

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549**

FORM 8-K

**CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported):
December 21, 2020**

Agios Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-36014
(Commission
File Number)

26-0662915
(IRS Employer
Identification No.)

88 Sidney Street
Cambridge, Massachusetts
(Address of principal executive offices)

02139
(Zip code)

(617) 649-8600
(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Title of Each Class	Trading Symbol(s)	Exchange
Common Stock, Par Value \$0.001 per share	AGIO	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 7.01 Regulation FD Disclosure.

An investor presentation containing additional information relating to the proposed transaction described in Item 8.01 is attached to this Current Report on Form 8-K as Exhibit 99.2.

The information in Exhibit 99.2 is being furnished and shall not be deemed filed for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall such information be deemed to be incorporated by reference in any registration statement or other document filed under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as otherwise stated in such filing.

Item 8.01 Other Events.

On December 21, 2020, Agios Pharmaceuticals, Inc. (the "Company") issued a press release announcing the execution of an agreement pursuant to which it agreed to sell the Company's oncology business to Servier Pharmaceuticals, LLC. A copy of the press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The Company will hold a conference call at 8:00 a.m., Eastern Time, on December 21, 2020 to provide supplemental information regarding the proposed transaction.

Item 9.01 Financial Statements and Exhibits.

(d) *Exhibits.*

<u>Exhibit Number</u>	<u>Description</u>
99.1	Press Release, dated December 21, 2020
99.2	Investor Presentation, dated December 21, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AGIOS PHARMACEUTICALS, INC.

Date: December 21, 2020

/s/ Jacquelyn A. Fouse
Jacquelyn A. Fouse, Ph.D.
Chief Executive Officer



AgiOS to Focus on Developing and Commercializing Innovative Treatments for Genetically Defined Diseases and Sell Its Oncology Business to Servier for Up to \$2 Billion Plus Royalties

– Agios to Dedicate All Resources to Advancing Mitapivat as a Potential Treatment for Three Initial Hemolytic Anemias and Building on its Scientific Expertise in Cellular Metabolism and PK Activation to Accelerate and Expand its Genetically Defined Disease Portfolio –

– Agios to Receive \$1.8 Billion in Upfront Cash, a Potential \$200 Million Regulatory Milestone Payment for Vorasidenib and Future Royalties on U.S. Net Sales of TIBSOVO® and Vorasidenib –

– Agios Plans to Return at Least \$1.2 Billion to Shareholders Following the Close of the Transaction, Anticipated in Q2 2021 –

– Company to Host Investor Event and Webcast Today at 8:00 a.m. ET –

CAMBRIDGE, Mass., December 21, 2020 — Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced that it will move forward with a singular focus on accelerating and expanding its genetically defined disease portfolio, including the mitapivat clinical programs and a robust pipeline of therapeutic candidates, and has entered into a definitive agreement to sell its commercial, clinical and research-stage oncology portfolio to Servier, an independent global pharmaceutical company. Agios will receive a cash consideration of up to \$2.0 billion, including \$1.8 billion in upfront cash and \$200 million in a potential future milestone payment for vorasidenib, as well as 5% royalties on U.S. net sales of TIBSOVO® (ivosidenib tablets) from transaction close through loss of exclusivity and 15% royalties on U.S. net sales of vorasidenib from first commercial sale through loss of exclusivity.

“Our decision to accelerate the next chapter of Agios’ success with a singular focus on genetically defined diseases and sell our oncology portfolio to Servier is a transformational milestone for Agios. The result of a deliberative strategic review, this decision reflects the progress we have made understanding and harnessing the science and promise of PK activation and captures the full value of our oncology assets,” said Jackie Fouse, Ph.D., chief executive officer of Agios. “With mitapivat poised to become a new potential treatment option for patients with pyruvate kinase (PK) deficiency, thalassemia and sickle cell disease and with a rich pipeline based on our pioneering leadership in PK activation and cellular metabolism, Agios’ near- and long-term future is filled with significant value-generating catalysts. The proceeds from the transaction will allow us to focus on rapidly advancing our genetically defined disease portfolio for patients in need, strengthen our capital structure and return at least \$1.2 billion to shareholders post-closing, achieve capital markets independence and participate in the future success of TIBSOVO® and vorasidenib.”



