agios

EMBRACING OUR PAST, REIMAGINING OUR FUTURE

39th Annual J.P. Morgan Healthcare Conference
January 11, 2021

Important Information for Investors and Stockholders

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.

Forward Looking Statements

This communication 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; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2021 and strategic vision for 2025; 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 communication 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. Any forward-looking statements contained in this communication 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.

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

Shirley, Acute Myeloid Leukemia

World Cholangiocarcinoma Day Panel of Patients & Advocates

Tamara, Pyruvate Kinase Deficiency

Cass, Sickle Cell Disease

We are at an inflection point

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

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

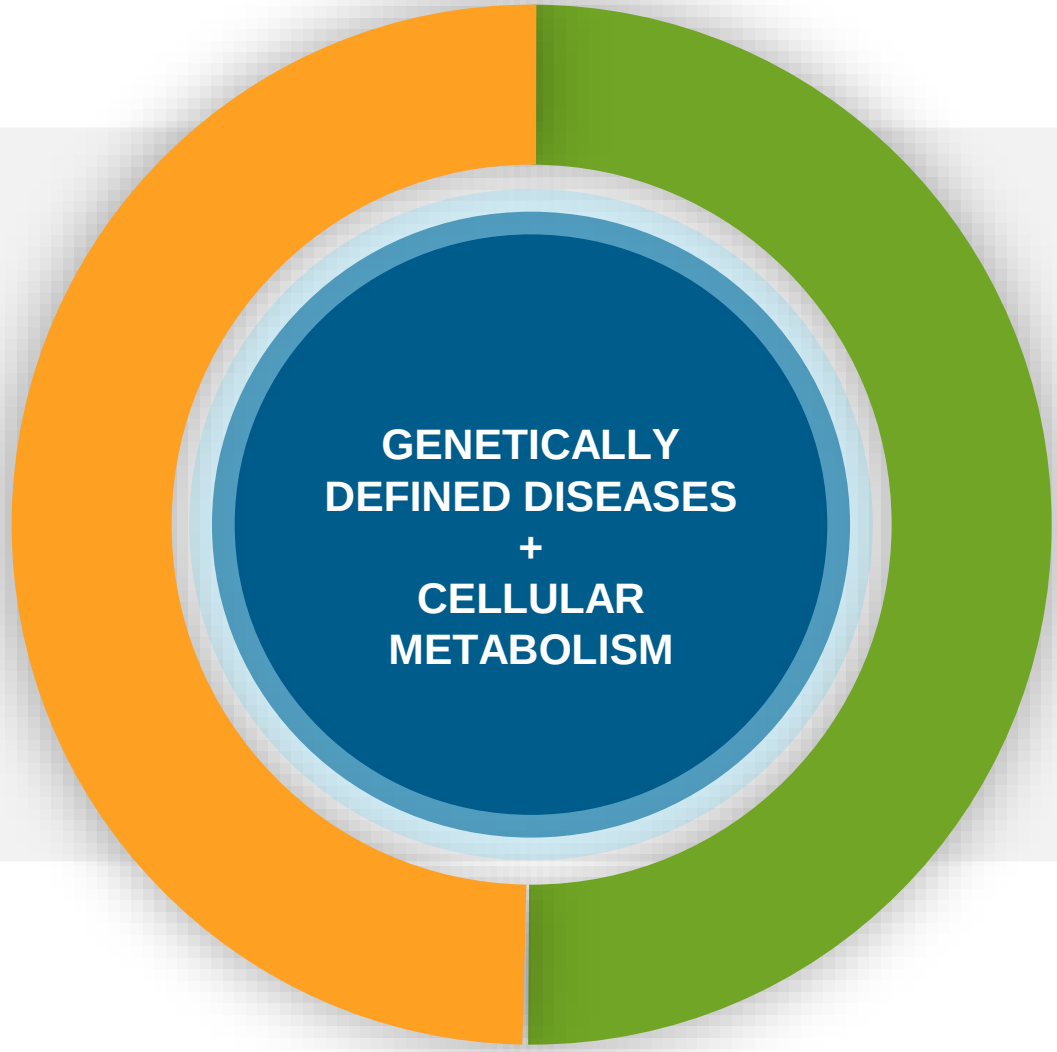
Selling our oncology portfolio to a capable and committed buyer allows us to maximize its patient impact.

