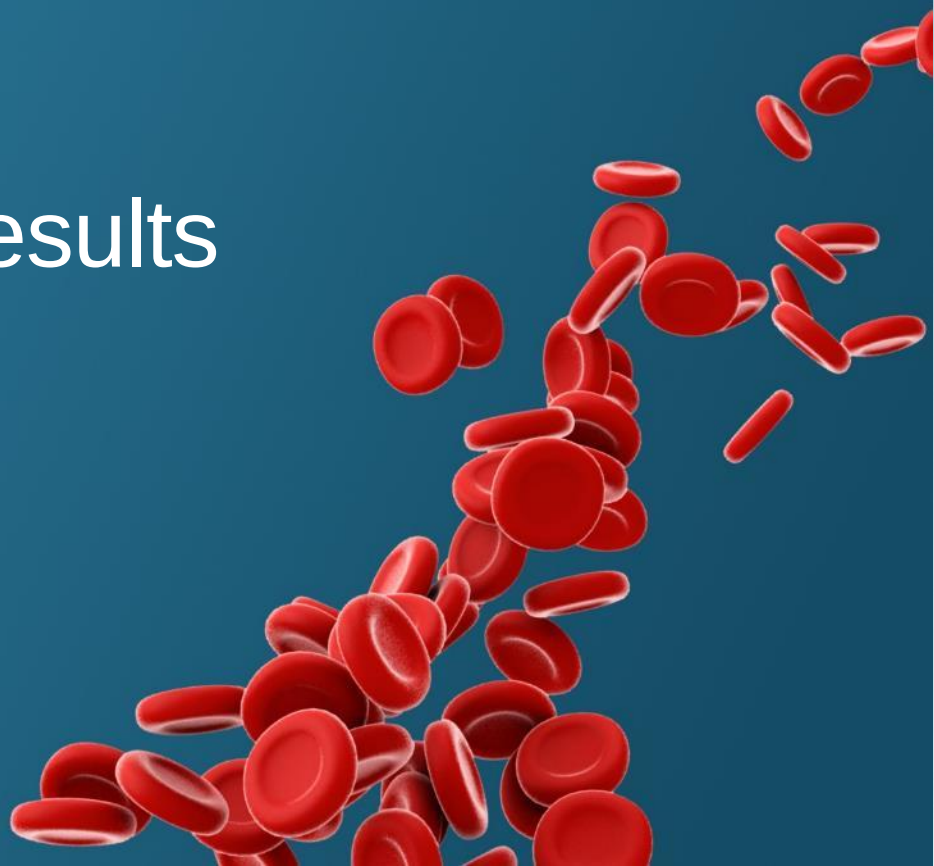
Q2 2024 Financial Results

August 1, 2024



Agios conference call participants

TOPIC	PARTICIPANT
Introduction	Chris Taylor, VP Investor Relations and Corporate Communications
Business Update	Brian Goff, Chief Executive Officer
R & D Update	Sarah Gheuens, M.D., Ph.D., Chief Medical Officer, Head of R&D
Commercial Update	Tsveta Milanova, Chief Commercial Officer
Second Quarter 2024 Financial Results	Cecilia Jones, Chief Financial Officer
Q&A	Mr. Goff, Dr. Gheuens, Ms. Milanova, Ms. Jones

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), tebapivat (AG-946), TMPRSS6 siRNA and AG-181, Agios' PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, tebapivat (AG-946) and AG-181; the potential FDA approval of vorasidenib; Agios' use of proceeds from the transaction with Royalty Pharma; potential U.S. net sales of vorasidenib and potential future royalty payments; Agios' strategic vision and goals, including its key milestones for 2024 and potential catalysts through 2026; and the potential benefits of Agios' strategic plans and focus. The words "anticipate", "expect", "goal", "hope", "milestone", "opportunity", "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 pandemics or other public health emergencies 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 establish and maintain key collaborations; uncertainty regarding any milestone or royalty payments related to the sale of its oncology business or its in-licensing of TMPRSS6 siRNA, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios' cash and cash equivalents; 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..

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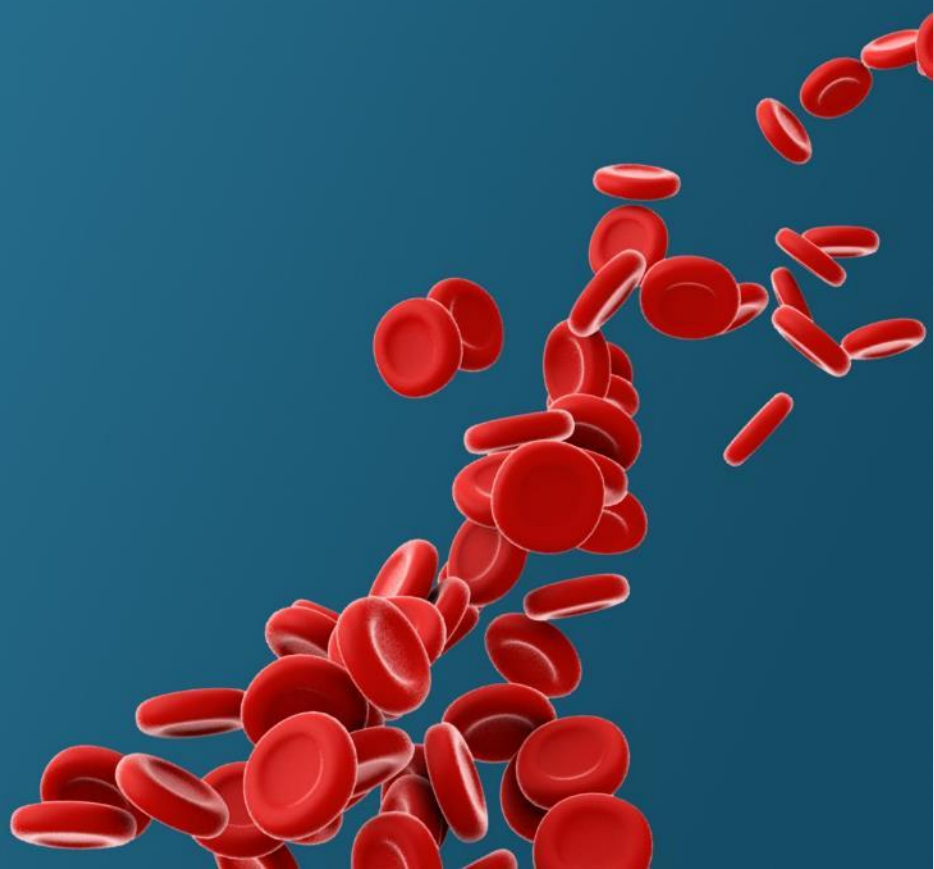
Business Overview

Brian Goff
Chief Executive Officer



Key milestones in Q2 2024 and continued positive momentum

Announced that Phase 3 ENERGIZE-T study of mitapivat met the primary endpoint and all key secondary endpoints in adults with transfusion-dependent alpha- or beta-thalassemia

Presented data from positive Phase 3 ENERGIZE study of mitapivat in adults with alpha- or beta-thalassemia who are not regularly transfused at a plenary session at EHA

Announced $905 million purchase agreement for vorasidenib royalty; Agios to receive a total of $1.1 billion in payments upon the potential FDA approval of vorasidenib

Reported today topline data from the Phase 3 ACTIVATE-KidsT study of mitapivat in children with PK deficiency who are regularly transfused

Announced today PYRUKYND commercial partnership for Gulf Cooperation Council (GCC) region

Continuing clinical and regulatory milestone momentum, with three Phase 3 data readouts in 2024

EARLY 2024

Thalassemia
PYRUKYND®
Phase 3 data readout for ENERGIZE study

PKU
AG-181
Begin Phase 1 dosing for AG-181 (PAH stabilizer) for the treatment of PKU