"We are proud of our heritage in oncology and the novel therapies we have advanced for patients with hematologic malignancies and solid tumors, and we are pleased to have found an excellent home for our oncology portfolio in Servier, a successful, patient-focused, global pharmaceutical company," continued Dr. Fouse. "Servier is committed to the oncology patient community and to investing in our assets and our people. This transaction will allow the oncology portfolio to grow and thrive with Servier and will provide Agios with the resources required to optimize the development of our promising genetically defined disease therapies, ultimately enabling the greatest overall positive impact for patients."

"The strategic acquisition of Agios' oncology business, including its precision medicine portfolio and pipeline, is aligned with our ambition to become a recognized player in oncology and further supports our commitment to provide innovative treatments to cancer patients with unmet medical needs. It is a key step for the Servier Group as it will significantly strengthen our position in the U.S. and reinforce our R&D capabilities in oncology," stated Olivier Laureau, president of Servier. "We look forward to welcoming the experienced Agios oncology teams to Servier following the closing."

"Agios is a leader in the cellular metabolism space with a proven track record of discovering, developing and commercializing precision medicines," said David K. Lee, CEO, Servier Pharmaceuticals, the U.S. subsidiary of Servier. "The acquisition of Agios' oncology business, including highly experienced talent from research, development, technical operations and commercial functions, allows for an immediate expansion of our U.S. business into other hematologic malignancies and provides the potential for longer-term growth into the solid tumor space, thus ensuring that we can serve more patients living with unmet cancer needs than ever before."

Transaction Details

The transaction includes the transfer of Agios' oncology portfolio and associated employees, including its marketed medicine TIBSOVO® which is approved in the U.S. as monotherapy for the treatment of adults with IDH1-mutant relapsed or refractory acute myeloid leukemia (AML) and for adults with newly diagnosed IDH1-mutant AML who are ≥75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy. TIBSOVO® is also under investigation in two Phase 3 combination trials in newly diagnosed AML, and as a potential treatment for previously treated IDH1-mutant cholangiocarcinoma and IDH1-mutant myelodysplastic syndrome (MDS). Servier will also acquire Agios' co-commercialization responsibilities for Bristol Myers Squibb's IDHIFA® (enasidenib) and conduct certain clinical development activities within the IDHIFA® development program.

In addition, the transaction includes Agios' oncology pipeline and clinical programs, including vorasidenib, an investigational, brain-penetrant, dual inhibitor of mutant IDH1 and IDH2 which is currently being studied in the registration-enabling Phase 3 INDIGO study in patients with IDH-mutant low-grade glioma; AG-270, an investigational first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor being evaluated in combination with taxanes in patients with methylthioadenosine phosphorylase (MTAP)-deleted non-small cell lung cancer and pancreatic cancer; AG-636, a novel inhibitor of dihydroorotate dehydrogenase (DHODH); and Agios' oncology research programs.



All of Agios' U.S.-based employees who primarily support the oncology business will receive a comparable offer at Servier.

The transaction has been approved by the Board of Directors and is subject to approval by Agios shareholders and satisfaction of regulatory conditions. It is currently expected that the transaction will close in the second quarter of 2021.

Goldman Sachs & Co. LLC and Morgan Stanley & Co. LLC are serving as financial advisers to Agios, and Wachtell, Lipton, Rosen & Katz is serving as its legal adviser.

