European Hematology Association Virtual Congress, Supporting Potential of PK Activation for Underserved Patients with Hemolytic Anemias –

– Chris Bowden, M.D., to Retire as Chief Medical Officer and Continue as Strategic Advisor; Vice President of Clinical Development Sarah Gheuens, M.D., Ph.D., to Assume Role –

CAMBRIDGE, Mass., July 29, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat genetically defined diseases, today reported business highlights and financial results for the second quarter ended June 30, 2021.

"Our first quarter as a company solely focused on genetically defined diseases was marked by several significant milestones, most notably our U.S. and EU regulatory submissions for mitapivat for the treatment of adults with PK deficiency, bringing us one step closer to potentially delivering the first disease-modifying treatment for people with this serious and underserved condition," said Jackie Fouse, Ph.D., chief executive officer at Agios. "We continue to unlock the potential of mitapivat for people with other chronic hemolytic anemias and are gaining momentum on our pivotal programs in thalassemia and sickle cell disease. This year, we look forward to initiating our two registrational Phase 3 trials – ENERGIZE and ENERGIZE-T – in not regularly transfused and regularly transfused adults with thalassemia, as well as our pivotal Phase 2/3 trial of mitapivat in sickle cell disease."

SECOND QUARTER 2021 & RECENT HIGHLIGHTS

- Completed two regulatory filings for approval of mitapivat in adults with PK deficiency; submitted new drug application (NDA) in the U.S. and marketing authorization application (MAA) in the EU.
- Presented full analysis of data from Phase 3 ACTIVATE and ACTIVATE-T studies of mitapivat in adults with PK deficiency at European Hematology Association (EHA) Virtual Congress; studies met primary and secondary endpoints, including patient-reported outcome (PRO) measures that address symptom burden and quality-of-life impact in adults.
- Presented data from Phase 2, open-label, multicenter study of mitapivat in adults with non-transfusion dependent α- or β-thalassemia at EHA Virtual Congress; study met its primary endpoint, with 16 of the 20 patients (80%) achieving a hemoglobin increase of ≥1.0 g/dL from baseline at one or more assessments during Weeks 4-12.
- Launched myAgios® patient support services for people living with PK deficiency and their caregivers, providing tailored support, educational resources and opportunities to connect with other patients and caregivers in the community.
- Completed hiring and training of customer-facing and patient support team that will support the U.S. launch of mitapivat in PK deficiency upon product approval.
- Announced succession plan for Chris Bowden, M.D., who will transition from his role as chief medical officer to strategic advisor following his retirement on Sept. 1, at which time Sarah Gheuens, M.D., Ph.D., vice president of clinical development, will assume the role.
- Repurchased approximately 10.5 million shares of Agios common stock, inclusive of shares acquired from Bristol-Myers Squibb Company (BMS) and its affiliates, at an average price of $50.41 per share. This accounts for $529 million of the up to $1.2 billion of share repurchases authorized by the Board of Directors following the company's sale of its oncology business to Servier.

KEY UPCOMING MILESTONES

- Initiate two Phase 3 studies of mitapivat, ENERGIZE and ENERGIZE-T, in not regularly transfused and regularly transfused adults with thalassemia, by year-end.
- Initiate Phase 2/3 study of mitapivat in sickle cell disease by year-end.
- Host investor day in fourth quarter to share more information about commercial launch planning for mitapivat in PK deficiency and research and development pipeline.

Data Presentations

- Submit data from ongoing collaborator-led clinical studies of mitapivat in sickle cell disease for presentation at the 63rd American Society of Hematology (ASH) Annual Meeting & Exposition, to be held Dec. 11-14.
- Submit data from the Phase 1 healthy volunteer study of AG-946, the company's next-generation PKR activator, for presentation at the 63rd ASH Annual Meeting & Exposition.

SECOND QUARTER 2021 FINANCIAL RESULTS

The financial results discussion compares Agios' continuing operations. All periods have been adjusted to exclude discontinued operations related to

the divested oncology business.

Research and Development (R&D) Expenses: R&D expenses for continuing operations were $62.0 million for the second quarter of 2021 compared to $54.1 million for the second quarter of 2020. The year-over-year increase in R&D was driven primarily by start-up costs associated with the Phase 3 studies of mitapivat in thalassemia and sickle cell disease, as well as the NDA and MAA filings for mitapivat in PK deficiency and launch preparation activities.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses for continuing operations were $29.2 million for both the second quarter of 2021 and the second quarter of 2020.