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

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

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**

SERVIER TRANSACTION

The Servier transaction is the result of a comprehensive strategic review of our 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 our potential for 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®

The recently announced deal with Servier captures the full value* of the oncology portfolio facilitating the acceleration of 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 markets 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*

The reimagined Agios presents a compelling investment opportunity

- 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; resulting in a share count reduction of approximately 25-35%*

- Remaining proceeds together with our YE 2020 cash balance of $670.5M 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

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

**Assumes volume-weighted average repurchase price between $50-70*

Our plan accelerates and increases the impact we can make for patients, our employees and shareholders

PATIENTS

We will have the ability to make a difference for 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
- Placing our oncology portfolio with Servier, a company committed to investing in expanded indications and global reach for our programs

EMPLOYEES

We 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 by 2025
- The ability to work on a robust and exciting research pipeline
- A chance to amplify the best of our existing culture with new opportunities to grow and make a difference

SHAREHOLDERS

Narrowed focus 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

REIMAGINING OUR FUTURE

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

- **Deliver groundbreaking science with an energized focus and mission**
- **Fully develop potential for PK franchise and grow our clinical pipeline**
- **Strengthen the business**

We are the pioneering leaders in PKR activation

6 YEARS

STUDYING PKR ACTIVATION IN THE CLINIC

~190	17	15	17	3
PATIENTS TREATED	CLINICAL TRIALS	JOURNAL ARTICLES PUBLISHED	MEDICAL/SCIENTIFIC COLLABORATIONS	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
Non-transfusion Dependent (NTD) Adult PK Deficiency (ACTIVATE)		NDA filing in Q2; MAA filing in 2H 2021	Positive topline data from ACTIVATE announced in Dec.
Transfusion Dependent Adult PK Deficiency (ACTIVATE-T)			Topline data expected in Q1 2021
Sickle Cell Disease			Finalize pivotal plan in 1H 2021; Initiate pivotal plan in 2021
Non-transfusion Dependent Adult Thalassemia (ENERGIZE)			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2H 2021
Transfusion Dependent Adult Thalassemia (ENERGIZE-T)			Finalized pivotal plan in Dec. 2020; Initiate pivotal study in 2H 2021
Pediatric PK Deficiency			Finalized pivotal plan in Dec. 2020
Pediatric Thalassemia			Planning in process
Pediatric Sickle Cell Disease			Planning in process

