Agios Reports Fourth Quarter and Full Year 2020 Financial Results

February 25, 2021

– Strong Commercial Execution for Second Full Year of TIBSOVO® with Fourth Quarter Net Revenue of $39.1M and $121.1M for Full Year 2020 –

– Sale of Oncology Portfolio Expected to Close Around March 31 Subject to March 25 Shareholder Vote, Allowing Agios to Focus on Genetically Defined Diseases –

– Company Outlines Pivotal Development Program for Mitapivat in Sickle Cell Disease, Enabling a Potential Broad Label by Year-end 2026 –

– Darrin Miles Appointed to Role of Chief Commercial Officer –

CAMBRIDGE, Mass., Feb. 25, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and genetically defined diseases, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2020.

"This past year was a transformative one for Agios," said Jackie Fouse, Ph.D., chief executive officer at Agios. "Despite the extraordinary challenges brought on by the global COVID-19 pandemic and civil and political unrest in the U.S., we remain hopeful for the future of our country and the promise of our industry and more confident than ever in our ability to execute on our plans on behalf of patients. The sale of our oncology business to Servier on attractive terms both allows our oncology portfolio to grow and flourish and facilitates our new laser focus on genetically defined diseases, where we anticipate a catalyst-rich year ahead for mitapivat across our three initial disease indications. In addition to our plans to file for approval for mitapivat in adults with PK deficiency in the U.S. and EU and launching our Phase 3 thalassemia trials later this year, we're pleased to share our pivotal strategy for mitapivat in sickle cell disease which we believe will enable us to pursue a broad label for patients who desperately need new treatment options."

FOURTH QUARTER 2020 & RECENT HIGHLIGHTS

- Entered into definitive agreement to sell commercial, clinical and research-stage oncology portfolio to Servier, an independent global pharmaceutical company, in December.
- Achieved TIBSOVO® (ivosidenib tablets) net sales of $39.1 million for the quarter and $121.1 million for the year, exceeding the $115 million updated net revenue target and representing a 102% increase in net sales year-over-year.
- Announced topline results from two Phase 3 studies of mitapivat in adults with pyruvate kinase (PK) deficiency who were not regularly transfused (ACTIVATE) and who were regularly transfused (ACTIVATE-T). Both studies achieved statistical significance for their primary endpoint with ACTIVATE demonstrating a clinically meaningful sustained increase in hemoglobin compared to placebo and ACTIVATE-T demonstrating a clinically meaningful reduction in transfusion burden. Statistical significance was also achieved for all pre-specified key secondary endpoints for ACTIVATE demonstrating an improvement compared to placebo, including in patient-reported outcomes (PRO) based on changes from baseline in pyruvate kinase deficiency diary (PKDD) score and pyruvate kinase deficiency impact assessment (PKDIA) score at Week 24. The safety profile was consistent with results from prior studies.
- Completed U.S. and EU regulatory interactions on the pivotal development plan for mitapivat in sickle cell disease, resulting in the Phase 2/3 trial design being announced today.
    - The Phase 2 will randomize 69 patients 1:1:1 to 50 mg mitapivat BID, 100 mg mitapivat BID or matched placebo. The primary endpoint is hemoglobin response defined as ≥1 g/dL change from baseline to Week 12.
    - The Phase 3 will randomize 198 patients 2:1 to the selected Phase 2 dose of mitapivat or matched placebo. The study includes two primary endpoints: hemoglobin response defined as ≥1 g/dL change from baseline to Week 52 and annualized rate of sickle cell pain crises.
    - Potential regulatory approval with a broad label based on the operationally seamless Phase 2/3 trial expected by the end of 2026.
- Appointed Darrin Miles, previously senior vice president, U.S. commercial and global marketing, to role of chief commercial officer.
- Presented final data, including mature overall survival (OS) results, from the Phase 3 ClarIDHy study of TIBSOVO® in patients with previously treated isocitrate dehydrogenase 1 (IDH1) mutated cholangiocarcinoma at the American Society of Clinical Oncology Gastrointestinal Cancers Symposium (ASCO-GI) in January.
- Presented updated Phase 1 data for mitapivat in sickle cell disease at the American Society of Hematology (ASH) Annual Meeting in December.
- Launched Anemia ID, a program providing no-cost genetic testing for patients with suspected hereditary anemias, including PK deficiency in November.

ANTICIPATED 2021 KEY MILESTONES

Corporate

- Complete sale of oncology portfolio to Servier in a transaction worth up to $2 billion plus royalties, following a shareholder vote on March 25, and execute a meaningful portion of the planned $1.2 billion capital return by year-end.

Genetically Defined Diseases

- File for regulatory approval for mitapivat in adults with PK deficiency: submit new drug application (NDA) in the U.S. in the second quarter of 2021 and marketing authorization application (MAA) in the EU in mid-2021.
- Initiate two Phase 3 studies of mitapivat, ENERGIZE and ENERGIZE-T, in not regularly transfused and regularly transfused adults with thalassemia in the second half of 2021.
- Initiate Phase 2/3 study of mitapivat in sickle cell disease by year-end.
- Prioritize new indications for pyruvate kinase R (PKR) and pyruvate kinase M2 (PKM2) activator clinical development by year-end.

