

Q1 2022 Financial Results

May 5, 2022

Agios conference call participants

TOPIC	PARTICIPANT	
Introductions	Holly Manning, Senior Director of Investor Relations	
Business Update	Jackie Fouse, Ph.D., Chief Executive Officer	
Clinical Development Update	Sarah Gheuens, M.D., Ph.D., Chief Medical Officer	
Commercial Update	Richa Poddar, Chief Commercial Officer	
First Quarter 2022 Financial Results	Jonathan Biller, Chief Financial Officer, Head of Corporate Affairs	
Q&A	Bruce Car, Ph.D., Chief Scientific Officer	



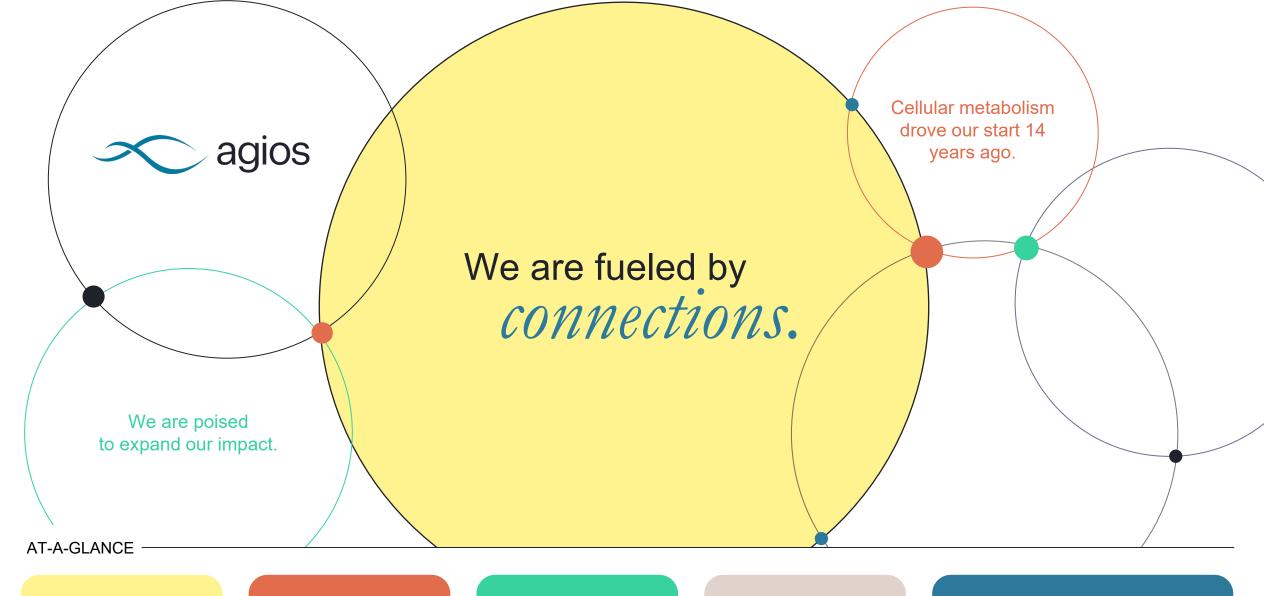
Forward-looking statements

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, including PYRUKYND® (mitapivat) and AG-946; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2022; its financial guidance regarding the period in which it will have capital available to fund its operations; 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 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: 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, 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 forwardlooking 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.









FOUNDED 2008

IPO *July 2013*

1ST APPROVED THERAPIES
2017 & 2018

1ST GENETICALLY DEFINED DISEASE APPROVAL 2022

HEADQUARTERS

Cambridge, Mass.