~3-8K
PATIENTS IN U.S. & EU5

Pyruvate Kinase Deficiency

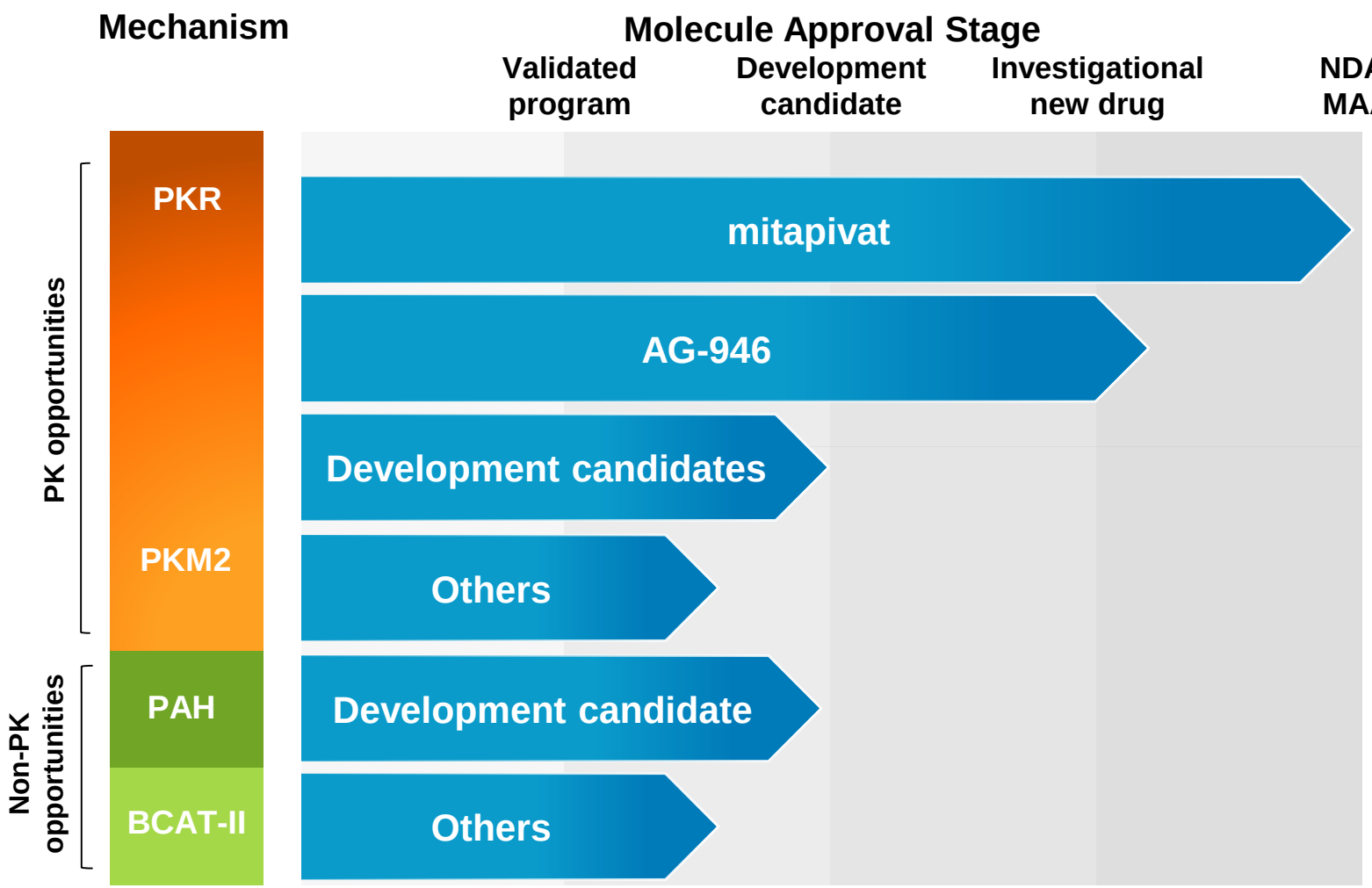
~18-23K
PATIENTS IN U.S. & EU5

β- and α-Thalassemia

~120-135K
PATIENTS IN U.S. & EU5

Sickle Cell Disease

Significant opportunities exist beyond our initial pipeline focus



Indications

***Pyruvate kinase activation*: 20+ potential indications**

- Pyruvate kinase deficiency
- Sickle cell disease
- α, β−Thalassemia

- Hereditary spherocytosis **(~64K U.S. patients)**
- Anemia of low-risk MDS **(~50K U.S. patients)**
- Multiple hereditary hemolytic anemias

