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): January 8, 2023

Agios Pharmaceuticals, Inc.
(Exact Name of Registrant as Specified in Charter)

Delaware	**001-36014**	**26-0662915**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

88 Sidney Street, Cambridge, MA	**02139**
(Address of Principal Executive Offices)	**(Zip Code)**

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

(Former Name or Former Address, if Changed Since Last Report)

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 (*see* General Instruction A.2. below):

☐ 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))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading symbol(s)**	**Name of each exchange on which registered**
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.

On January 8, 2023, Agios Pharmaceuticals, Inc. (the "Company") issued a press release outlining its anticipated 2023 milestones and significant value-driving catalysts through 2026, which will be discussed at the Company's presentation at the 41st Annual J.P. Morgan Healthcare Conference on January 11, 2023. The full text of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference. The slides to be presented by the Company at the 41st Annual J.P. Morgan Healthcare Conference are furnished as Exhibit 99.2 to this Current Report on Form 8-K and are incorporated herein by reference.

The information in this Item 7.01 (including Exhibit 99.1 and Exhibit 99.2) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press release dated on January 8, 2023
99.2	Presentation at the 41st Annual J.P. Morgan Healthcare Conference
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

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: January 9, 2023

By: /s/ Brian Goff
Brian Goff
Chief Executive Officer

Agios Unveils 2023-2026 Value-driving Catalysts Enabling Company's Vision to Transform Patient Outcomes in Rare Diseases

– Consistent and compelling clinical data across rare hematological diseases support five ongoing pivotal trials in thalassemia, sickle cell disease and pediatric pyruvate kinase (PK) deficiency –

– Driving toward two additional PYRUKYND® indications, with potential FDA approvals in thalassemia in 2025 and sickle cell disease in 2026 –

– Strong cash position expected to enable completion of ongoing programs and pipeline expansion to cash-flow positivity –

CAMBRIDGE, Mass., January 8, 2023 – Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today announced its anticipated 2023 milestones and significant value-driving catalysts through 2026 that support the company's mission to transform patient outcomes in rare diseases. Agios will present at the 41st Annual J.P. Morgan Healthcare Conference on Wednesday, January 11, 2023, at 7:30 a.m. PT, and a live webcast will be available at investor.agios.com.

"Agios is poised for significant growth with the potential for approvals in two additional PYRUKYND® indications by 2026, and is well capitalized to advance its robust existing clinical pipeline and expand its portfolio within our core areas of expertise," said Brian Goff, chief executive officer at Agios. "As the pioneering leader in PK activation with more than seven years of clinical experience with PYRUKYND®, we have generated an impressive body of consistent and compelling data across rare hematological diseases with shared underlying pathophysiology that further builds confidence in our five ongoing pivotal clinical trials in thalassemia, sickle cell disease and pediatric PK deficiency. Furthermore, we are executing our first rare disease U.S. product launch with PYRUKYND® in adult PK deficiency, providing the first disease-modifying therapy for this patient community that previously had no treatment options and building the capabilities to set us up for success with our expected launches in meaningfully larger patient populations."

Recent Highlights

- *Adult PK Deficiency:* Received marketing authorization for PYRUKYND® in adults with PK deficiency in the EU and Great Britain
- *Thalassemia:* Enrolled approximately half of patients in the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® in not regularly transfused and regularly transfused adults with thalassemia, respectively
- *Sickle Cell Disease:* Closed screening in the Phase 2 portion of the RISE UP study of PYRUKYND® in adults with sickle cell disease in December and expect to complete enrollment in January
- *Data Presentations:* Presented broad set of clinical and translational data at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition, including long-term PYRUKYND® data in adults with non-transfusion-dependent thalassemia and in adults with PK deficiency

- *Leadership:* Appointed Tsveta Milanova to the role of chief commercial officer, bringing two decades of experience in rare disease commercial strategy and global market access

Anticipated 2023 Milestones

- *Thalassemia:* Complete enrollment of the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® by mid-year
- *Pediatric PK Deficiency:* Enroll at least half of patients in the Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® by year-end
- *Sickle Cell Disease:* Announce data readout from Phase 2 portion of RISE UP study of PYRUKUND® and go/no-go to Phase 3 decision by mid-year
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):* Complete enrollment of Phase 2a study of novel PK activator AG-946 by year-end
- *Earlier-stage Pipeline:* File investigational new drug (IND) application for PAH stabilizer for the treatment of phenylketonuria (PKU) by year-end