01

We intentionally cultivate internal and external connections

The future of Agios is driven by innovation & impact

02

We have a strong balance sheet and are well capitalized to execute on our near- and long-term business strategy

03

Our unmatched expertise in cellular metabolism has yielded a pipeline with the depth, breadth and optionality to deliver sustained productivity

04

We pioneered PK activation clinical development with a differentiated approach to global development and community partnerships

05

We are ready to maximize the success of our first genetically defined disease product launch in a serious disease with no approved therapies









Our 7+ years of clinical experience with PYRUKYND® continues to validate the potential of PK activation across therapeutic areas



We pioneered PK activation clinical development with a differentiated approach to global development and community partnerships

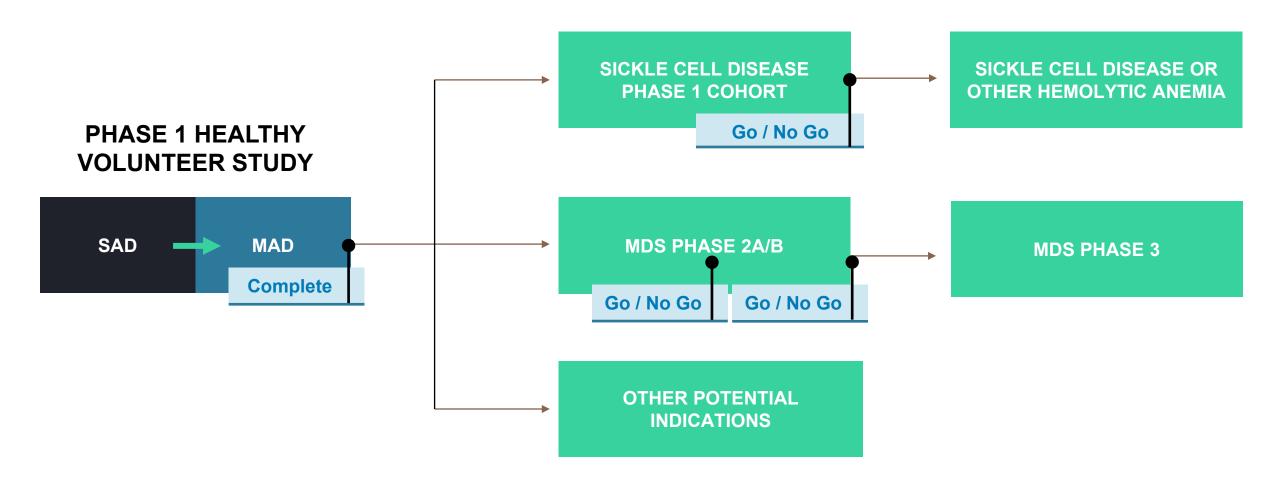
Long-term extension data show durability of hemoglobin response, transfusion burden reduction, and improvement in ineffective erythropoiesis and iron overload in adults with PK deficiency

Extension data for PYRUKYND® highlight long-term safety profile and durable improvement in hemoglobin and markers of hemolysis in thalassemia patients for up to 72 weeks

Data from *investigator-led studies* of PYRUKYND® in adults with sickle cell disease underscore potential of mitapivat to improve clinically meaningful outcomes for patients, including anemia, hemolysis and sickling parameters

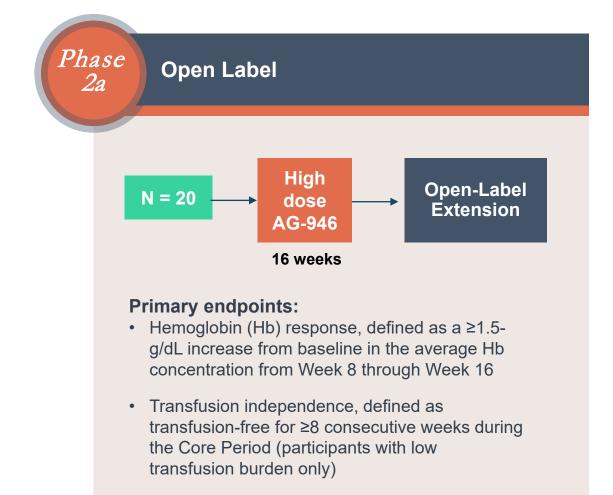


AG-946 clinical development plan: Ability to pursue multiple paths in parallel if data support advancement