MID-YEAR 2024

Thalassemia
PYRUKYND®
Phase 3 data readout for ENERGIZE-T study

Lower-Risk MDS
Tebapivat (AG-946)
Begin patient enrollment of Phase 2b study

Pediatric PK Deficiency
PYRUKYND®
Complete enrollment Phase 3 ACTIVATE-Kids study

Pediatric PK Deficiency
PYRUKYND®
Phase 3 data readout ACTIVATE-KidsT study

YEAR-END 2024

Thalassemia
PYRUKYND®
Filing for FDA Approval

Sickle Cell Disease
PYRUKYND®
Complete Phase 3 enrollment

PKU = Phenylketonuria; PAH = Phenylalanine hydroxylase

★ Announced August 1, 2024

Capturing larger patient populations positions PYRUKYND® for significant near-term growth as a first- and best-in-class therapy

pyrukynd® (mitapivat) tablets

3-8K patients
in the U.S./EU5

PK deficiency
2022
Approved for adults in the U.S., EU, and Great Britain

OUR GOAL
Deliver the first approved therapy for pediatric PK deficiency

18-23K patients
in the U.S./EU5

~70K patients in GCC

>1M patients worldwide

Thalassemia
2025
Potential U.S. approval

OUR GOAL
Deliver the first therapy approved for all thalassemia subtypes

120-135K patients
in the U.S./EU5

~150K patients
in GCC

>3M patients
worldwide

Sickle cell disease
2026
Potential U.S. approval

OUR GOAL
Deliver a novel oral therapy that improves anemia and reduces VOCs

PYRUKYND® is approved in the U.S., EU, and Great Britain for adult pyruvate kinase (PK) deficiency and is under investigation for pediatric PK deficiency, thalassemia, and sickle cell disease.
Source: Agios internal estimates

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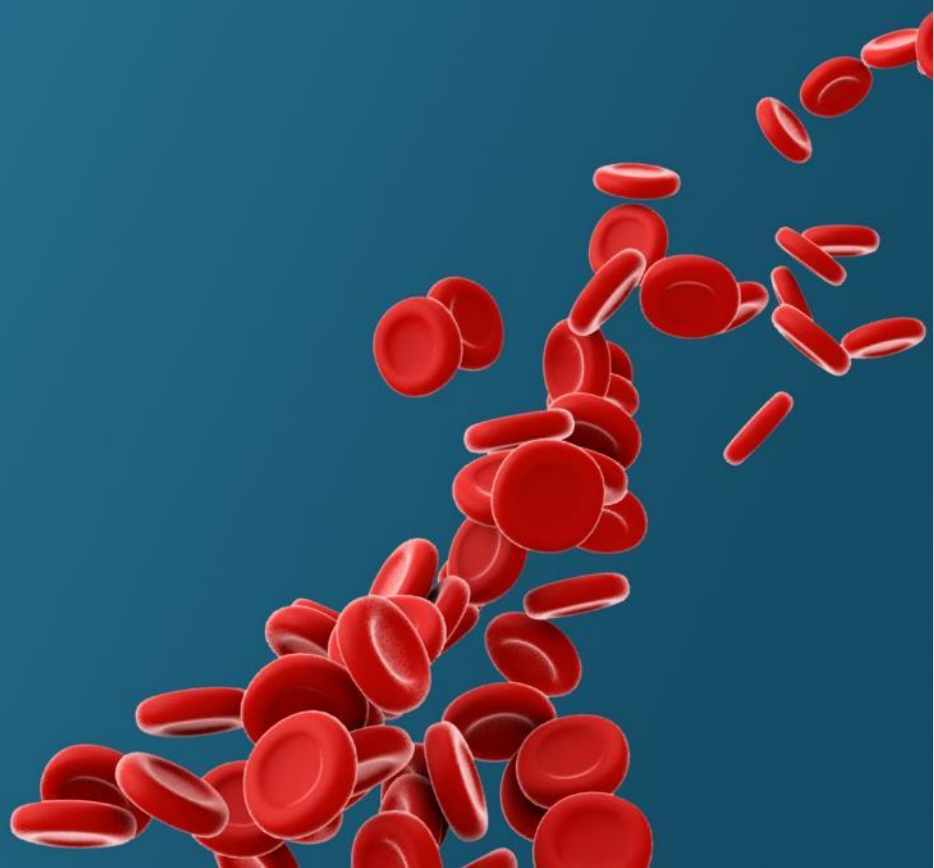
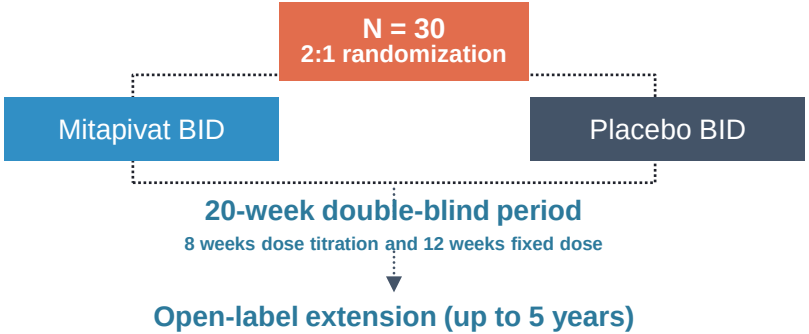
Clinical Overview

Sarah Gheuens, M.D., Ph.D.
Chief Medical Officer, Head of R&D



Pediatric PK Deficiency Program: Two Phase 3 studies evaluating regularly transfused and not regularly transfused pediatric patients with PKD

ACTIVATE-Kids



Primary endpoint

- > 1.5 g/dL (0.93 mmol/L) increase in Hb concentration from baseline that is sustained at 2 or more scheduled assessments at weeks 12, 16 and 20 during the double-blind period

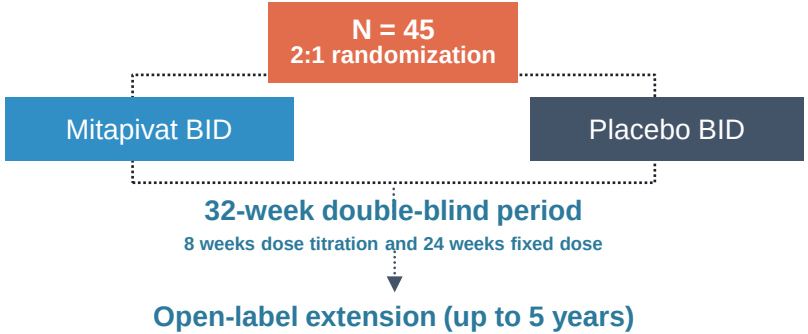
Secondary endpoints

- Additional measures of Hb ↑, hemolysis, HRQOL, iron metabolism, safety, PK/PD

Key inclusion criteria

- Aged 1 to <18 years
- Clinical laboratory confirmation of PK deficiency
- Not regularly transfused defined as ≤5 RBC transfusion episodes during the 52-week period before informed consent/assent and no RBC transfusions ≤12 weeks prior to randomization
- Hb ≤10 g/dL for subjects 12 to <18 years of age or ≤9 g/dL for subjects 1 to <12 years of age`

ACTIVATE-KidsT



Primary endpoint

- ≥33% reduction in the total RBC transfusion volume from Week 9 through Week 32 of the double-blind period normalized by weight and actual study drug duration compared with the historical transfusion volume standardized by weight and to 24 weeks

Secondary endpoints

- Additional measures of transfusion reduction, HRQOL, safety, PK/PD

Key inclusion criteria

- Aged 1 to <18 years
- Clinical laboratory confirmation of PK deficiency
- Regularly transfused defined as 6 to 26 RBC transfusion episodes during the 52-week period before informed consent/assent

BID = twice daily; Hb = hemoglobin; PK = pharmacokinetics; PD = pharmacodynamics, RBC = red blood cell.

