agios

Agios Investor Webcast

June 12, 2020

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 Agios' use of proceeds from the transaction with RPI; developments regarding Agios' collaboration agreement with Celgene; the potential benefits of mitapivat; Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development, including mitapivat; Agios' key milestones and guidance for 2020; Agios' plans regarding future data presentations; Agios' 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. For example, there can be no guarantee that any product candidate Agios or its collaborators 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; 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.

Agios Conference Call Participants

TOPIC	PARTICIPANT
Opening Remarks	Dr. Jackie Fouse, Chief Executive Officer
Mitapivat Mechanism-of-Action & Clinical Development Plan	Dr. Chris Bowden, Chief Medical Officer
Thalassemia Overview & Review of Interim Results from the Phase 2 Study of Mitapivat in Thalassemia	Dr. Kevin Kuo, University Health Network, University of Toronto
Sickle Cell Disease Overview & Review of Proof-of-Concept Data for Mitapivat in Sickle Cell Disease	Dr. Swee Lay Thein, National Institutes of Health
Q&A	Dr. Kuo, Dr. Thein, Dr. Bowden, Dr. Fouse, Darrin Miles, SVP of U.S. Commercial and Global Marketing, and Andrew Hirsch, Chief Financial Officer and Head of Corporate Development


agios
AGIOS 2025 VISION:
Focused Innovation. Ambitious Development.
Transformative Treatments for Patients Across Three Focus Areas.
4
MEDICINES
8+
INDICATIONS
6+
MOLECULES IN THE CLINIC
$
CASH FLOW POSITIVE

New Clinical Data Support Significant Expansion of Mitapivat Program

Mitapivat data in thalassemia support broad pivotal development

- α- and β-thalassemia pivotal development plan expected to be finalized by YE 2020; pivotal program to be initiated in 2021

Proof-of-concept established for mitapivat in sickle cell disease (SCD)

- Data support pivotal development in SCD; pivotal program to be initiated in 2021

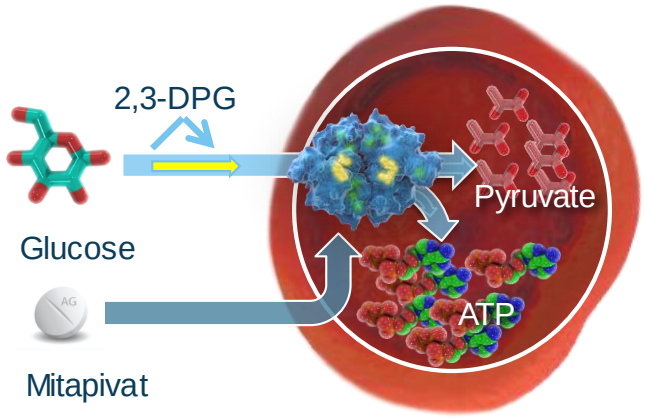
Monetization of IDHIFA royalty provides $255M non-dilutive capital

- Additional capital supports pivotal development of mitapivat in both thalassemia and SCD