Agios 2026 Vision

By 2026, Agios' vision is to establish a classical hematology franchise with PYRUKYND® approvals across PK deficiency, thalassemia and sickle cell disease; expand its portfolio by advancing AG-946 and the preclinical pipeline as well as through disciplined business development aligned with the company's core therapeutic focus areas and capabilities; and achieve cash-flow positivity. Agios provided a roadmap of additional significant potential catalysts between 2024 and 2026 to enable the realization of this vision, as follows:

2024

- Data readout from Phase 3 ENERGIZE study of PYRUKYND® in adults with non-transfusion-dependent thalassemia (first half of 2024)
- Data readout from Phase 3 ENERGIZE-T study of PYRUKYND® in adults with transfusion-dependent thalassemia (second half of 2024)
- Data readout from Phase 2a study of AG-946 in LR-MDS

2025

- Potential FDA approval for PYRUKYND® in thalassemia
- Data readout from Phase 3 portion of RISE UP study of PYRUKYND® in sickle cell disease, pending go/no-go decision in 2023
- Data readouts from Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® in pediatric PK deficiency

2026

- Potential FDA approval for PYRUKYND® in sickle cell disease
- Potential FDA approval for PYRUKYND® in pediatric PK deficiency
- Achieve cash-flow positivity

Presentation at 41st Annual J.P. Morgan Healthcare Conference

Agios will webcast its corporate presentation from the 41st Annual J.P. Morgan Healthcare Conference on Wednesday, January 11 at 7:30 a.m. PT. A live webcast of the presentation can be accessed under "Events & Presentations" in the Investors section of the company's website at agios.com. A replay of the webcast will be archived on the Agios website for at least two weeks following the presentation.

About Agios

Agios is a biopharmaceutical company that is fueled by connections. The Agios team cultivates strong bonds with patient communities, healthcare professionals, partners and colleagues to discover, develop and deliver therapies for rare diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has multiple investigational therapies in preclinical development and deep scientific expertise in classical hematology. 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 the potential benefits of PYRUKYND® (mitapivat), AG-946 and its PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and its PAH stabilizer; Agios' strategic vision and goals, including its key milestones for 2023 and potential catalysts through 2026; 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. For example, there can be no guarantee that any product candidate Agios is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. There can be no guarantee that any positive developments in Agios' business will result in stock price appreciation. 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 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; 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; 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 maintain key collaborations; 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; 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. Any forward-looking statements contained in this press release 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.

Contacts

Investors:
Adam Bero, Ph.D.
Kendall Investor Relations
abero@kendallir.com

Media:
Jessica Rennekamp
Senior Director, Corporate Communications
Jessica.Rennekamp@agios.com

Exhibit 99.2

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Fueled by Connections to Transform Rare Diseases

Brian Goff, Chief Executive Officer

January 11, 2023

Forward-looking statements

This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of PYRUKYND® (mitapivat), AG-946 and its PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and its PAH stabilizer; Agios' strategic vision and goals, including its key milestones for 2023 and potential catalysts through 2026; 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. For example, there can be no guarantee that any product candidate Agios is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. There can be no guarantee that any positive developments in Agios' business will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this presentation and various remarks we make during this presentation 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 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; 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; 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 maintain key collaborations; 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; 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. Any forward-looking statements contained in this presentation and various remarks we make during this presentation 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.

Unmatched expertise in cellular metabolism

The Leader in Pyruvate Kinase (PK) Activation

agios

Track record of success in discovering, developing and commercializing therapies

DEVELOPED APPROVED THERAPIES IN ONCOLOGY

2017 & 2018

FIRST PYRUKYND® APPROVAL ADULTS WITH PYRUVATE KINASE (PK) DEFICIENCY

2022

RARE DISEASE FOCUS: POTENTIAL APPROVALS IN THALASSEMIA AND SICKLE CELL DISEASE

By 2026

Leader in pyruvate kinase (PK) activation poised for significant growth

Compelling and consistent data across connected diseases

Robust clinical data set supports potential of PK activation to transform patient function, quality of life, and long-term outcomes

Meaningful commercial opportunities on the horizon

First rare disease launch building capabilities to maximize anticipated franchise expansion

Potential for two additional PYRUKYND® indications by 2026

Well capitalized to advance and expand

Strong cash position expected to support completion of ongoing programs and disciplined portfolio expansion