ACTIVATE-KidsT: First clinical study in regularly transfused pediatric patients with PKD

ACTIVATE-KidsT

Topline Results

Enrollment & Completion

- A total of 49 patients aged 1 to <18 years were enrolled in the study, with 32 randomized to mitapivat twice-daily and 17 randomized to matched placebo.
- 30 patients (93.8%) in the mitapivat arm and 16 (94.1%) in the placebo arm completed the 32-week double-blind period of the study.

Transfusion Reduction Response

- 28.1% (9/32) of patients in the mitapivat arm achieved a transfusion reduction response, compared to 11.8% (2/17) of patients in the placebo arm.

Secondary Endpoints

- 6 patients (18.8%) in the mitapivat arm compared to 0 in the placebo arm had no red blood cell transfusions from week 9 through week 32 of the double-blind period (transfusion-free response).
- 4 patients (12.5%) in the mitapivat arm compared to 0 in the placebo arm achieved hemoglobin concentrations within normal limits at least once, 8 weeks or more after a transfusion, from Week 9 through Week 32 of the double-blind period (normal hemoglobin response).

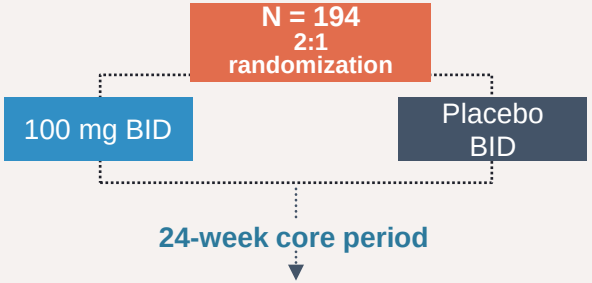
Safety

- In the 32-week double-blind treatment period of the study, a similar proportion of patients had adverse events (AEs) in the mitapivat and placebo arms and there were no discontinuations of study treatment due to AEs.
- Safety results in this pediatric study were consistent with the safety profile for mitapivat previously observed for adult subjects with PK deficiency who are regularly transfused.

The analysis of the primary endpoint of transfusion reduction response (TRR) was based on Bayesian statistical methodology whereby the TRR data from the adult ACTIVATE-T study informed and contributed to the analysis of TRR in the ACTIVATE-KidsT study. The analysis was performed using a range of relative weights of borrowing from the adult ACTIVATE-T study, representing the prior degree of belief in the similarity of the treatment effect in the pediatric and adult populations. The prespecified statistical criterion for the primary endpoint in ACTIVATE-KidsT was not met with low or moderate borrowing weights; however, the results were clinically meaningful.

Two global, Phase 3, randomized controlled trials of PYRUKYND® encompass broad range of thalassemia patients

ENERGIZE



Primary endpoint

- Mean Hb ↑ ≥ 1 g/dL from baseline

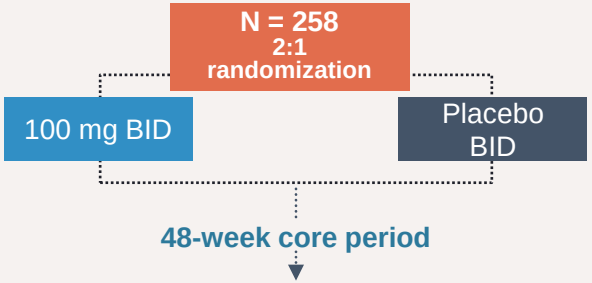
Secondary endpoints

- Fatigue, additional measures of Hb ↑, hemolysis, patient-reported outcomes, physical activity, iron metabolism, safety, PK/PD

Key inclusion criteria

- ≥ 18 years
- β-thalassemia ± α-globin mutations, HbE β-thalassemia, or α-thalassemia (HbH disease)
- Non-transfusion-dependent defined as ≤5 RBC units during the 24-week period before randomization and no RBC transfusions ≤8 weeks prior
- Hb ≤ 10.0 g/dL

ENERGIZE-T



Primary endpoint

- 50% reduction in transfusion burden in any 12-week rolling period

Secondary endpoints

- Additional measures of transfusion reduction, safety, PK/PD

Key inclusion criteria

- ≥ 18 years
- β-thalassemia ± α-globin mutations, HbE β-thalassemia, or α-thalassemia (HbH disease)
- Transfusion-dependent defined as 6 to 20 RBC units transfused and ≤6-week transfusion-free period during the 24-week period before randomization

HbE = hemoglobin E; HbH = hemoglobin H

Phase 3 ENERGIZE study attained primary and key secondary endpoints; statistically significant improvements in Hb and fatigue

ENERGIZE

- This global study was the first to enroll patients with α-thalassemia in addition to β-thalassemia
- The primary and key secondary endpoints were met, with statistically significant improvements in Hb and fatigue with mitapivat vs placebo
  - All prespecified subgroup analyses favored mitapivat vs placebo
- Improvements in markers of hemolysis and erythropoietic activity were observed, consistent with the mechanism of mitapivat[1–3]
- Mitapivat was generally well tolerated in this study, with a low treatment discontinuation rate and a safety profile consistent with other studies[3–6]

ENERGIZE demonstrated efficacy of mitapivat, a disease-modifying therapy, with significant improvements in both Hb and fatigue across the full range of NTDT, including both α- and β-thalassemia

NTDT = non–transfusion-dependent thalassemia

1. Kung C et al. Blood 2017;130:1347–56; 2. Matte A et al. J Clin Invest 2021;131:e144206; 3. Kuo KHM et al. Lancet 2022;400:493–501; 4. Al-Samkari H et al. NEJM 2022;386:1432–42; 5. Glenthøj A et al. Lancet Haematol 2022;9:e724–32; 6. Idowu M et al. Blood 2023;142:271.

Significant improvements in quality-of-life-related outcomes data from the Phase 3 ENERGIZE study

ENERGIZE

- In the 24-week double-blind period of ENERGIZE, significant improvements in fatigue, measured by FACIT-Fatigue, were demonstrated in the mitapivat arm compared with the placebo arm
  - A higher proportion of patients reported clinically meaningful improvements with mitapivat vs placebo
- Functional improvement in patients with mitapivat, measured by the 6MWT, exceeded a previously reported meaningful change threshold from the literature[18]
- A higher proportion of patients with mitapivat reported improved fatigue, disease symptoms, and walking capacity via PGIC with mitapivat vs placebo

Mitapivat is the first oral, disease-modifying, investigational therapy to improve fatigue and walking capacity in patients with α- or β-NTDT

6MWT= 6-minute walk test; FACIT-Fatigue = Functional Assessment of Chronic Illness Therapy–Fatigue Scale; PGIC = Patient Global Impression of Change

1. St. Lezin E et al. *Transfusion* 2019;59;1934–43. Kuo KHM et al. Poster presentation at the European Hematology Association Congress 2024, Madrid.

Phase 3 ENERGIZE-T Study: Primary endpoint achieved

ENERGIZE-T

- Total of 258 patients were randomized 2:1 to 100 mg mitapivat (n=171) or placebo (n=87)
- 155 patients (90.6%) in the mitapivat arm and 83 patients (95.4%) in the placebo arm completed the 48-week double-blind period of the study
- Transfusion reduction response (TRR) is defined as ≥50% reduction in transfused RBC units of ≥2 units of transfused RBCs in any consecutive 12-week period compared to baseline
- **Treatment with mitapivat demonstrated a statistically significant transfusion reduction response compared to placebo**

Primary Endpoint	Placebo N=87	Mitapivat 100 mg BID N=171
TRR responders, n (%)	11 (12.6)	52 (30.4)
Adjusted difference TRR rate (Mitapivat-Placebo), %		17.6
95% CI		(8.0, 27.2)
2-sided p-value		0.0003

TRR = transfusion reduction response.