Genetically Defined Disease Data Presentations

- Submit data from the following clinical studies for presentation at the EHA Virtual Congress, hosted June 9-17, 2021:
  - Phase 3 ACTIVATE study of mitapivat in adults with PK deficiency who do not receive regular transfusions
  - Phase 3 ACTIVATE-T study of mitapivat in adults with PK deficiency who receive regular transfusions
  - Phase 2 study of mitapivat in adults with α- and β-thalassemia who do not receive regular transfusions
- Submit data from ongoing clinical studies of mitapivat in sickle cell disease for presentation at medical meetings throughout 2021.
- Present data from the single ascending dose (SAD) and multiple ascending dose (MAD) cohorts of the Phase 1 study of AG-946, the company's next-generation PKR activator, in healthy volunteers by year-end.

Oncology

- Submit supplemental new drug application (sNDA) in the U.S. for TIBSOVO® in patients with previously treated IDH1-mutant cholangiocarcinoma in Q1 2021.
- Enrollment in the Phase 3 AGILE trial of TIBSOVO® in combination with azacitidine in adult patients with previously untreated IDH1-mutant acute myeloid leukemia is expected to complete by year-end.
- Enrollment in the relapsed or refractory myelodysplastic syndrome arm of the TIBSOVO® Phase 1 study of IDH1-mutant advanced hematologic malignancies is expected to complete by year-end.
- Full-year 2021 net product revenue for TIBSOVO® is expected to be $160-170 million.

FOURTH QUARTER AND FULL YEAR 2020 FINANCIAL RESULTS

Revenue: Total revenue for the fourth quarter of 2020 was $44.0 million, which includes $2.0 million in collaboration revenue, $39.1 million of net product revenue from sales of TIBSOVO® and $2.9 million in royalty revenue from net global sales of IDHIFA® under our collaboration agreement with Celgene. This compares to $35.4 million for the fourth quarter of 2019, which included $12.9 million in collaboration revenue, $19.6 million of net product revenue from U.S. sales of TIBSOVO® and $3.0 million in royalty revenue from net global sales of IDHIFA®. Total revenue for the year ended December 31, 2020 was $203.2 million compared to $117.9 million for the year ended December 31, 2019. The increase in 2020 revenue was primarily driven by a 102% increase in TIBSOVO® net product revenue and higher collaboration revenue due to recognition of the remainder of the deferred revenue balance related to the completion of the metabolic immuno-oncology collaboration with Celgene Corporation, a wholly owned subsidiary of Bristol Myers Squibb Company.

Cost of Sales: Cost of sales were $1.0 million for the fourth quarter of 2020 compared to $0.3 million for the fourth quarter of 2019, and $2.8 million for the year ended December 31, 2020 compared to $1.3 million for the year ended December 31, 2019.

Research and Development (R&D) Expenses: R&D expenses were $95.7 million for the fourth quarter of 2020 compared to $106.2 million for the fourth quarter of 2019 and $367.5 million for the year ended December 31, 2020 compared to $410.9 million for the year ended December 31, 2019. The decrease in R&D expense was primarily attributable to a decrease in TIBSOVO® clinical development costs, including winding down the ClarIDHy Phase 3 study.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $39.8 million for the fourth quarter of 2020 compared to $34.8 million for the fourth quarter of 2019, and $149.1 million for the year ended December 31, 2020 compared to $132.0 million for the year ended December 31, 2019. The increase in SG&A expense was primarily attributable to increased workforce expenses and professional fees related to the Servier transaction, partially offset by a decrease in external spending due to cost savings initiatives and reduced employee travel related expenses due to restrictions.

Net Loss: Net loss was $97.7 million for the fourth quarter of 2020 compared to $102.4 million for the fourth quarter of 2019, and $327.4 million for the year ended December 31, 2020 compared to a net loss of $411.5 million for the year ended December 31, 2019.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of December 31, 2020 were $670.5 million compared to $717.8 million as of December 31, 2019. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2020, together with anticipated product and royalty revenue, interest income and expense reimbursements under our collaboration agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its planned operating expenses and capital expenditure requirements to the end of 2022. Following the closing of the Servier transaction and net of the planned capital return, Agios expects to be able to fund its operation 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 fourth quarter and full year 2020 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 referring to conference ID 8442238. 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 malignant hematology, solid tumors and genetically defined diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across these three therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or preclinical development. For more information, please visit the company’s website at www.agios.com.