AgiOS' Genetically Defined Disease Portfolio

AgiOS' genetically defined disease portfolio is anchored by its lead clinical candidate, mitapivat, which the company believes is a potential blockbuster across three distinct hemolytic anemias. Agios is conducting two global, pivotal Phase 3 studies to evaluate mitapivat as a potential treatment for adults with pyruvate kinase (PK) deficiency; the company recently announced positive topline results from the ACTIVATE study and expects to report data from the ACTIVATE-T study in the first quarter of 2021. Agios anticipates filing for U.S. and EU regulatory approval in adults with PK deficiency in 2021, with a potential 2022 commercial launch in both geographies. Mitapivat is also being evaluated in a fully enrolled Phase 2 study in adults with non-transfusion-dependent a- or b-thalassemia, and as a potential treatment for sickle cell disease under a Cooperative Research and Development Agreement (CRADA) with the U.S. National Institutes of Health. In 2021, Agios expects to initiate global, pivotal Phase 3 studies in thalassemia, including both a- and b-thalassemia, as well as transfusion dependent and non-transfusion dependent patient populations, and in sickle cell disease. In addition, Agios intends to evaluate mitapivat in pediatric patients across all three diseases.

Beyond mitapivat, Agios is advancing a growing genetically defined disease pipeline based on its core expertise in cellular metabolism and pioneering leadership in PK activation. AG-946, a clinical-stage, next-generation oral activator of both wild-type and mutated pyruvate kinase R (PKR) enzymes, entered a first-in-human clinical study in the third quarter of 2020. Agios' late-stage research pipeline is evolving to include a rich and sustainable portfolio of genetically defined disease targets with clear disease area applications. These include hereditary and acquired anemias, myopathies, retinal diseases and diseases of inborn errors of metabolism such as aminoacidurias, aminoacidemias and others. As the company's research efforts continue to develop, Agios may pursue value-adding partnerships that may bring complementary expertise for certain disease areas.

Investor Webcast Information

AgiOS will host an investor webcast today at 8:00 a.m. ET to discuss today's announcement. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors section of Agios' website at www.agios.com. The archived webcast will be available on Agios' 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 rare genetic 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.

About Servier Group

Servier is a global pharmaceutical Group governed by a non-profit foundation, with its headquarters in France (Suresnes). With a strong international presence in 150 countries and a total revenue of 4.6 billion euros in 2019, Servier employs 22,000 people worldwide. Entirely independent, the Group invests on average 25% of its total revenue (excluding generics) every year in research and development and uses all its profits for its development. Corporate growth is driven by Servier's constant commitment in five areas of excellence: cardiovascular, immune-inflammatory, and neurodegenerative diseases, cancer and diabetes, as well as by its activities in high-quality generic drugs. Servier also offers eHealth solutions beyond drug development.

More information: www.servier.com

About Servier Pharmaceuticals

Servier Pharmaceuticals, LLC is a commercial-stage pharmaceuticals company with a passion for innovation and improving the lives of patients, their families and caregivers. In the United States, Servier Pharmaceuticals is committed to building a robust portfolio, starting with Oncology, with future growth driven by innovation in other areas of unmet medical need, leveraging Servier's global portfolio and seeking acquisitions, licensing deals and partnerships.

Servier Pharmaceuticals believes co-creation is fundamental to driving innovation and is actively building alliances that bring solutions to patients' lives and can accelerate access to therapies. We are building relationships with academia, venture capitalists, biotech and pharmaceutical peers and advocates with the aim of entering into mutually beneficial and complementary partnerships where each organization's skill sets are recognized and leveraged for the benefit of patients.

With our commercial expertise, global reach, scientific expertise and commitment to clinical excellence, Servier Pharmaceuticals is dedicated to bringing the promise of tomorrow to the patients that we serve.

Learn more at www.servier.us.