Non-Operating Income: Non-operating income included approximately $2.0 million from TIBSOVO® (ivosidenib) royalties for the second quarter of 2021.

Net Loss: Net loss was $86.2 million for the second quarter of 2021 compared to a net loss of $90.5 million for the second quarter of 2020.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of June 30, 2021, were $1.7 billion compared to $794.4 million as of June 30, 2020. The company expects that its cash, cash equivalents and marketable securities as of June 30, 2021 will enable the company to execute its operating plan through major catalysts and to cash-flow positivity without the need to raise additional equity.

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 2021 financial results and recent business activities. To participate in the conference call, please dial 1-877-377-7098 (domestic) or 1-631-291-4547 (international) and refer to conference ID 2663508. 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 focused on discovering and developing novel investigational medicines to treat genetically defined diseases through scientific leadership in the field of cellular metabolism. The company’s most advanced drug candidate is a first-in-class pyruvate kinase R (PKR) activator, mitapivat, that is currently being evaluated for the treatment of three distinct hemolytic anemias. In addition to its active late-stage clinical pipeline, Agios has multiple novel, investigational therapies in clinical and preclinical development. 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 Agios’ plans, strategies and expectations for the preclinical, clinical and commercial advancement of its drug development programs, including mitapivat and AG-946; the potential benefits of Agios’ products and product candidates; Agios’ key milestones and guidance for 2021; its financial guidance regarding the period in which it will have capital available to fund its operations; expectations regarding the return of capital to shareholders following the sale of Agios’ oncology business; its chief medical officer transition plan 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. 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 failure of Agios to receive milestone or royalty payments related to the sale of its oncology business, the uncertainty of the timing of any receipt of any such payments, and the uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; the impact of the COVID-19 pandemic on 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 future approved products, and launching, marketing and selling 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, including with respect to the regulatory submissions for mitapivat, 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 and 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 collaborations; 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, or SEC, including the risks and uncertainties set forth under the heading Risk Factors in our filings with the SEC. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this communication are made only as of the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	June 30, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 1,734,301	$ 670,537
Assets held for discontinued operations	-	50,460
Total assets	1,874,603	852,952
Liabilities held for discontinued operations	-	299,728

Stockholders' equity	1,721,816	399,500

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Cost and expenses:				
Research and development	$ 62,007	$ 54,086	$ 119,674	$ 109,445
Selling, general and administrative	$ 29,215	$ 29,178	$ 62,765	$ 60,849
Total cost and expenses	$ 91,222	$ 83,264	$ 182,439	$ 170,294
Loss from operations	$ (91,222)	$ (83,264)	$ (182,439)	$ (170,294)
Gain on sale of oncology business	$ 2,000	$ -	$ 2,000	$ -
Interest (expense) income, net	$ (92)	$ 1,769	$ 248	$ 4,705
Other income, net	$ 6,524	$ -	$ 6,524	$ -
Net loss from continuing operations	$ (82,790)	$ (81,495)	$ (173,667)	$ (165,589)
Net income (loss) from discontinued operations, net of tax	$ (3,427)	$ (8,983)	$ 1,961,775	$ 34,855
Net income (loss)	$ (86,217)	$ (90,478)	$ 1,788,108	$ (130,734)
Net loss from continuing operations per share - basic and diluted	$ (1.36)	$ (1.18)	$ (2.66)	$ (2.41)
Net income (loss) from discontinued operations per share - basic and diluted	$ (0.06)	$ (0.13)	$ 30.05	$ 0.51
Net income (loss) per share - basic and diluted	$ (1.41)	$ (1.31)	$ 27.39	$ (1.90)
Weighted-average number of common shares used in computing net income (loss) per share – basic and diluted	61,066,977	68,958,091	65,281,827	68,784,109

Contacts

Investors:
Steve Klass, 1AB
steve@1abmedia.com

Media:
Jessica Rennekamp, 857-209-3286
Director, Corporate Communications
Jessica.Rennekamp@agios.com

Source: Agios Pharmaceuticals, Inc.