PKR Activation Program Overview

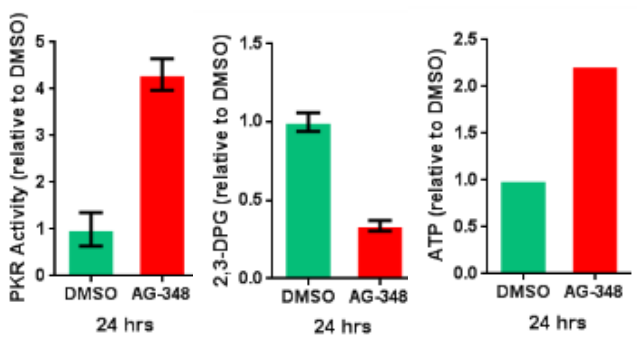
Dr. Chris Bowden, Chief Medical Officer

Transforming Chronic Hemolytic Anemias with a Small Molecule

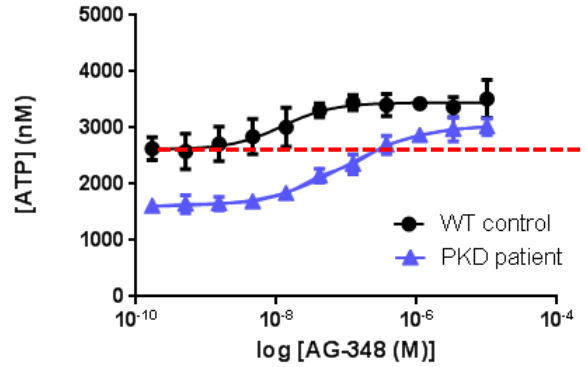


DRIVEPK

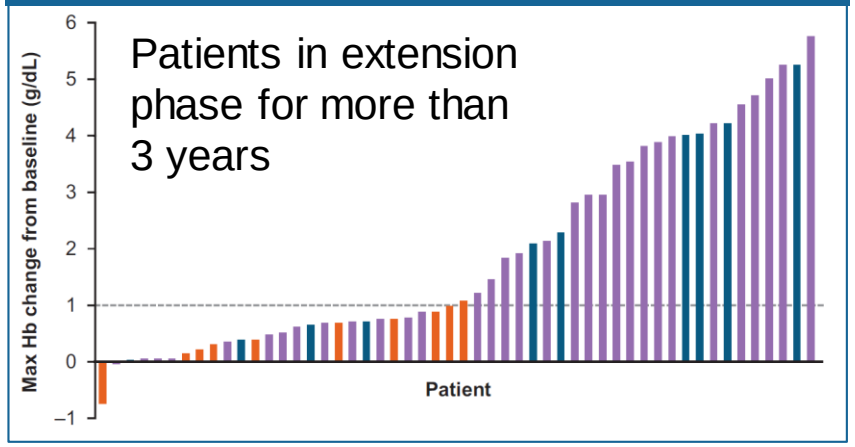
Patient A (R510Q/G511R)