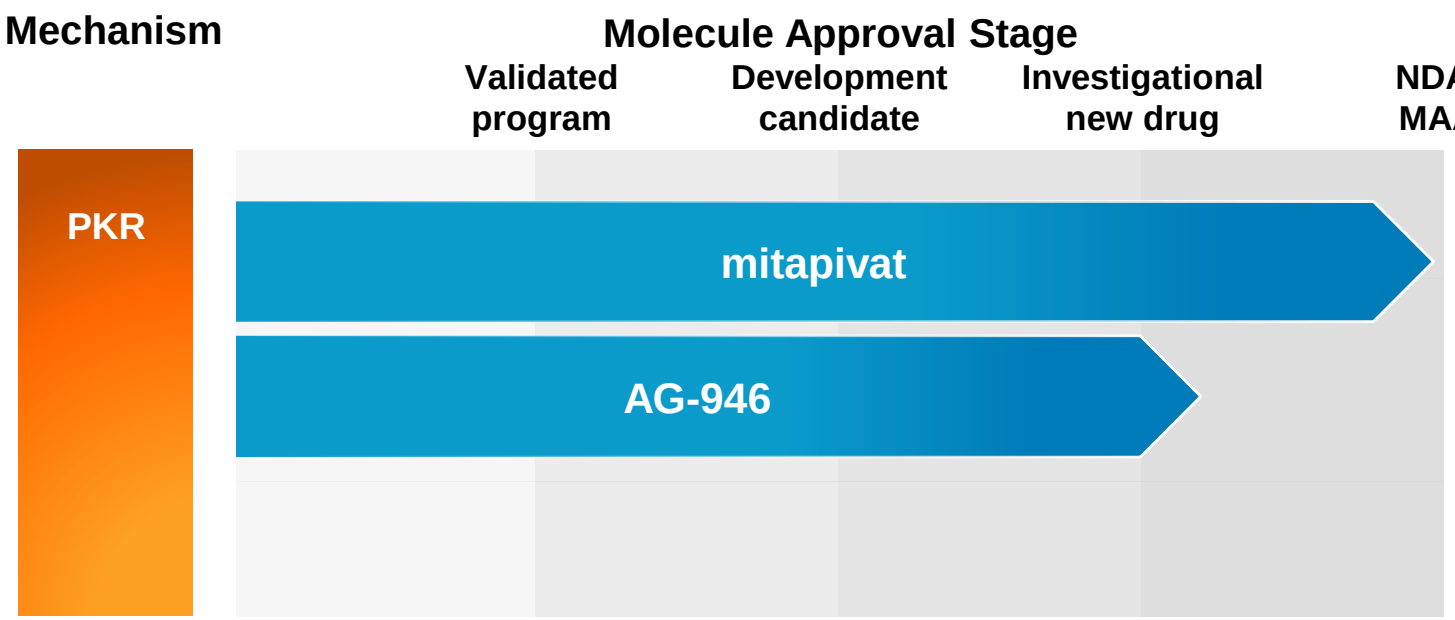
- Anemia of chronic kidney disease
- Retinal indications
- Diabetic retinopathy/nephropathy
- Hereditary myopathies
- Neurologic (ALS)

***Aminoacidurias/acidemia*: 6+ potential indications**

- Phenylketonuria (**~16,500 U.S. patients)**

- Methylmalonic acidemia
- Propionic acidemia
- Multiple rare inborn errors of metabolism