Focused on expanding from PK deficiency to other diseases with shared pathophysiology, limited treatment options, and profound unmet needs

Pediatric PK Deficiency

No approved therapy for pediatric PK deficiency patients

Our goal: Deliver the first approved therapy for pediatric PK deficiency

Thalassemia

No approved therapy for ~60% of thalassemia patients

Our goal: Deliver the first therapy approved for all thalassemia subtypes

Sickle Cell Disease

No novel oral therapy improves anemia and reduces sickle cell pain crises

Our goal: Deliver a novel oral therapy that improves anemia and reduces VOCs

Lower-Risk MDS

No oral therapy addresses ineffective erythropoiesis

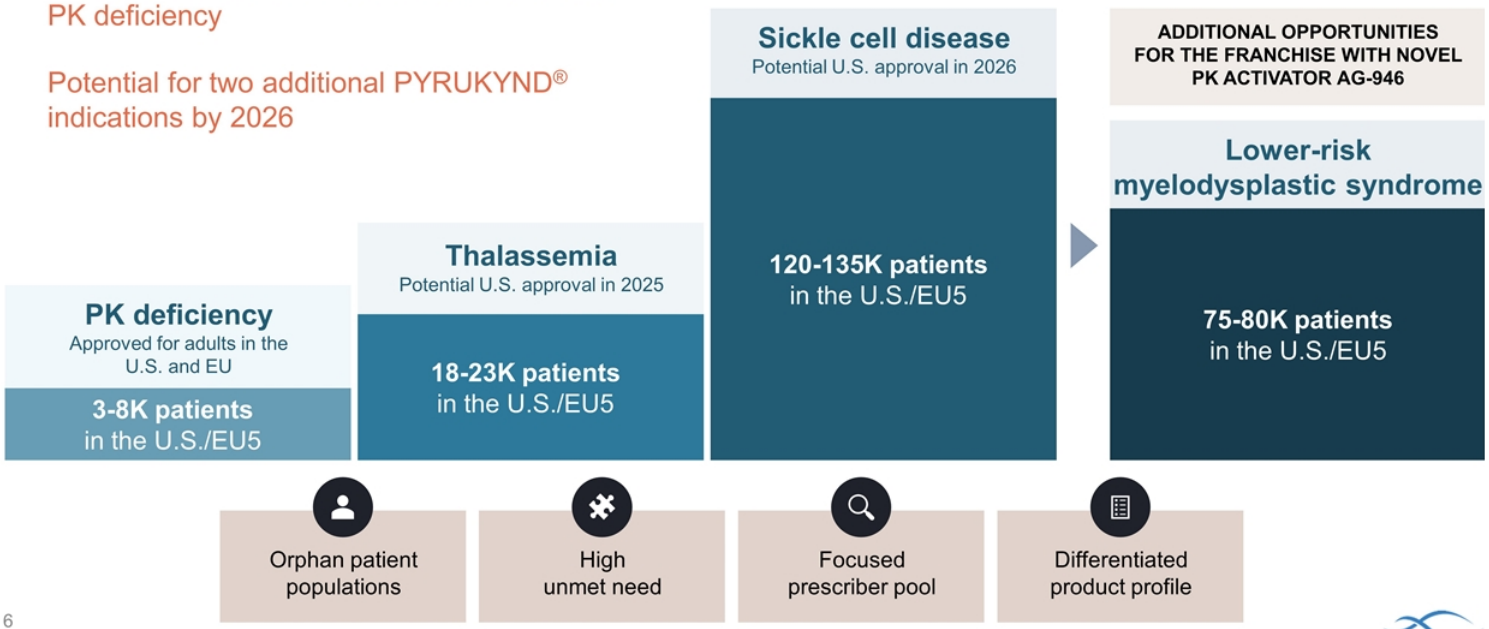
Our goal: Deliver the first oral therapy that addresses ineffective erythropoiesis

PYRUKYND® is approved in the U.S., EU, and Great Britain for adult PK deficiency and is under investigation for pediatric PK deficiency, thalassemia, and sickle cell disease.
AG-946 is under investigation for LR-MDS.