Additional Information and Where to Find It

This communication relates to the proposed transaction involving the sale by Agios Pharmaceuticals, Inc. (“Agios”) of its oncology business to Servier Pharmaceuticals, LLC. In connection with the proposed transaction, Agios filed with the U.S. Securities and Exchange Commission (the “SEC”) a definitive proxy statement on Schedule 14A on February 11, 2021 and Agios commenced mailing the definitive proxy statement to its stockholders on February 12, 2021. BEFORE MAKING ANY VOTING DECISION, STOCKHOLDERS OF AGIOS ARE URGED TO READ THE DEFINITIVE PROXY STATEMENT REGARDING THE TRANSACTION AND ANY OTHER RELEVANT DOCUMENTS FILED OR THAT WILL BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THOSE DOCUMENTS, BECAUSE THEY CONTAIN OR WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION. Investors and security holders will be able to obtain the documents (when available) free of charge at the SEC’s website, at http://www.sec.gov, and Agios’ website, at www.agios.com. In addition, the documents (when available) may be obtained free of charge by accessing Agios’ website at www.agios.com under the heading “Investors” or, alternatively, directing a request to Holly Manning by email at holly.manning@agios.com or by calling 617-649-8600.

Participants in the Solicitation

Agios and its directors and executive officers may be deemed to be participants in the solicitation of proxies from the holders of Agios common stock in respect of the proposed transaction. Information about the directors and executive officers of Agios is set forth in the proxy statement for Agios’ 2020 annual meeting of stockholders, which was filed with the SEC on April 16, 2020, and in other documents filed by Agios with the SEC. Other information regarding the participants in the proxy solicitation and a description of their direct and indirect interests, by security holdings or otherwise, is contained in the definitive proxy statement and in other relevant materials filed or to be filed with the SEC in respect of the proposed transaction when they become available.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of within the meaning of the Private Securities Litigation Reform Act of 1995, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Such forward-looking statements include those regarding Agios’ plans, strategies and expectations for the preclinical, clinical and commercial advancement of its drug development programs; the potential benefits of Agios’ products and product candidates; Agios’ key milestones and guidance for 2021 and strategic vision; its financial guidance regarding the period in which it will have capital available to fund its operations; expectations regarding the sale of Agios’ oncology portfolio and associated return of capital to shareholders; and the potential benefits of Agios’s 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: (i) Agios’s sale of its oncology portfolio, including the occurrence of any event, change or other circumstance that could give rise to the termination of the purchase and sale agreement; the failure of Agios to obtain stockholder approval for the proposed transaction or the failure to satisfy any of the other conditions to the completion of the proposed transaction; the effect of the announcement of the proposed transaction on the ability of Agios to retain and hire key personnel and maintain relationships with its customers, suppliers, advertisers, partners and others with whom it does business, or on its operating results and businesses generally; risks associated with the disruption of management’s attention from ongoing business operations due to the proposed transaction; the ability to meet expectations regarding the timing and completion of the proposed transaction, including with respect to receipt of required regulatory approvals; the failure of Agios to receive milestone or royalty payments under the purchase and sale agreement and 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 proposed transaction; (ii) the impact of the COVID-19 pandemic 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; (iii) Agios’ results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; (iv) 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; (v) Agios’ ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; (vi) unplanned cash requirements and expenditures and competitive factors; (vii) Agios’ ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; (viii) Agios’ ability to maintain key collaborations; and (ix) 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, including the risks and uncertainties set forth in Item 1A under the heading Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2019, our Quarterly Report on Form 10-Q for the fiscal quarter ended on September 30, 2020 filed with the SEC on November 5, 2020, our definitive proxy statement and other subsequent periodic reports we file with the SEC, which are available at http://www.sec.gov and our website at http://www.agios.com. 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.

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Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	December 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 670,537	$ 717,806
Accounts receivable, net	21,328	8,952
Collaboration receivable – related party	2,123	1,539
Royalty receivable – related party	-	2,900
Inventory	14,698	7,331
Total assets	852,952	890,741
Deferred revenue – related party	-	61,513
Stockholders' equity	399,500	640,528

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

	Three Months Ended December 31,		Years Ended December 31,	
	2020	2019	2020	2019
Revenues:				
Product revenue, net	$ 39,118	$ 19,564	$ 121,089	$ 59,851
Collaboration revenue – related party	1,236	6,843	68,274	39,257
Collaboration revenue – other	785	6,060	3,571	8,262
Royalty revenue – related party	2,906	2,973	10,262	10,542
Total Revenue	44,045	35,440	203,196	117,912
Cost and expenses:				
Cost of sales	959	287	2,805	1,317
Research and development	95,742	106,248	367,470	410,894
Selling, general and administrative	39,778	34,834	149,070	132,034
Total cost and expenses	136,479	141,369	519,345	544,245
Loss from operations	(92,434)	(105,929)	(316,149)	(426,333)
Interest income, net	791	3,579	6,611	14,861
Non-cash interest expense for the sale of future revenue	(6,014)	-	(17,832)	-
Net loss	$ (97,657)	$ (102,350)	$ (327,370)	$ (411,472)
Net loss per share – basic and diluted	$ (1.41)	$ (1.60)	$ (4.74)	$ (6.86)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	69,271,163	63,949,870	68,997,879	59,994,539

Source: Agios Pharmaceuticals, Inc.