Significant opportunities exist beyond our initial pipeline focus



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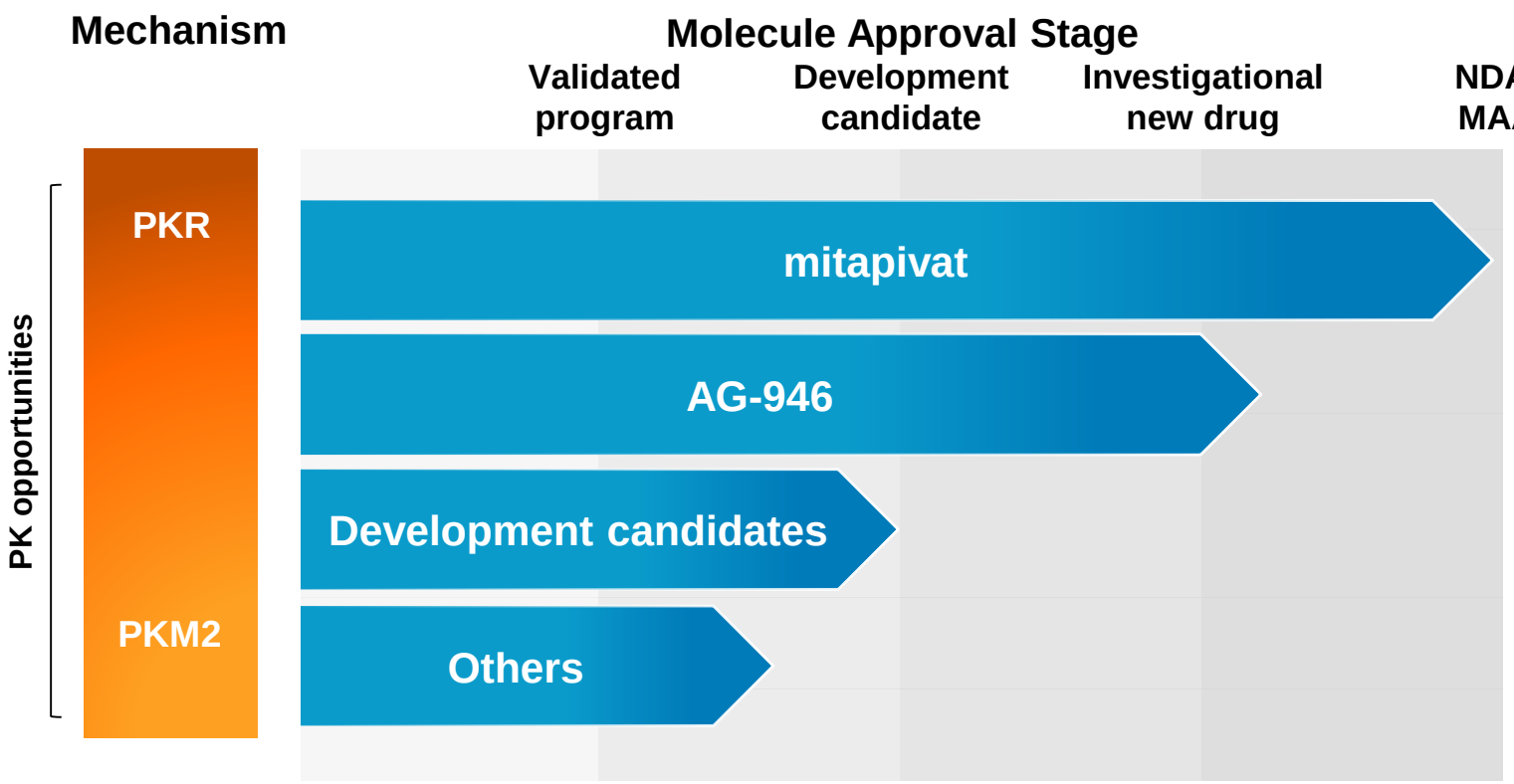
Data from the SAD/MAD cohorts for the AG-946 healthy volunteer study to be submitted for presentation by YE 2021