PK activation franchise positioned for meaningful expansion, with near-term opportunity in thalassemia

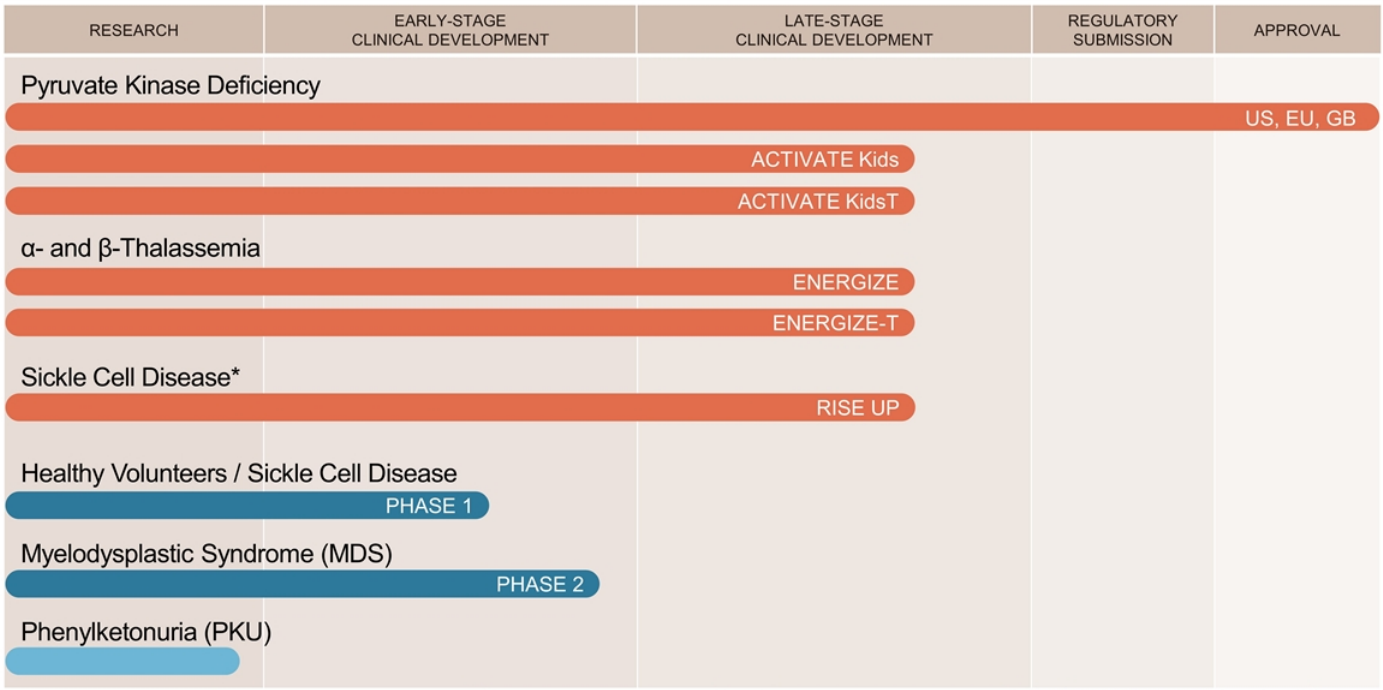
PYRUKYND® is the first and only disease-modifying treatment approved for adults with PK deficiency

Potential for two additional PYRUKYND® indications by 2026



Source: Agios internal estimates

Building a diverse pipeline leveraging our expertise in cellular metabolism



*In addition to RISE UP, two investigator-sponsored trials are ongoing with the NIH and University of Utrecht.