Subjects withdrawn from the study before Week 12 (Day 85) are considered non-responders.

Baseline transfusion burden standardized to 12 weeks=total number of RBC units transfused during the 24-week period (168 days) before 'reference date' x12/24, where 'reference date' is the randomization data for subjects randomized and not dosed or the start of study treatment for subjects randomized and dosed.

The 95% CI and p-value are based on the Mantel-Haenszel stratum weighted method adjusting for randomization stratification factors.

ENERGIZE-T: Additional results demonstrate mitapivat's durability of effect

ENERGIZE-T

Efficacy

Treatment with mitapivat demonstrated statistically significant improvements on all key secondary endpoints evaluating additional measures of reduction in transfusion burden:

- ≥50% reduction in transfused RBC units in any consecutive 24-week period through week 48 compared to baseline
- ≥33% reduction in transfused RBC units from week 13 through week 48 compared to baseline
- ≥50% reduction in transfused RBC units from week 13 through week 48 compared to baseline

Transfusion independence

- A higher proportion of patients in the mitapivat arm (9.9%) compared to the placebo arm (1.1%) achieved the secondary endpoint of transfusion independence (transfusion-free for ≥8 consecutive weeks through week 48)

Safety

- Overall, during the 48-week double-blind period, incidence of adverse events (AEs) was similar across mitapivat and placebo arms
- In the mitapivat arm, 5.8% of the patients experienced an AE leading to discontinuation, compared to 1.2% of patients in the placebo arm

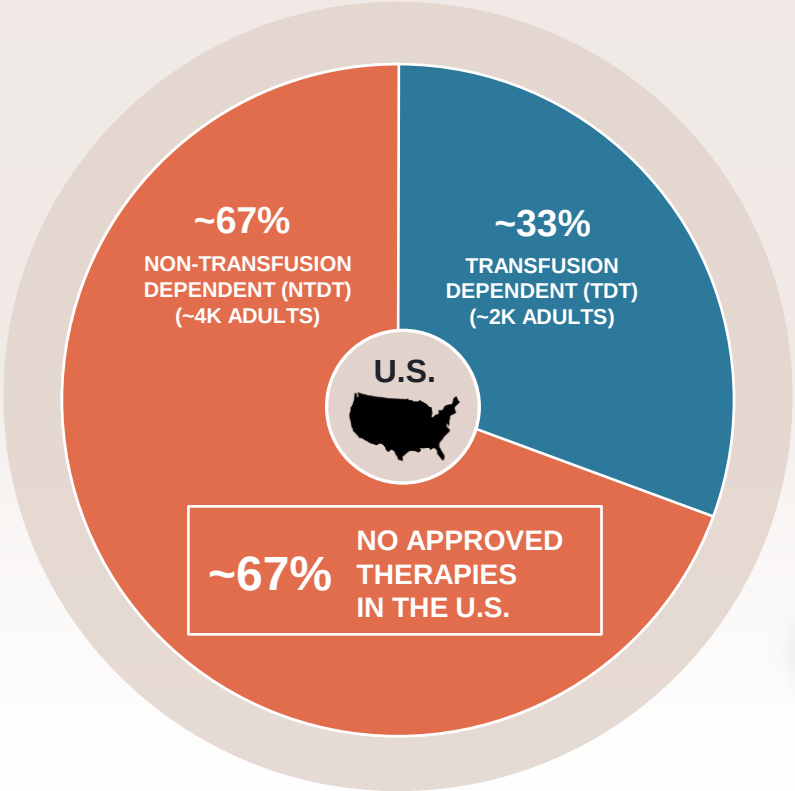
Agios aims to deliver the first therapy for all thalassemia subtypes

Mitapivat Thalassemia Phase 3 program

ENERGIZE

- **Alpha- and Beta-thalassemia Non-transfusion dependent** patients
- Primary endpoint achieved: Hemoglobin (Hb) response

Data announced January 3, 2024



Mitapivat Thalassemia Phase 3 program

ENERGIZE-T

- **Alpha- and Beta-thalassemia Transfusion dependent** patients
- Primary endpoint achieved: Transfusion Reduction Response

Data announced June 3, 2024

Beta-THAL prevalence: HEOR Global THAL Epidemiology SLE (XCENDA, 2021); US: Paramore, et.al; DE: Borchert, et.al; Alpha-THAL prevalence: Agios internal estimates; LEK Analysis | Beta-THAL TD/NTD split: Thuret, et.al., Haematologica 2010; Magnolia TPP MR, April 2020 | Alpha-THAL TD/NTD split; 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.

PYRUKYND: A novel oral therapy with potential to be best-in-class improving anemia, reducing SCPCs and improving how patients feel and function

RISE UP

Phase 2 Data

- **Statistically significant increase in hemoglobin response** rate observed at both doses compared to placebo
- **Improvements in markers of hemolysis and erythropoiesis** observed at both doses compared to placebo
- A **trend in sickle cell pain crises reduction** was observed at both doses compared to placebo
- No adverse events (AEs) leading to discontinuation

Phase 3 Design(1)

- **Phase 3 primary endpoints:** Hb response(2) and annualized rate of SCPCs
- **N = 198** with a 2:1 randomization (100 mg mitapivat and placebo)
- **52-week** double blind period followed by 216-week open label extension