Cautionary Note Regarding Forward-Looking Statements

Certain statements contained in this communication may constitute 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 and Section 21E of the Securities Exchange Act of 1934. Forward-looking statements are based on our current plans and expectations and involve risks and uncertainties which are, in many instances, beyond our control, and which could cause actual results to differ materially from those included in or contemplated or implied by the forward-looking statements. Such risks and uncertainties include the following: (i) the occurrence of any event, change or other circumstance that could give rise to the termination of the purchase and sale agreement; (ii) 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; (iii) 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; (iv) risks associated with the disruption of management's attention from ongoing business operations due to the proposed transaction; (v) the ability to meet expectations regarding the timing and completion of the proposed transaction, including with respect to receipt of required regulatory approvals; (vi) 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; (vii) the uncertainty of the results and effectiveness of the use of proceeds from the proposed transaction; and (viii) other risks and uncertainties described in our reports and filings with the SEC, 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 and other subsequent periodic reports we file with the SEC, which are available at www.sec.gov and Agios' website at 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.

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 will file relevant materials with the U.S. Securities and Exchange Commission (the "SEC"), including Agios' proxy statement on Schedule 14A (the "Proxy Statement"). This communication is not a substitute for the Proxy Statement or any other document that Agios may file with the SEC or send to its stockholders in connection with the proposed transaction. BEFORE MAKING ANY VOTING DECISION, STOCKHOLDERS OF AGIOS ARE URGED TO READ ALL RELEVANT DOCUMENTS FILED WITH THE SEC, INCLUDING THE PROXY STATEMENT, WHEN THEY BECOME



AVAILABLE BECAUSE THEY 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's website, at www.agios.com. In addition, the documents (when available) may be obtained free of charge by accessing Agios's 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, will be contained in the Proxy Statement and other relevant materials to be filed with the SEC in respect of the proposed transaction when they become available.

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Contacts

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Director, Investor Relations
Holly.Manning@agios.com

Media:

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EMBRACING OUR PAST, REIMAGINING OUR FUTURE

December 21, 2020



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Forward Looking Statements

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As always,
we are driven
by our sense
of urgency to
help patients.



“On a bad day, it's like watching some electronic toy slowly lose the battery.”
—Tamara S., Minnesota



“The disease has affected my career. I spent 11 years to get a PhD in nutrition...My heart wants more but my body can't handle it.”

—**Tamara S., Minnesota**

Currently 50 years old. Diagnosed with PK deficiency at the age of 6.

[LEARN MORE AT KNOWPKDEFICIENCY.COM](https://www.knowpkdeficiency.com)

We are at an inflection point

For more than a decade, our mission has been to create **differentiated, small molecule medicines for patients**. Our successes have resulted in a wealth of promise and opportunity. We intend to leverage this position of strength to make the best choices for our future boldly and strategically. **The path ahead is shaped by three important decisions:**

1

**Move forward
with a singular
focus on
genetically
defined diseases**

2

**Maximize the
value and impact
of our oncology
portfolio for
patients and
stakeholders**

3

**Thoughtfully
pursue capital
markets
independence
while right-sizing
the company**



To maximize the value and promise of our diverse portfolio, we have made a deliberate choice of where to focus our efforts and investment

Placing our oncology portfolio in the hands of a dedicated partner allows us to maximize its patient impact.

We had early and unprecedented success with IDHIFA and TIBSOVO.

Investing in clinical development across both oncology and genetically defined diseases will not do justice to either portfolio.

A partner with significant resources and an oncology focus can maximize the patient impact of our oncology assets.

Expanding our genetically defined disease pipeline and mining the extensive, untapped potential in the PK platform provides a clear path to success.

As a potential blockbuster drug, mitapivat allows the business to thrive in the near- to mid-term.