PYRUKYND®
First-in-class PK activator

AG-946
Novel PK activator

Phenylalanine hydroxylate (PAH) stabilizer

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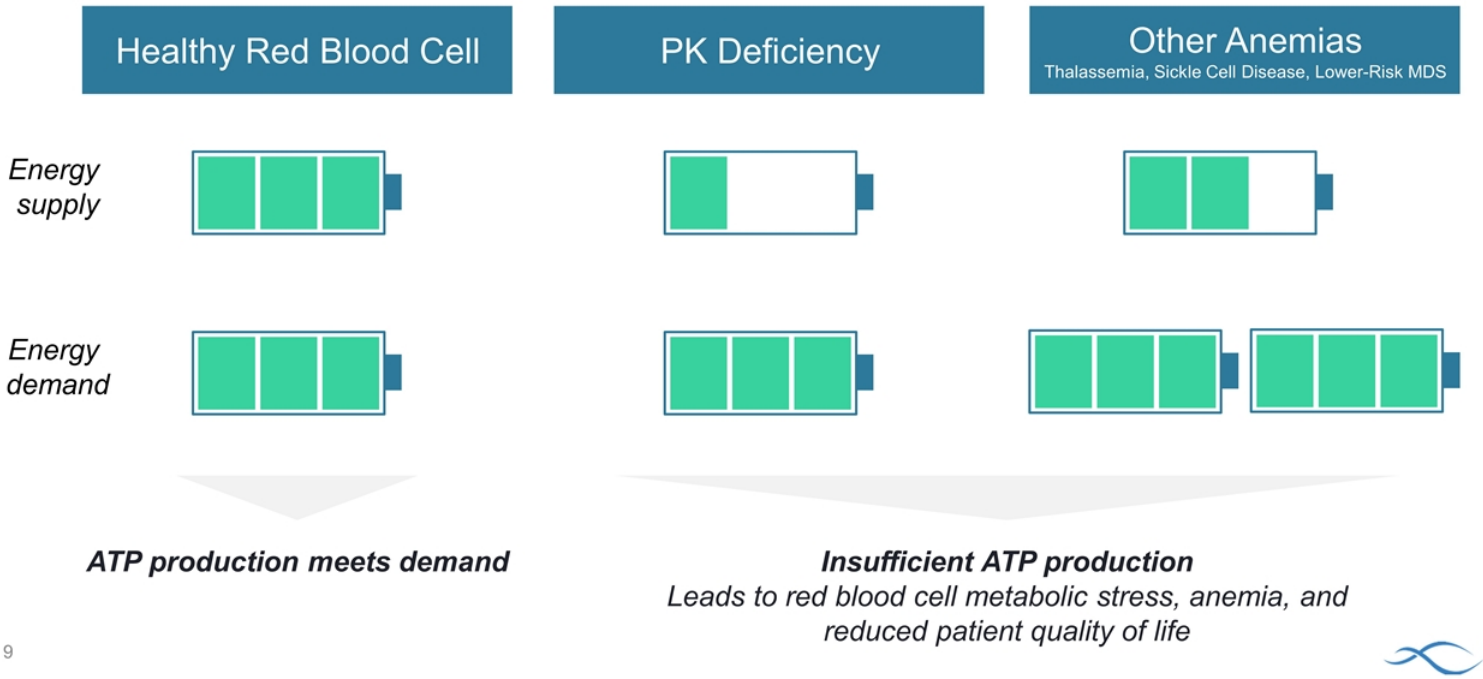
Compelling and consistent data across connected diseases

PK activation may address a range of hemolytic and acquired anemias underpinned by shared pathophysiology

Energy imbalance in red blood cells can lead to severe hematological disease



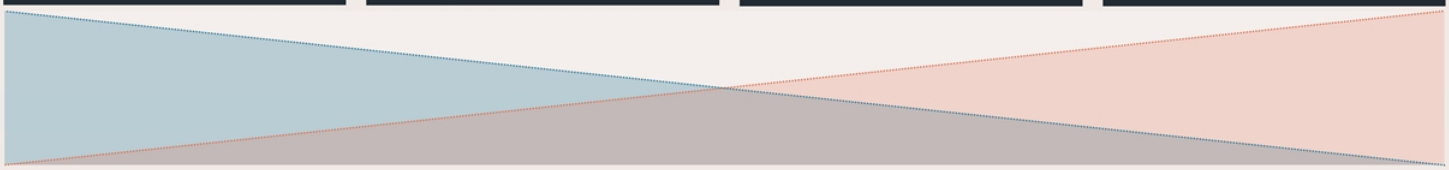
ATP production meets demand

Insufficient ATP production
Leads to red blood cell metabolic stress, anemia, and reduced patient quality of life

Disease areas share common pathophysiology and severely impact quality of life

Red Blood Cell Metabolic Stress

PK Deficiency | Sickle Cell Disease | Thalassemia | MDS Associated Anemia



Chronic Hemolysis
Destruction of red blood cells

Ineffective Erythropoiesis
Decreased output of red blood cells

Significant near and long-term clinical consequences of chronic hemolysis and ineffective erythropoiesis…

ANEMIA	TRANSFUSION BURDEN	IRON OVERLOAD	REDUCED BONE MINERAL DENSITY	LONG-TERM ORGAN DAMAGE

..that lead to major implications for patient quality of life and how they feel and function.