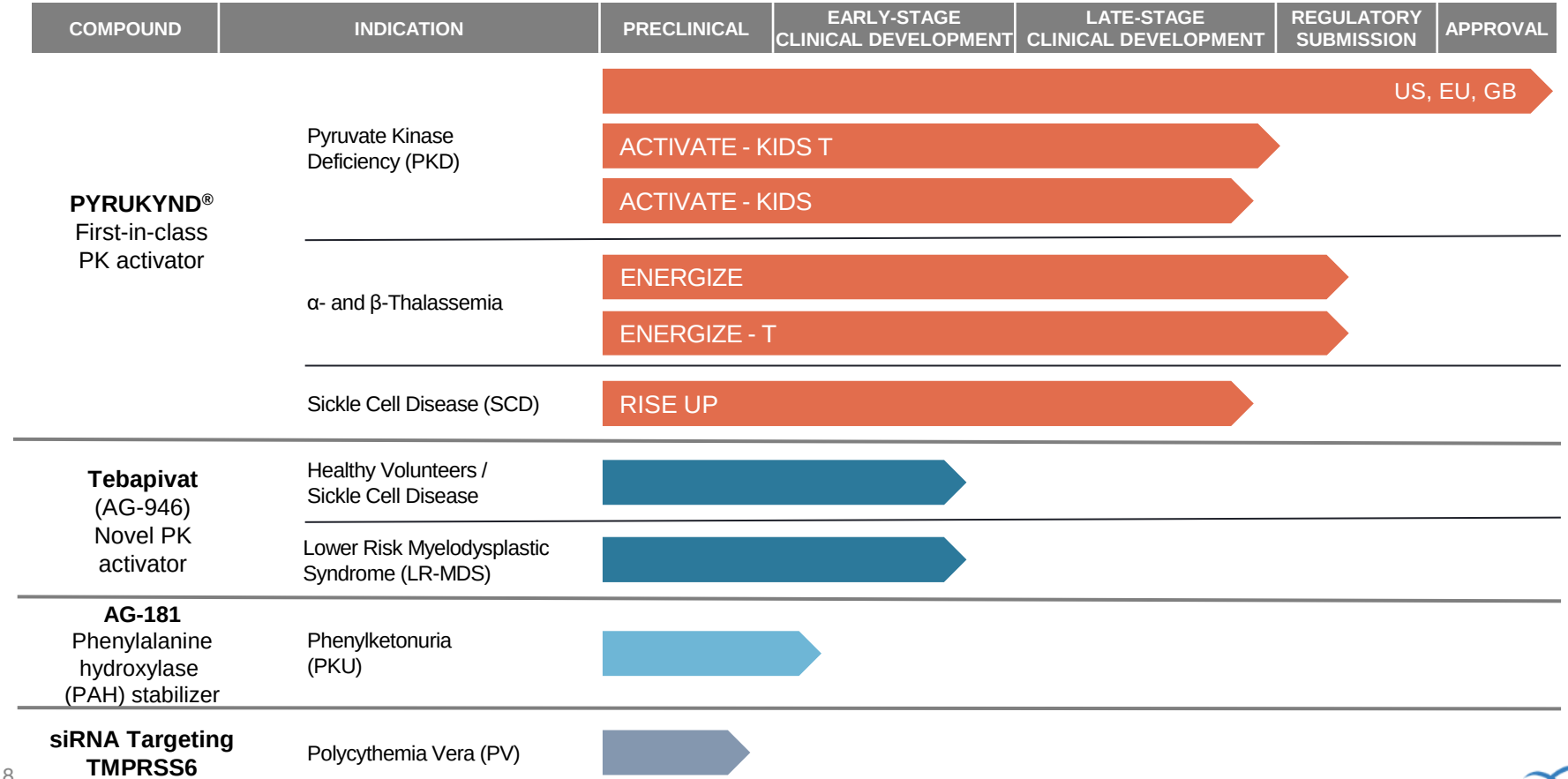
PYRUKYND

- Seamless Phase 2/3 global study **designed with community input**
- Potential for mitapivat to:
  - **improve anemia**
  - **reduce sickle cell pain crises**
  - **improve how patients feel and function**
- Expected data readout in 2025
- Potential US **launch in 2026**

SCPC = sickle cell pain crises

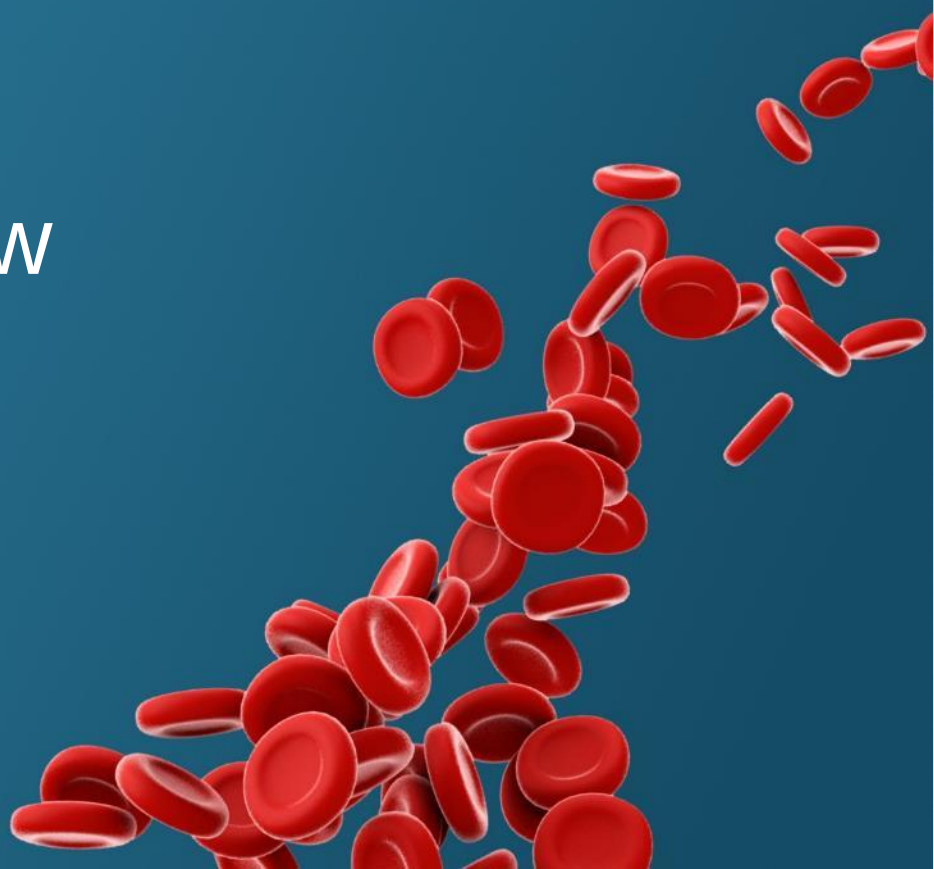
(1) Phase 2 and phase 3 components are part of a single study/protocol; 100mg was selected for Phase 3 portion of the study
(2) Hb response is defined as a ≥ 1.0 g/dL increase in average Hb concentration over Weeks 24–52 compared with baseline

Significant advancement building depth and breadth in our rare disease pipeline



Commercial Overview

Tsveta Milanova
Chief Commercial Officer



Thalassemia remains an area of high unmet need with few treatment options and significant burden of disease regardless of transfusion needs

Increased Mortality	Serious, Irreversible Morbidities	Poor Quality of Life	Healthcare Resource Utilization & Cost
Lower survival for thalassemia patients, and **significantly worse in those who remain non-regularly transfused**	**High rates of morbidities** and frequency of complications increasing as patients age	Adult patients with **NTDT may have similar or worse Healthcare Related QoL** compared with patients with TDT	A 1g/dL decrease in average Hb levels is associated with **increased inpatient, outpatient and ER visits/costs, Rx costs, and total healthcare costs** in patients with NTDT

TDT = transfusion dependent thalassemia

Source: Musallam, K, et al.. 2022. Hemasphere 6(12) e806;Thalassemia International Federation, 2023; Musallam, K, et al., 2021. Am J Hematol 97(2) E78-E80; Association of Hemoglobin Levels with Healthcare Resource Utilization and Costs in Non-Transfusion Dependent Alpha and Beta Thalassemia: A Retrospective Observational Study Using Real-World Data (August 1, 2023); Musallam KM et al. Ann Hematol 2021. doi: 10.1007/s00277-020-04370-2; Musallam K., et al. Haematologica. 2021 Sep 1; 106(9): 2489-2492

PYRUKYND® has the potential to become the first therapy approved for all thalassemia subtypes

Thalassemia
~8K in US

Adult Thalassemia (18+)
~6K (~70%)

Alpha Thalassemia
~3K (~47%)

Beta Thalassemia
~3K (~53%)

Non-Transfusion Dependent Alpha Thalassemia	Transfusion Dependent Alpha Thalassemia	Non-Transfusion Dependent Beta Thalassemia	Transfusion Dependent Beta Thalassemia
~3K (~95%)	~0.1K (~5%)	~1K (~40%)	~2K (~60%)