Significant opportunities exist beyond our initial pipeline focus



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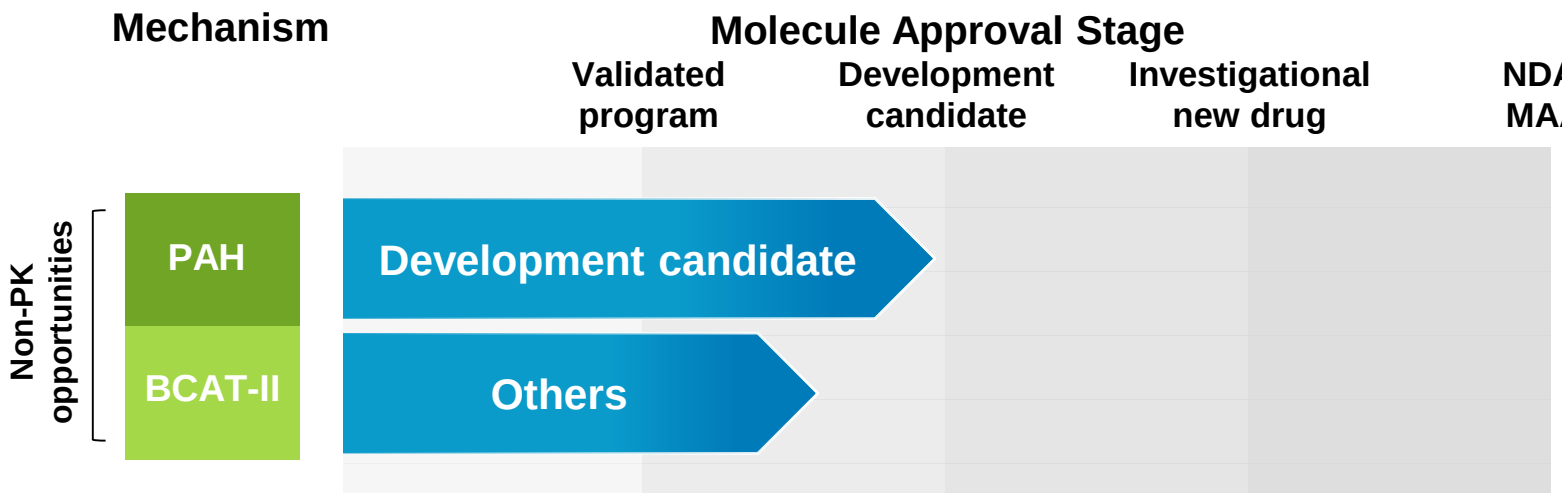
Research efforts to prioritize new PKR and PKM2 indications for clinical development currently in process; development decisions to be made in 2021

Significant opportunities exist beyond our initial pipeline focus



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Development candidate declared and IND enabling activities for the PAH program ongoing

Lead optimization in process for BCAT-II program

Anticipated 2021 key milestones

GDD PROGRAM MILESTONES

- Submit NDA in the U.S. for mitapivat in adults with PK deficiency in Q2
- Submit MAA in the EU for mitapivat in adults with PK deficiency in mid-2021
- Initiate two Phase 3 studies of mitapivat – ENERGIZE-T and ENERGIZE – in regularly transfused and not regularly transfused thalassemia in 2H 2021
- Provide an overview of our pivotal program for mitapivat in sickle cell disease in 1H and initiate pivotal program in 2021
- Prioritize new PKR and PKM2 indications for clinical development in 2021

GDD DATA PRESENTATIONS

- Report topline data from the ACTIVATE-T study of mitapivat in regularly transfused PK deficiency in Q1
- Submit data from the mitapivat ACTIVATE and ACTIVATE-T studies for presentation at EHA
- Submit data from the mitapivat thalassemia Phase 2 study for presentation at EHA
- Submit data from ongoing clinical trials of mitapivat in sickle cell disease for presentation at medical meetings throughout 2021
- Submit data from the SAD/MAD cohorts of the AG-946 healthy volunteer study for presentation at a medical meeting by YE

CORPORATE

- Close the sale of the oncology portfolio to Servier in Q2
- Commence the return of capital to shareholders post-close in Q2

NEAR-TERM ONCOLOGY MILESTONES & DATA PRESENATIONS

- Achieve full-year revenue for TIBSOVO® of $160-170M
- Present mature OS for ClarIDHy at ASCO GI on Jan. 17
- Submit sNDA for TIBSOVO in previously treated cholangiocarcinoma in Q1
- Complete enrollment in AGILE by YE
- Complete enrollment in the MDS cohort of the Phase 1 study by YE

AGIOS 2025 VISION:

Focused Innovation. Ambitious Development.
Transformative Treatments for Patients with Genetically Defined Diseases.

MITAPIVAT APPROVALS IN 3 INITIAL INDICATIONS

5+ MOLECULES EXPLORING 10+ INDICATIONS

PIPELINE POISED TO DELIVER NEW IND EVERY 12-24 MONTHS

CASH FLOW POSITIVE

THANK YOU