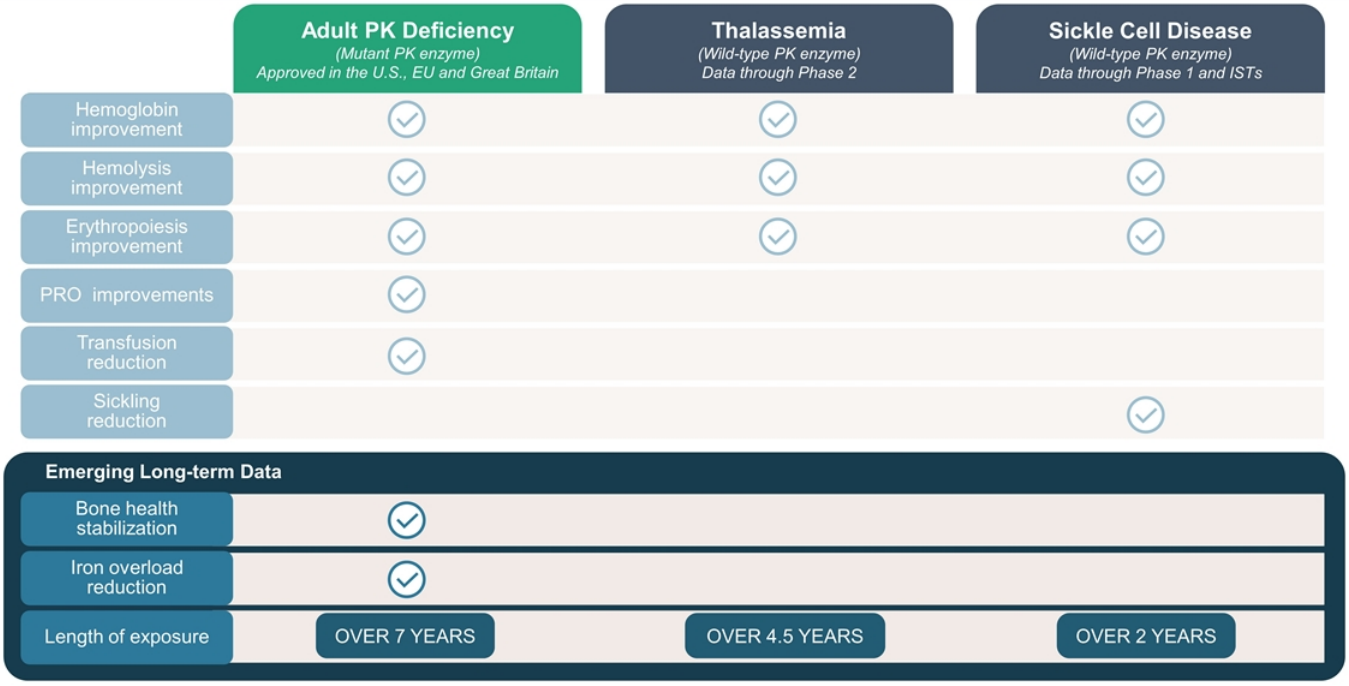
CHRONIC FATIGUE, SUSCEPTIBILITY TO ILLNESS	CHALLENGES WITH BASIC SOCIAL, SCHOOL / WORK ACTIVITIES	IMPACT ON EMOTIONAL AND MENTAL HEALTH	PAIN AND FRACTURES	ECONOMIC BURDEN

Consistency of clinical data to date supports potential of PYRUKYND® to address unmet patient needs across hemolytic anemias

	Adult PK Deficiency *(Mutant PK enzyme)* *Approved in the U.S., EU and Great Britain*	**Thalassemia** *(Wild-type PK enzyme)* *Data through Phase 2*	**Sickle Cell Disease** *(Wild-type PK enzyme)* *Data through Phase 1 and ISTs*
Hemoglobin improvement	✓	✓	✓
Hemolysis improvement	✓	✓	✓
Erythropoiesis improvement	✓	✓	✓
PRO improvements	✓		
Transfusion reduction	✓		
Sickling reduction			✓
	ONGOING STUDY	ONGOING STUDIES	ONGOING STUDIES
	Phase 3 long-term extension study	Phase 3 ENERGIZE and ENERGIZE-T studies Phase 2 long-term extension study	Phase 2/3 RISE UP study Investigator-sponsored trials