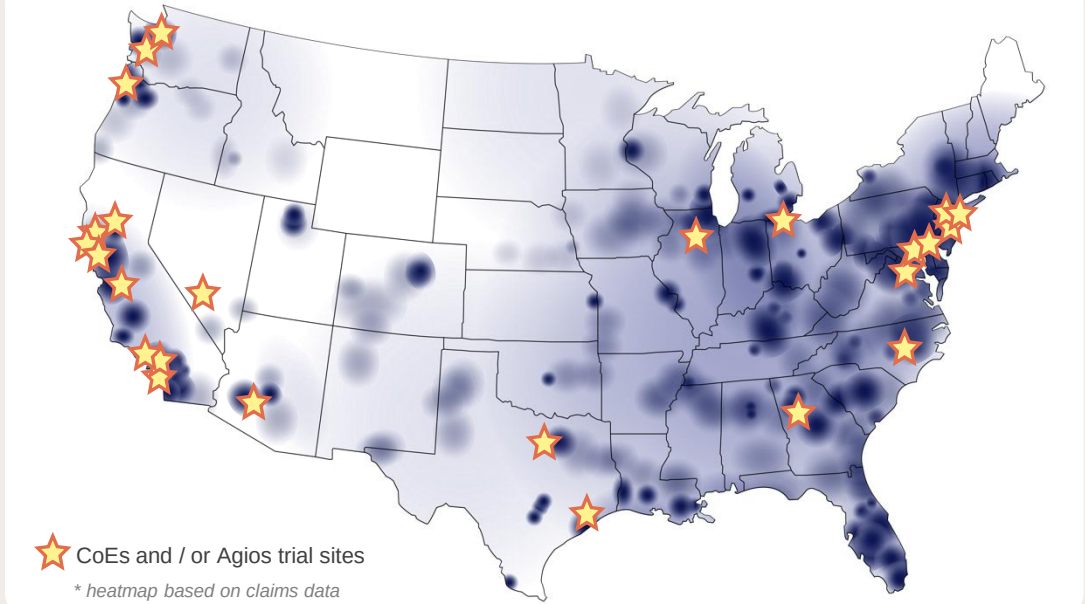
~67% OF ADULTS DO NOT HAVE AN APPROVED THERAPY IN THE US

Approved treatment in US*	✗	✗	✗	✓
PYRUKYND potential label	✓	✓	✓	✓

Beta-THAL prevalence: HEOR Global THAL Epidemiology SLE (XCENDA, 2021); US: Paramore, et.al; 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: 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. *Note: Reblozyl also approved in non-transfusion dependent beta-thalassemia EU

Strong alignment with trial sites, key centers for thalassemia patients across US

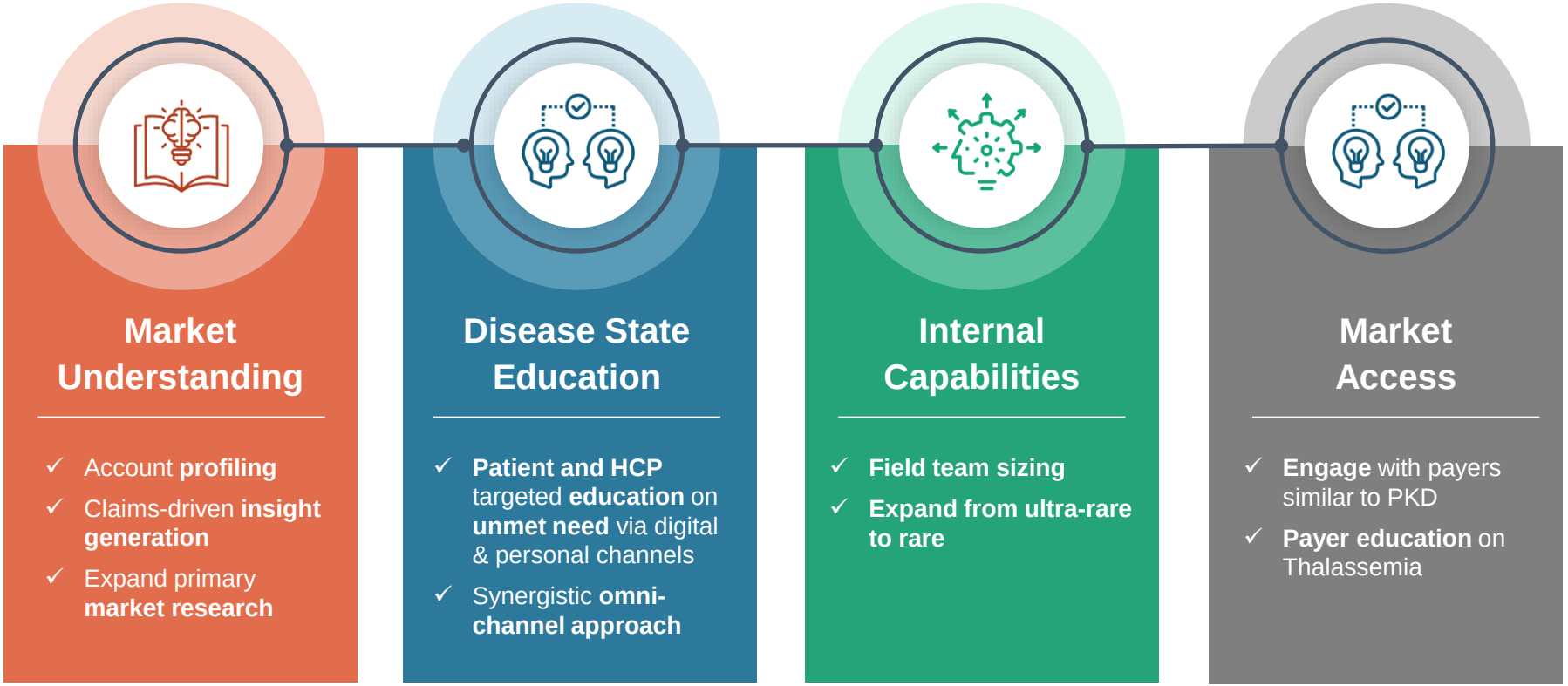
Patient prevalence heatmap alongside Agios trial sites and /or Centers of Excellence (COE)



- Availability of **newborn** Thalassemia **screening**
- Well established **ICD-10 codes in Thalassemia**
- **Agios trial sites** aligned to key Health Care Providers and Centers of Excellence

Centers of Excellence: CDC Funded Thalassemia Treatment Centers and Thalassemia Western Consortium
Source: Compile Claims Analysis August 2023; BELIEVE NEJM Appendix 2020, BEYOND Lancet Hematology Appendix 2022, ENERGIZE ct.gov (accessed August 2023), ENERGIZE-T ct.gov (accessed August 2023); Agios Medical Affairs

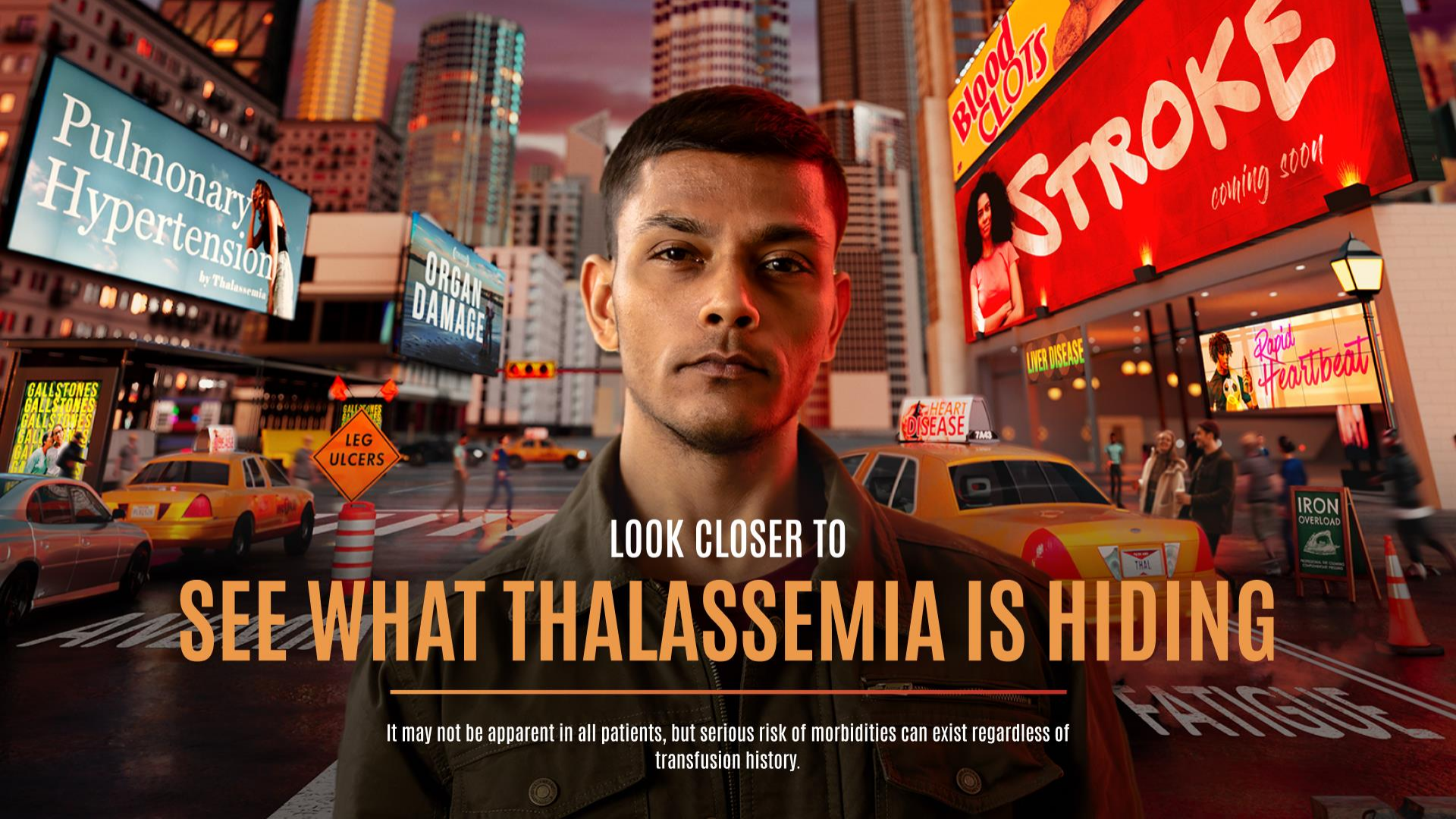
Strengthening our commercial capabilities to support thalassemia launch in a meaningfully larger patient population