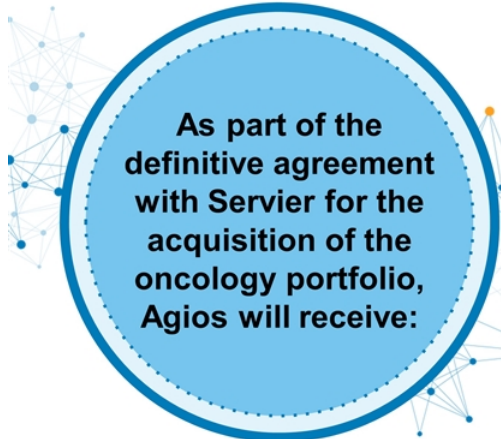
In the realm of genetically defined diseases, our expertise in cellular metabolism has the potential to drive greater differentiation and unlock a deeper pipeline of therapies and indications.

Pursuing this path taps into Agios' unique differentiators and core expertise to make a positive impact for patients.

By singularly focusing on genetically defined diseases and positioning our oncology portfolio for success with Servier, we can make the greatest impact.



Today we announce a deal with Servier that captures the full value* of the oncology portfolio to accelerate and expand our efforts in genetically defined diseases



As part of the definitive agreement with Servier for the acquisition of the oncology portfolio, Agios will receive:

- 1** Cash consideration of up to \$2B, including \$1.8B in upfront cash and a \$200M milestone upon FDA approval of vorasidenib**
- 2** 5% royalties on U.S. net sales of TIBSOVO from transaction close through loss of exclusivity
- 3** 15% royalties on U.S. net sales of vorasidenib from first commercial sale through loss of exclusivity

Agios plans to return at least \$1.2B to shareholders; residual proceeds will be retained to achieve capital market independence to fund the company through major catalysts and to profitability

*Risk adjusted

**FDA approval of Vorasidenib on or before January 1, 2027 with label permitting use as single agent in adjuvant setting for Grade 2 glioma with IDH1 or IDH2 mutation

These decisions are the result of a comprehensive strategic review of the company's business and a competitive sale process



We conducted a comprehensive strategic review of the company's assets led by our board of directors and management team, with assistance from independent financial advisors, aimed at maximizing the potential of the PK / cellular metabolism platform, achieving superior outcomes for patients, and delivering sustainable, long-term value to shareholders



We ran a broad, competitive process that included large biopharma, midsize biopharma with oncology growth strategies and regional biopharma with U.S. expansion strategies



The consideration to be received from Servier captures the full intrinsic value of our oncology business – the significant upfront cash proceeds de-risks the oncology portfolio while the regulatory milestone and royalties provide significant participation in the future success of vorasidenib and TIBSOVO®



Profile of the reimagined Agios will present a compelling investment opportunity

Transaction subject to customary regulatory approvals and a shareholder approval; closing expected in Q2

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- Focusing solely on genetically defined diseases will enable us to drive greater differentiation for Agios, unlock a deeper pipeline of therapies and indications at a more rapid pace that leverage our core expertise in cellular metabolism

- Will realign capital structure to reflect stage of maturity based on our genetically defined disease pipeline with a plan to return at least \$1.2B of the \$1.8B upfront proceeds

- Remaining proceeds together with current cash on hand expected to be sufficient to fund company through major catalysts and to cash flow profitability in 2025

- TIBSOVO® royalty and potential vorasidenib milestone and royalty provide meaningful participation in these opportunities and complementary sources of future cash flow



Singular focus in genetically defined diseases sets the stage for building long-term value


Transformative deal with Servier supports near-term priorities

Initiate pivotal development of mitapivat in thalassemia and sickle cell disease

File NDA for mitapivat in PK deficiency; prepare for launch

Determine next steps for AG-946 development based on healthy volunteer study

Advance next research program to IND


2025 & Beyond

Mitapivat approvals in 3 initial indications

Broad clinical pipeline of at least 5 molecules exploring at least 10 indications

Robust research pipeline poised to deliver a new IND every 12-24 months

Cash-flow positive





AFFIRMING OUR LEGACY

Embracing who we are and what we built

Our journey has delivered great successes and an expanded focus...