1. Al-Samkari et al. N Engl J Med 2022; 386:1432-1442; 2. Glenthoj et al. The Lancet Haematology, 2022, Vol 9, e724 - e732; 3. Van Beers et al. ASH 2021: Abstract 757; 4. Al-Samakri et al. ASH 2021. Abstract 924; 5. Kuo et al. ASH 2022: Abstract 506; 6. Van Beers et al. ASH 2022: Abstract 1021; 7. Grace et al. ASH 2022: Abstract 2328; 8. Kuo KHM et al. Lancet 2022; 400:10351; 9. Kuo KHM et al. ASH 2021: Abstract 576; 10. Kuo KHM et al. ASH 2022: Abstract 1030; 11. Van Dijk MJ et al. Am J Hematol. 2022;97:E226; 12. Xu JZ et al. Blood. 2022;140:2053; 13. Van Dijk MJ et al. EHA 2022: Abstract P1501.

Consistency of clinical data to date supports potential of PYRUKYND® to address unmet patient needs across hemolytic anemias



1. Al-Samkari et al. N Engl J Med 2022; 386:1432-1442; 2. Glenthoj et al. The Lancet Haematology, 2022, Vol 9, e724 - e732; 3. Van Beers et al. ASH 2021: Abstract 757; 4. Al-Samakri et al. ASH 2021. Abstract 924; 5. Kuo et al. ASH 2022: Abstract 506; 6. Van Beers et al. ASH 2022: Abstract 1021; 7. Grace et al. ASH 2022: Abstract 2328; 8. Kuo KHM et al. Lancet 2022; 400:10351; 9. Kuo KHM et al. ASH 2021: Abstract 576; 10. Kuo KHM et al. ASH 2022: Abstract 1030; 11. Van Dijk MJ et al. Am J Hematol. 2022;97:E226; 12. Xu JZ et al. Blood. 2022;140:2053; 13. Van Dijk MJ et al. EHA 2022: Abstract P1501.

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Meaningful commercial opportunities on the horizon

FDA approval and launch of PYRUKYND in PK deficiency builds capabilities to maximize potential product expansion

PYRUKYND® launch in PK deficiency building commercial capabilities to support potential expansion in meaningfully larger patient populations

Optimizing Patient & Provider Journey

Awareness & Education

Know **PK Deficiency**

Drive awareness and urgency to manage PK deficiency among providers and patients

Diagnostic Efficiency

Anemia ID®

Free genetic testing to help confirm diagnosis of hereditary anemias

PK deficiency patient identification via claims-based targeting

Therapy Onboarding

Activate physicians to prescribe and eligible patients to advocate for treatment

Access & Adherence

myagios

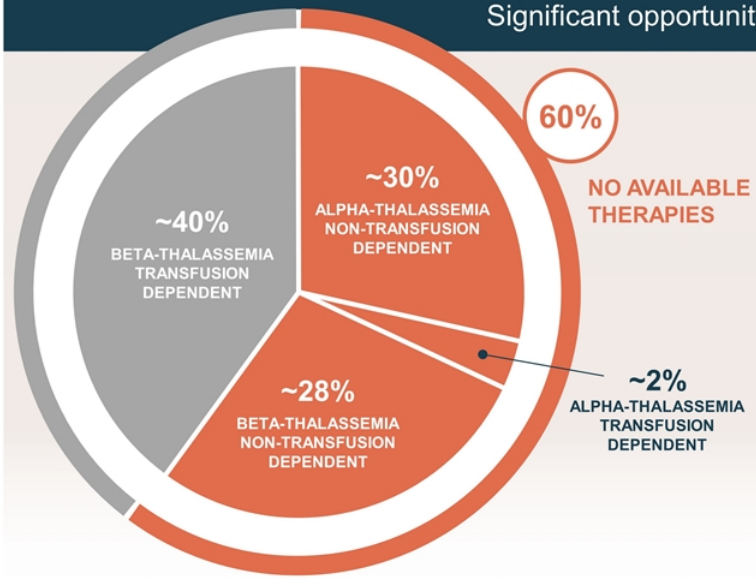
Coverage support, free product eligibility, copay program

Thalassemia: no approved therapies for ~60% of patients

High unmet need across patient segments and geographies

~18-23K Patients in U.S./EU5

Significant opportunity outside U.S./EU5



TARGET PROFILE

PYRUKYND®

- Address full range of thalassemia patients
- Chronic therapy
- Oral
- Improved Hb and reduced transfusion burden
- Improved ineffective erythropoiesis
- Safety profile consistent with prior clinical experience