Disease state education (DSE) campaigns have been launched for both patients and health care providers

DSE Objective	Example DSE Messaging
 Highlight Disease Burden	*"**Hemolysis and ineffective erythropoiesis** are the primary drivers of downstream complications, including **chronic anemia**"* *"Patients with NTD thalassemia reported a **similar or worse quality of life** as measured by SF-36 vs those who were transfusion-dependent"*
 Encourage Disease Monitoring and Management	*"Frequent monitoring and management of NTD patients with thalassemia is critical to identify and help mitigate the development of morbidities, such as **pulmonary hypertension**, **thrombotic disease**, and **liver disease**"* *"Patients with thalassemia should receive a **long-term thalassemia management plan** for optimal care"*

NTD: Non-transfusion-dependent; SF-36:36-Item Short Form Survey
Source: Rethink Thalassemia



LOOK CLOSER TO

SEE WHAT THALASSEMIA IS HIDING

It may not be apparent in all patients, but serious risk of morbidities can exist regardless of transfusion history.

Recent market research identified top clinical characteristics healthcare providers will consider when prescribing PYRUKYND

Top Clinical Patient Characteristics HCPs Will Consider When Prescribing PYRUKYND

Hemoglobin Levels

Transfusion Burden

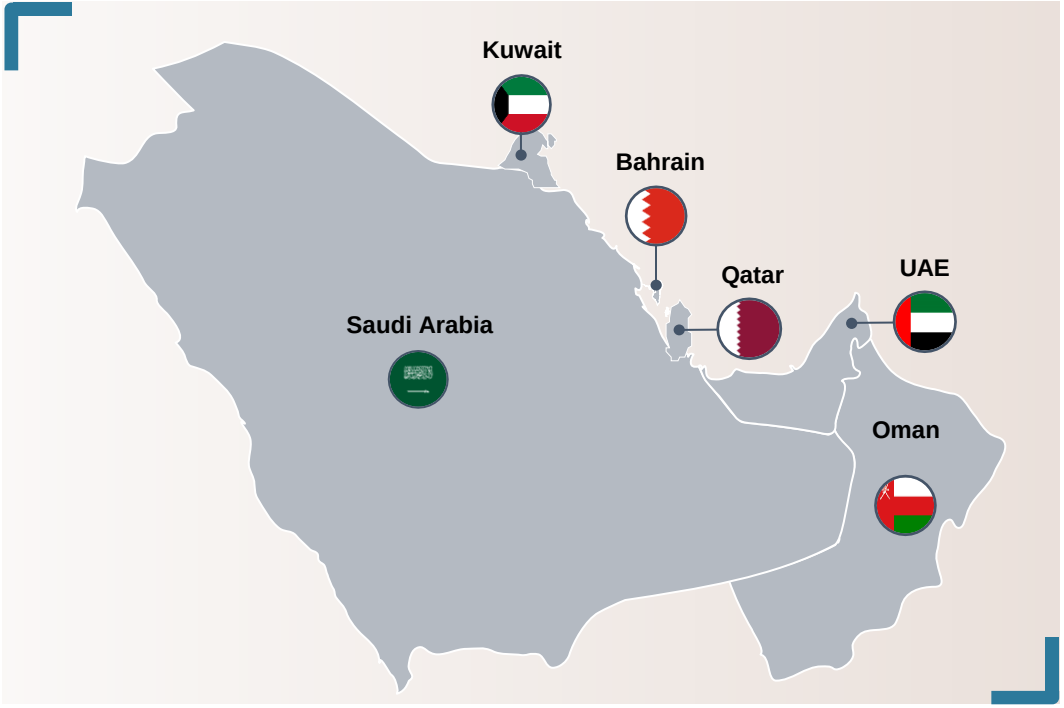
Fatigue

Iron Overload / Ferritin Levels

Source: US THAL Demand Study (N=97 Heme/Oncs)

The GCC region represents a significant opportunity for PYRUKYND in thalassemia; distribution partner signed to unlock value

Approximately 70k Thalassemia Patients in Gulf Cooperation Council (GCC) Countries



- **High unmet need** given disproportionally high prevalence
- Entered **distribution agreement** with **NewBridge;** a **leading** specialty company with **regulatory and commercial expertise in the GCC**
- Saudi Arabia accounts for the majority of patients in GCC region
- Mitapivat received **Breakthrough Medicine Designation** by the SFDA (Saudi FDA); one of the first products to receive BMD
- The **access path** in the GCC region begins with a price set at the regulatory level, followed by access with health authorities, local institutions, and the private sector, and national tenders

GCC: Gulf Cooperation Council

PYRUKYND® Q2 2024 performance metrics highlight continued progress

$8.6M net sales of PYRUKYND®
5% growth over Q1 2024

128 patients on PYRUKYND®,
which includes new prescriptions and those continuing treatment, a 7% increase from Q1

Patients on therapy represent broad demographic range;
consistent with the adult PK deficiency population

201 unique patients completed PYRUKYND® prescription enrollment forms, including 13 in Q2, a 7% increase over Q1 2024

Unique prescriber base of 173 physicians, diversified across the country, a 6% increase over Q1 2024