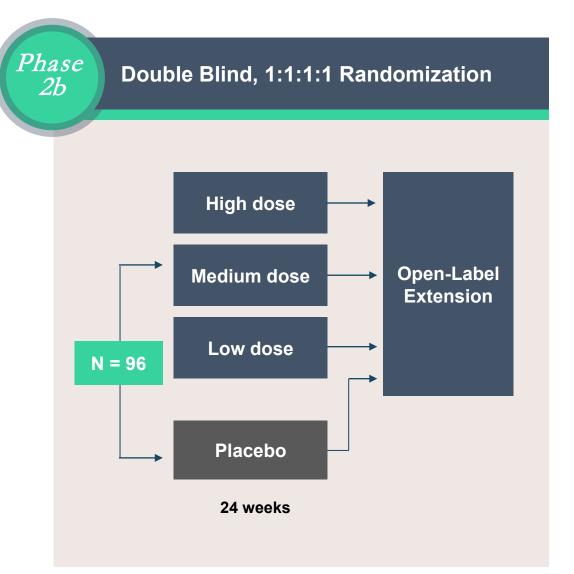


AG-946 MDS clinical development plan: Seamless Phase 2a proof-of-concept + Phase 2b



Secondary endpoints: safety, additional

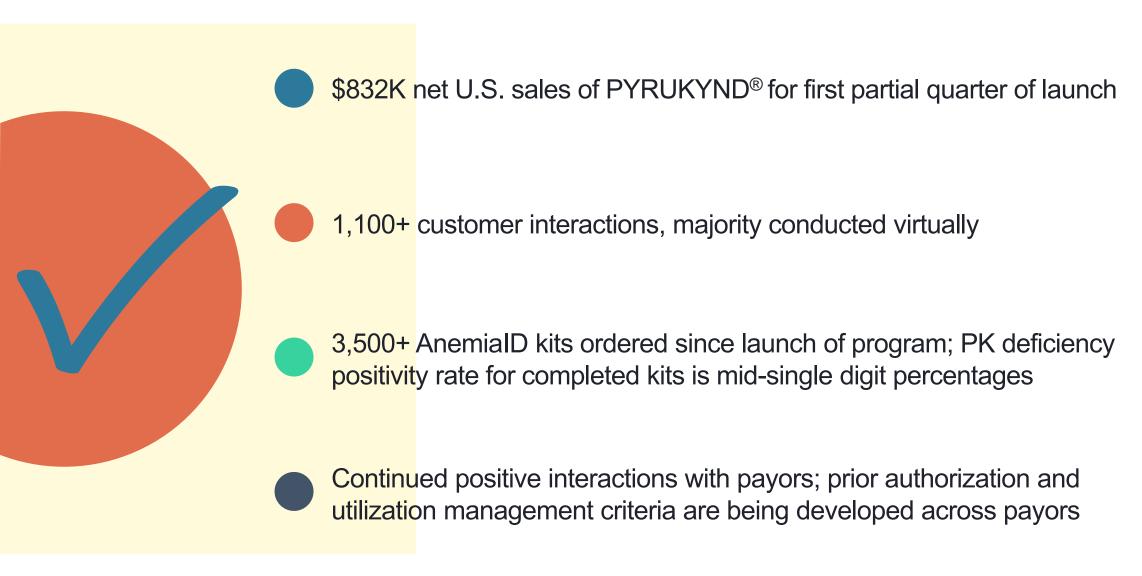
measures of anemia, PK and PD biomarkers