Beta-THAL prevalence: HEOR Global THAL Epidemiology SLE (XCENDA, 2021); US: Paramore, et.al; DE: Borchert, et.al; IT: Italian Society of Thal & Hemoglobinopathies Patient Registry, Jan 2021, Angelucci, et.al, 2017; FR: French registry for thal (Thuret, et.al.); ES: Cela, et.al.; UK Registry for Hemoglobinopathies, 2020; Alpha-THAL prevalence: Agios internal estimates; LEK Analysis | Beta-THAL TD/NTD split (60% / 40%): Thuret, et.al., Haematologica 2010; Magnolia TPP MR, April 2020 | Alpha-THAL TD/NTD split (5% / 95%): Taher, et.al., Vox Sanguinis, 2015; Magnolia TPP MR, April 2020.

PYRUKYND® is under investigation for thalassemia and is not approved anywhere for that use.

Sickle Cell Disease: no novel oral therapy improves anemia and reduces sickle cell pain crises

- Estimated 120-135K patients across the U.S. & EU5
- Significant opportunity outside of U.S./EU

Global Reach

- Deliver a novel oral therapy that improves anemia, reduces VOCs and is supported by the largest body of clinical evidence

PYRUKYND® Opportunity

- Innovative seamless Phase 2/3 trial RISE UP developed with community input
- Global approach to clinical development
- Connections with SCD patient and physician communities

Critical Success Factors

TARGET PROFILE

PYRUKYND®

- Chronic Therapy
- Oral
- Improved hemolytic anemia
- Improvement in sickle cell pain crises (vaso-occlusive crises; VOCs)
- QoL data
- Safety profile consistent with prior clinical experience

PYRUKYND® is under investigation for sickle cell disease and is not approved anywhere for that use.

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Well capitalized to advance and expand

Long-term growth and value creation fueled by accomplished leadership team and strong balance sheet

Strong cash position expected to support portfolio advancement and expansion

Focused on rare diseases rooted in cellular metabolism

CLINICAL-STAGE PIPELINE

PYRUKYND®

- First and only disease-modifying treatment for adults with PK deficiency
- Potential for two additional indications by 2026

AG-946

- Potential to be first oral therapy to address the underlying cause of ineffective erythropoiesis in MDS

EARLIER-STAGE PIPELINE

LEAD RESEARCH PROGRAM

- PAH stabilizer for the treatment of PKU
- ~15-20K patients in the U.S.; ~20K in the EU5
- Target to file IND by year-end 2023

BUSINESS DEVELOPMENT

DISCIPLINED BD STRATEGY

- Prioritize opportunities based on:
  - Rare disease focus
  - Transformative for patients
  - Identified regulatory pathway
  - Potential to de-risk early
  - Clear path to value creation

Deliver transformative therapeutics for patient communities with profound unmet need

*PAH: phenylalanine hydroxylate; PKU: Phenylketonuria

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2023 and Beyond

Clinical development strategy driving toward broader commercial opportunities

Clinical and regulatory milestones targeted in 2023 lay the foundation for transformational data readouts

Thalassemia PYRUKYND®	Complete enrollment of Phase 3 ENERGIZE and ENERGIZE-T studies (mid-year)
Pediatric PK Deficiency PYRUKYND®	Enroll at least half of patients in the Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies (year-end)
Sickle Cell Disease PYRUKYND®	Phase 2 RISE UP data readout & go/no-go to Phase 3 decision (mid-year)
Lower-Risk MDS AG-946 (Novel PK Activator)	Complete enrollment of Phase 2a study (year-end)
Earlier-stage Pipeline	File IND for PAH stabilizer for the treatment of PKU (year-end)

Build commercial capabilities to efficiently launch additional indications and evaluate business development opportunities to expand pipeline

Potential for two additional PYRUKYND® indications by 2026

	2024	2025	2026
Thalassemia PYRUKYND®	Phase 3 ENERGIZE (1H) and ENERGIZE-T (2H) readouts	Potential approval	
Pediatric PK Deficiency PYRUKYND®		Phase 3 ACTIVATE-kids and ACTIVATE-kidsT readouts	Potential approval
Sickle Cell Disease PYRUKYND®		Potential Phase 3 RISE UP readout*	Potential approval
Lower-Risk MDS AG-946 (Novel PK Activator)	Phase 2a readout		

*Pending Go/NoGo decision in 2023

Driven to transform patient outcomes in rare diseases

2026 VISION


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Q&A