2009

Published groundbreaking research in *Nature*, re: role of the IDH1 mutation in cancer genesis

2013

Initiated first clinical study of enasidenib in patients with IDH2 mutant hematologic malignancies

Completed initial public offering on NASDAQ

Expanded research focus beyond oncology into genetically defined diseases.

2015

Initiated Phase 1 study of vorasidenib in patients with advanced solid tumors and IDH mutation

Initiated a Phase 2 study of mitipivat in patients with pyruvate kinase (PK) deficiency

2019

Received FDA approval of TIBSOVO® in frontline AML

Achieved proof-of-concept for mitipivat in thalassemia

Published clinical data of mitipivat in PK deficiency in NEJM

CANCER
METABOLISM

CANCER
METABOLISM + RARE GENETIC
DISEASES

CANCER
METABOLISM + RARE GENETIC
DISEASES + METABOLIC
IMMUNO-
ONCOLOGY



2008

Founded Agios to unlock a new field of discovery in cellular metabolism

2010

Entered into strategic collaboration with Celgene to discover and develop novel cellular metabolism-related therapies

2014

Initiated first clinical study of ivosidenib in patients with IDH1 mutant hematologic malignancies

2018

Received FDA approval of TIBSOVO® (ivosidenib tablets), which is wholly owned by Agios, in R/R AML

2020

Achieved proof-of-concept for mitipivat in sickle cell disease

Initiated first clinical study of next-generation PKR activator AG-946

Announced positive data from ACTIVATE Phase 3 trial of mitipivat in adults with PK deficiency who are not regularly transfused



...supported by incredible productivity, from early research to commercially available medicines



INVESTIGATIONAL NEW
DRUG CANDIDATES



PEER-REVIEWED
PUBLICATIONS



RESEARCH
PROGRAMS



PATIENTS TREATED
BY OUR MEDICINES



MEDICINES
APPROVED



ADDITIONAL MOLECULES IN
CLINICAL DEVELOPMENT





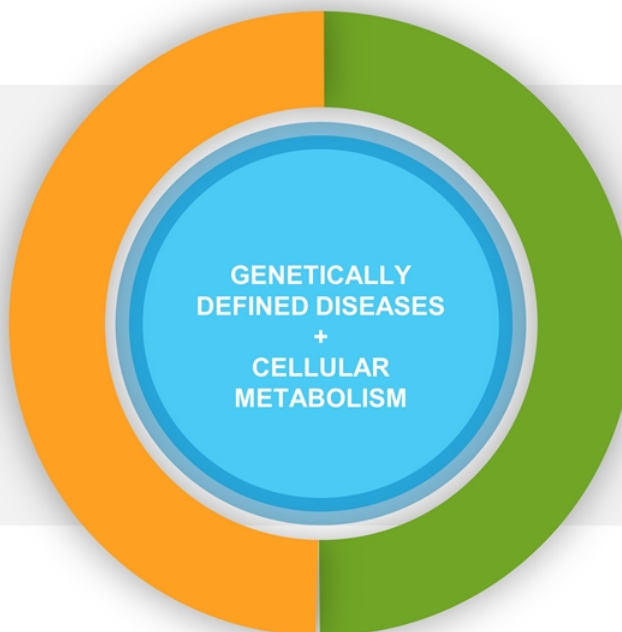
ACCELERATING OUR IMPACT

Adapting to preserve and expand the difference we make

Our refocused therapeutic area is defined by a combination of our most differentiated foundational elements

CELLULAR METABOLISM

Cellular metabolism is a central part of our heritage and scientific competency



GENETICALLY DEFINED DISEASE

Genetically defined disease is a broad umbrella that encompasses both rare and more common diseases



We are the pioneering leaders in PKR activation

6 YEARS

STUDYING PKR ACTIVATION IN THE CLINIC

~190

PATIENTS
TREATED

17

CLINICAL
TRIALS

15

JOURNAL ARTICLES
PUBLISHED

17

MEDICAL/SCIENTIFIC
COLLABORATIONS

3

DISEASES WITH
POC ACHIEVED

+ A LOT
OF FIRSTS:

1st GLOBAL PK
DEFICIENCY
REGISTRY

1st INTERNATIONAL PK
DEFICIENCY
ADVOCACY COUNCIL

1st HEMOLYTIC
ANEMIA ADVOCACY
COALITION BUILDING

1st POSITIVE PHASE
3 READOUT IN PK
DEFICIENCY



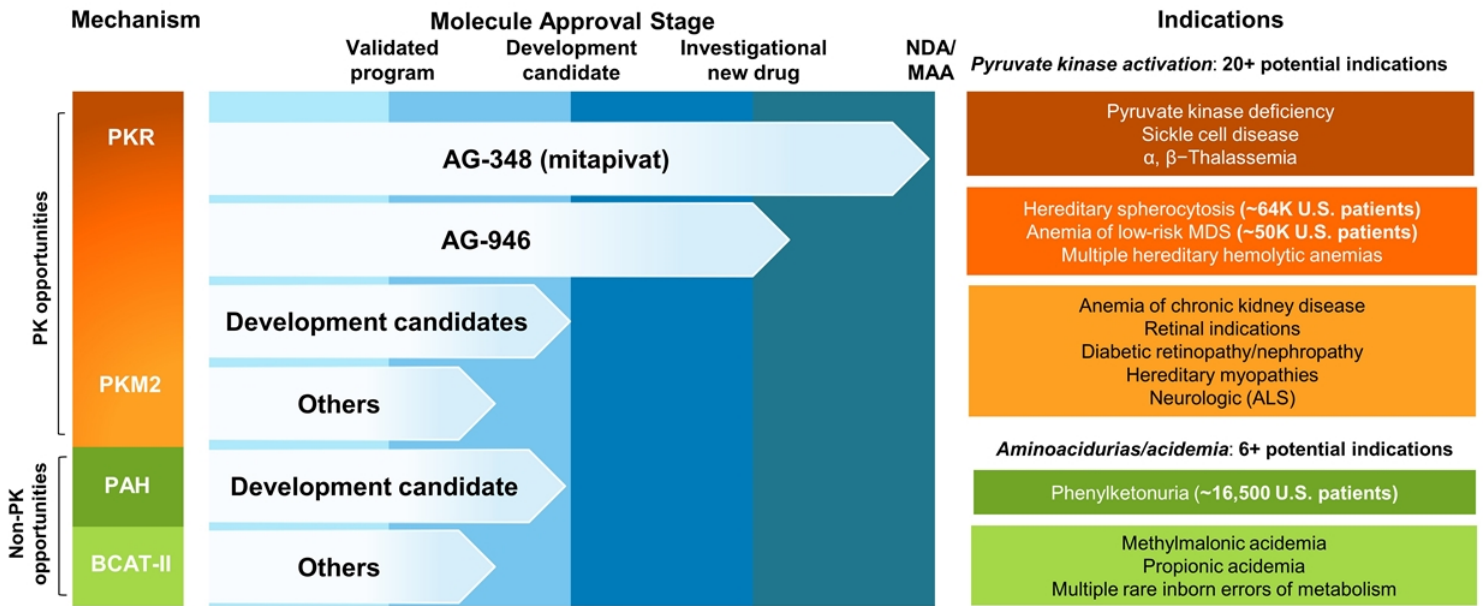
In mitapivat, we are building a robust pipeline with the ability to rapidly expand to three indications