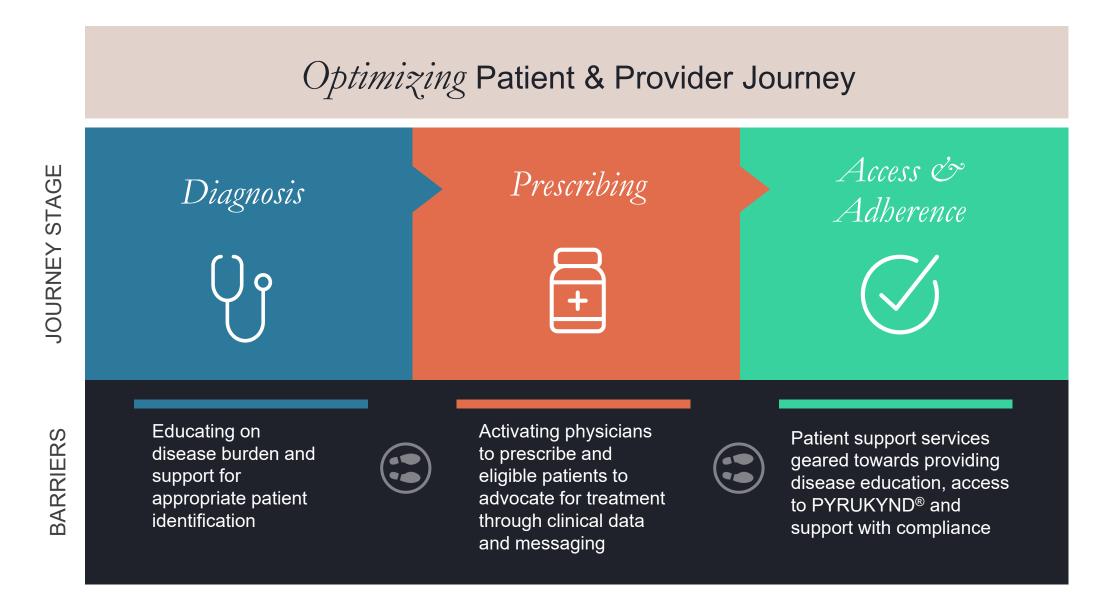


PYRUKYND® Q1 2022 performance



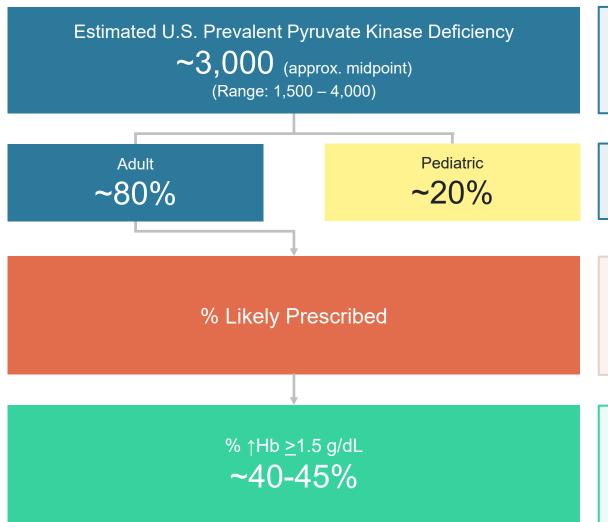


Commercial strategy to inform launch success





Understanding U.S. commercial opportunity: State of play today



Estimate ~30%+ diagnosed at time of launch

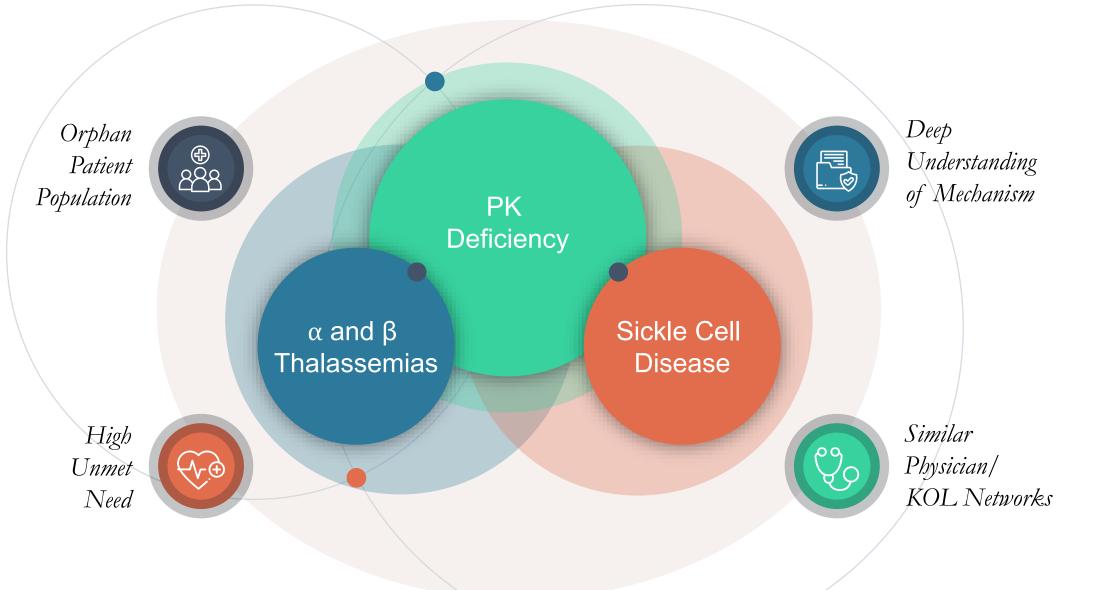
- 30% patients have Hb ≥10 g/dL (Natural History Study)
- 9-15% double non-missense (Peak Registry)

- Expect eligible patients who initiate treatment to try for
 ~3-6 months before clinical assessment of response
- Payors may want to recertify patients at 3-6 months

- An additional 5% of patients will achieve improvement between 1.0 to 1.5 g/dL
- Consider likely adherence to chronic treatment



Research, clinical and commercial experience with PK deficiency positions Agios well for thalassemias and sickle cell disease







First quarter 2022 financial results¹

Statement of Operations	Three Months Ended 3/31/22	Three Months Ended 3/31/21
PYRUKYND® Revenue	\$832K	
Cost of Sales	339K	
Research & Development Expense	70.1M	\$57.7M