PYRUKYND® expansion into diseases with larger patient populations provides significant near-term growth potential for first- and best-in-class therapies

pyrukynd® (mitapivat) tablets

3-8K patients
in the U.S./EU5

PK deficiency
2022
Approved for adults in the U.S., EU and Great Britain

OUR GOAL
Deliver the first approved therapy for pediatric PK deficiency

18-23K patients
in the U.S./EU5

~70K patients in GCC

>1M patients worldwide

Thalassemia
2025
Potential U.S. approval

OUR GOAL
Deliver the first therapy approved for all thalassemia subtypes

120-135K patients
in the U.S./EU5

~150K patients
in GCC

>3M patients
worldwide

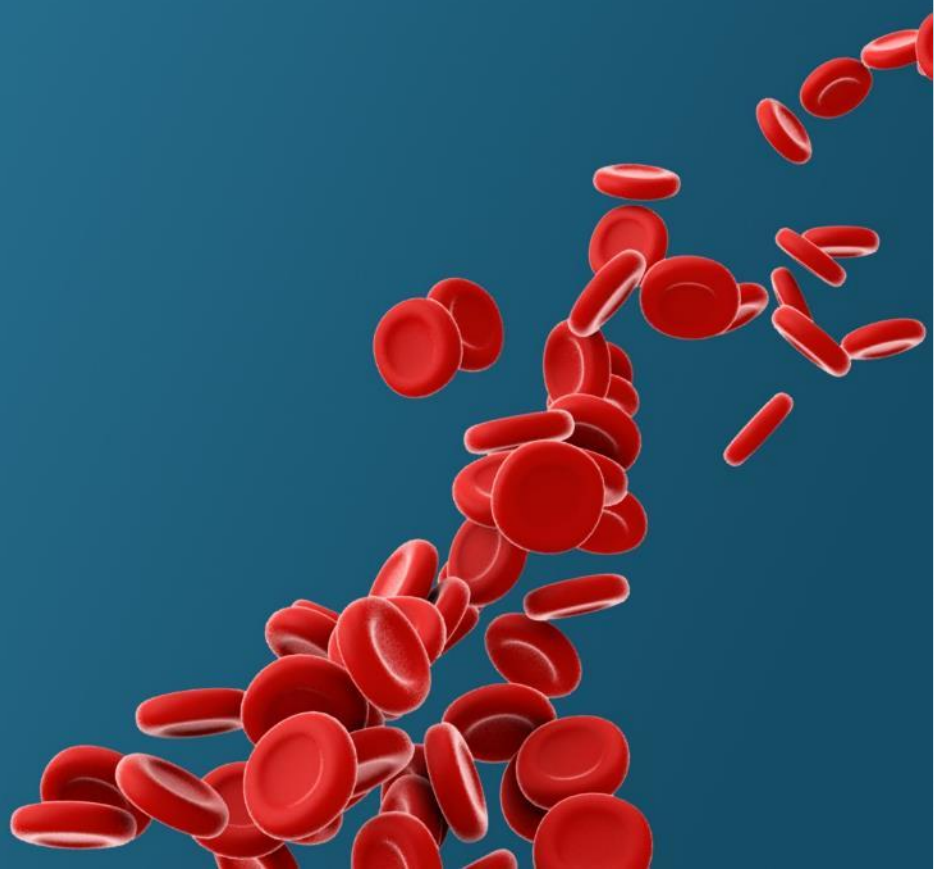
Sickle cell disease
2026
Potential U.S. approval

OUR GOAL
Deliver a novel oral therapy that improves anemia and reduces VOCs

PYRUKYND® is approved in the U.S., EU, and Great Britain for adult pyruvate kinase (PK) deficiency and is under investigation for pediatric PK deficiency, thalassemia, and sickle cell disease.
Source: Agios internal estimates

Financial Overview

Cecilia Jones
Chief Financial Officer



Second quarter 2024 financial results

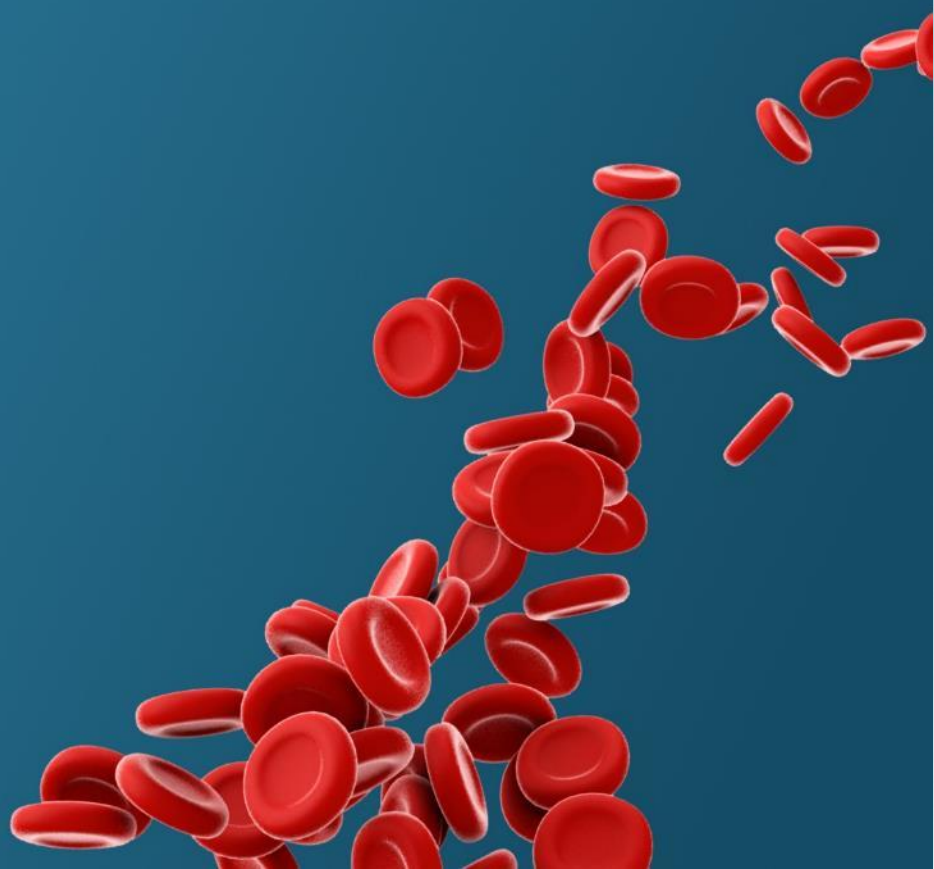
Statement of Operations	Three Months Ended 6/30/24	Three Months Ended 6/30/23
PYRUKYND® Net Revenue	$8.6M	$6.7M
Cost of Sales	$1.5M	$1.1M
Research & Development Expense	$77.4M	$68.9M
Selling, General & Administrative Expense	$35.5M	$30.4M
Net Loss	($96.1M)	($83.8M)

Balance Sheet	6/30/24	12/31/23
Cash, Cash Equivalents and Marketable Securities*	$645.3M	$806.4M

*Agios to receive a total of $1.1 billion in payments upon the potential FDA approval of vorasidenib (PDUFA: August 20, 2024). Agios retains a 3% royalty on annual U.S. net sales greater than $1 billion.

Closing Remarks

Brian Goff
Chief Executive Officer



Growing stack of Phase 3 data readouts positions mitapivat to become a multi-billion-dollar franchise

2024

Thalassemia
PYRUKYND®
Phase 3 ENERGIZE readout

Thalassemia
PYRUKYND®
Phase 3 ENERGIZE-T readout

Pediatric PK Deficiency
PYRUKYND®
Phase 3 ACTIVATE-KidsT readout

2025

Sickle Cell Disease
PYRUKYND®
Phase 3 RISE UP readout

Thalassemia
PYRUKYND®
Potential approval

Pediatric PK Deficiency
PYRUKYND®
Phase 3 ACTIVATE-Kids readout

2026

Sickle Cell Disease
PYRUKYND®
Potential approval

Pediatric PK Deficiency
PYRUKYND®
Potential approval

Well-positioned with multiple clinical and regulatory catalysts to enter multi-billion-dollar markets and deliver significant value

PKa franchise with multi-billion-dollar potential	Differentiated mechanism of action	Increasing probability of success	Growing pipeline
Large opportunities with substantial value - potential for two additional **first and best-in-class** indications for PYRUKYND® by 2026	**Clearly differentiated** PK activation franchise targeting red blood cell health **beyond hemoglobin** increase	Proven track record supported by **compelling and consistent data** to date	Diversified pipeline addressing the underlying pathophysiology of **rare diseases with high unmet need**

$645.3M in cash and equivalents as of June 30, 2024

Agios to receive a total of $1.1 billion in payments upon the potential FDA approval of vorasidenib (PDUFA: August 20, 2024)

Pka = pyruvate kinase activation

agios

Thank You