Mitapivat Pipeline Overview

Early Stage Clinical	Late Stage Clinical	Regulatory Submission	Near-Term Milestones	Expected Approval	
Non-transfusion Dependent (NTD) Adult PK Deficiency (ACTIVATE)			Positive topline data announced in Dec. 2020	2022	~3-8K PATIENTS IN U.S. & EU5
Transfusion Dependent Adult PK Deficiency (ACTIVATE-T)			Topline data expected in Q1 2021	2022	Pyruvate Kinase Deficiency
Sickle Cell Disease			Finalize pivotal plan in 1H 2021; Initiate pivotal plan in 2021	TBD	~18-23K PATIENTS IN U.S. & EU5
Non-transfusion Dependent Adult Thalassemia ¹			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2021	TBD	β- and α-Thalassemia
Transfusion Dependent Adult Thalassemia ¹			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2021	TBD	
Pediatric PK Deficiency			Finalized pivotal plan in Dec. 2020	TBD	~120-135K PATIENTS IN U.S. & EU5
Pediatric Thalassemia			TBD	TBD	Sickle Cell Disease
Pediatric Sickle Cell Disease			TBD	TBD	



Significant opportunities exist beyond our initial pipeline focus





ACHIEVING OUR POTENTIAL

Taking action to realize our goals and opportunity



PATIENTS

With a clear, singular focus on genetically defined diseases, we will have the ability to make a difference for more patients by:

- Shifting existing resources to execute on our PKD launch and rapidly advance our thalassemia and sickle cell disease programs
- Evaluating opportunities to broaden our PKR activator franchise and expand into non-PKR modalities
- Focusing our energy on the promising targets within our research organization



EMPLOYEES

The reimagined Agios will be a best-in-class organization with a clear vision and focus on genetically defined diseases providing:

- An opportunity to bring three mitapivat indications to market in four years
- The ability to work on a robust and exciting research pipeline
- The fulfillment of experiencing the full cycle of development for a greater number of therapies
- A chance to amplify the best of our existing culture with new opportunities to grow and make a difference



SHAREHOLDERS

Focusing solely on genetically defined diseases offers superior long term shareholder value creation driven by:

- A clearer path to sustained growth and profitability
- Significant upside potential as mitapivat and PK activation opportunities play out
- The return of at least \$1.2B to shareholders to realign our capital structure
- Capital markets independence



AGIOS TOMORROW

Focused Innovation.
Ambitious Development.
Increased Patient Impact.

BY LEVERAGING

Culture of continuous development and patient-first orientation

Deep understanding of disease biology and expertise in cellular metabolism

Emphasis on translational research, starting in early stage discovery

Proven success in drug discovery, development and commercialization

Focus on Genetically Defined Diseases

WE CAN

Continue to deliver groundbreaking science with an energized focus and mission and funding to adequately support and develop the full potential of our research

Fully develop potential for PK franchise by commercializing at least 3 indications by 2025 that represent a market of +140K patients and growing our clinical pipeline

Strengthen the business through financial markets independence, growing our U.S. commercial infrastructure and exploring options outside of the U.S.

Transaction Highlights

Accelerate and Expand the Difference We Make for Patients with Genetically Defined Diseases



- Facilitates focus as a genetically defined disease company
 - Realize the full potential of mitapivat as a potential blockbuster
 - Accelerate the emerging potential of our PK activation program and expand the pipeline
- Further upside through upcoming near-term catalysts

Maximize Value of the Oncology Portfolio for Patients and Shareholders



- Definitive agreement with Servier for the acquisition of the oncology portfolio maximizes value of these programs and positions them for success
- **Agius will receive:**
 - Cash consideration of up to \$2B
 - 5% royalties on U.S. net sales of TIBSOVO
 - 15% royalties on U.S. net sales of vorasidenib

Realign Capital Structure and Achieve Financial Independence



- Intend to return at least \$1.2B of the \$1.8B upfront proceeds to shareholders
- Residual proceeds will be retained to achieve capital market independence
- Transaction subject to customary regulatory approvals and a shareholder vote
- Closing expected in Q2

²² FDA approval of Vorasidenib on or before January 1, 2027 with label permitting use as single agent in adjuvant setting for Grade 2 glioma with IDH1 or IDH2 mutation





THANK YOU