Selling, General & Administrative Expense	31.5M	33.6M
Royalty Income from Gain on Sale of Oncology Business (TIBSOVO® Royalties)	2.7M	

Balance Sheet	3/31/22	3/31/21
Cash, Cash Equivalents and Marketable Securities	\$1.2B	\$2.4B



Anticipated 2022 key milestones & priorities

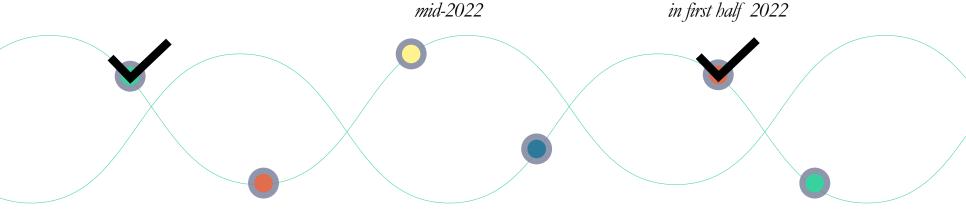
FDA approval and launch of mitapivat in adults with PK deficiency

Initiate two Phase 3 studies of mitapivat in pediatric patients with PK deficiency

Initiate the SCD cohort of the AG-946 Phase 1 study Enroll a meaningful portion of patients in the ENERGIZE and ENERGIZE-T thalassemia studies

in first half 2022

by year-end



Potential EMA approval of mitapivat in adults with PK deficiency

by year-end

Complete enrollment in the Phase 2 portion of the RISE UP study of mitapivat in adults with SCD

by year-end

Initiate the Phase 2a study of AG-946 in L-IR MDS

by year-end

Continue to publish clinical and translational data supporting the utility of PK activators across key disease areas and elucidating the burden of disease for PK deficiency, thalassemia and sickle cell





Q&A