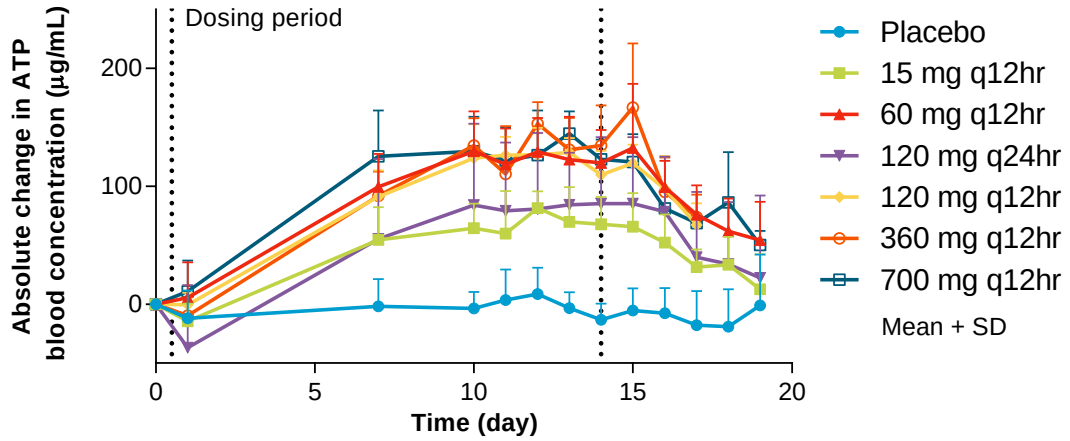


WT vs PKD ATP levels RBCs treated with AG-348 24 hrs



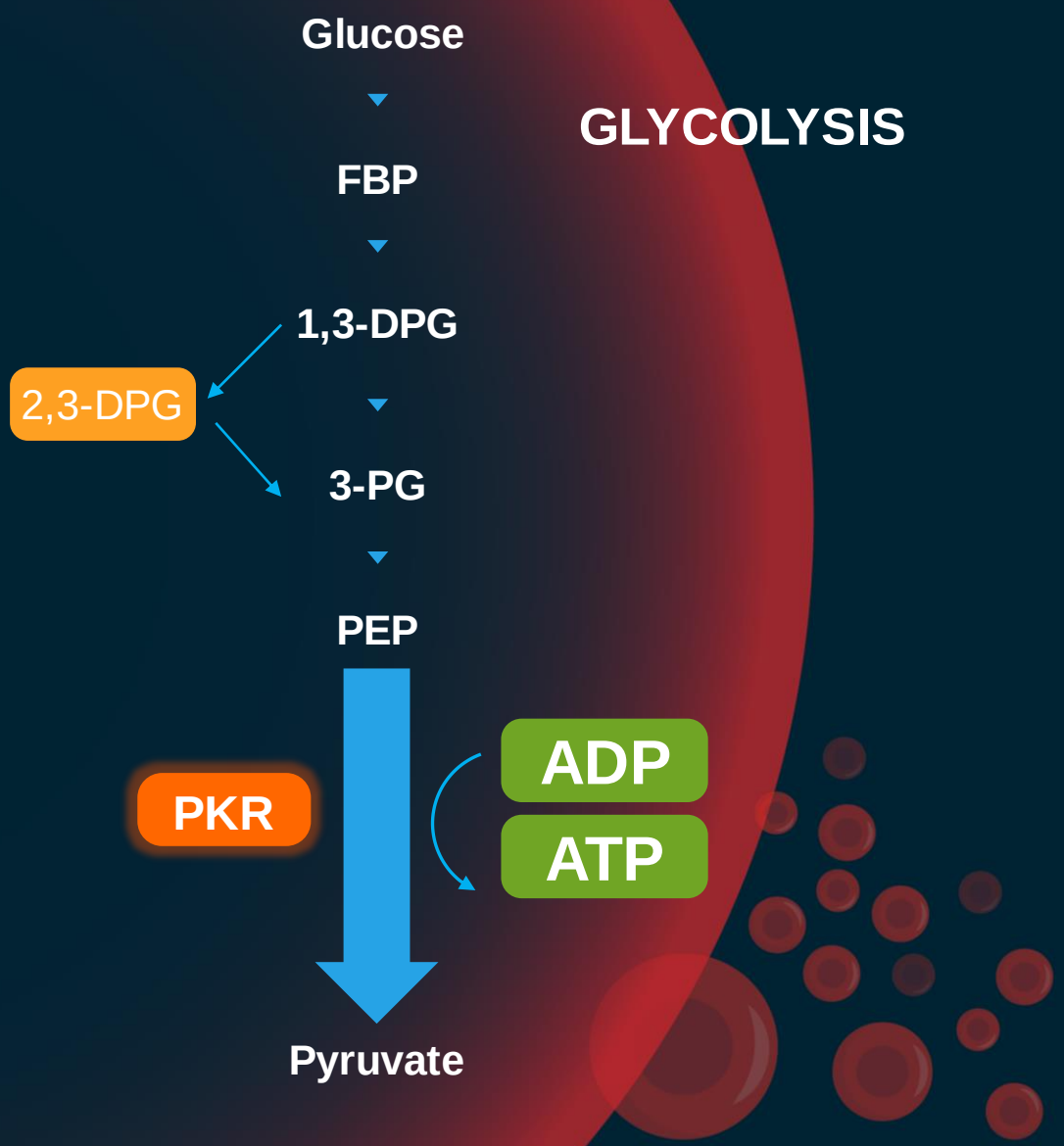
Maximum Hb change from baseline





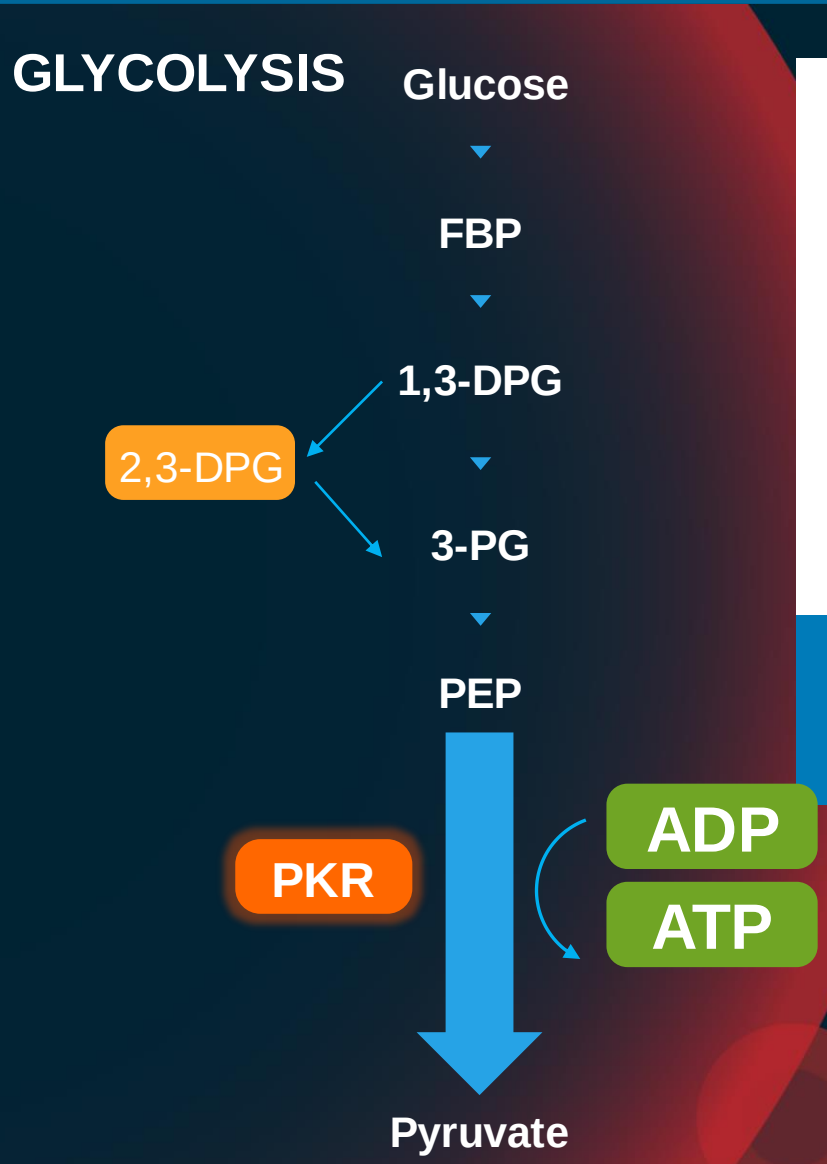
ASH 2014; EHA 2016; EHA 2018; ASH 2019

The Role of Pyruvate Kinase-R in Glycolysis



- Pyruvate kinase-R (PKR) is:
  - The red blood cell (RBC)-specific form of PK
  - One of two PK isoenzymes expressed in human tissues from the *PKLR* gene
  - A key enzyme for maintaining energy homeostasis in red blood cells, which rely almost exclusively on glycolysis to generate ATP
- Glycolysis is critical in maintaining erythroid cellular health and differentiation
- PKR catalyzes the final step in glycolysis
- Levels of 2,3-DPG and ATP are often dysregulated in hemolytic anemias

The Role of Pyruvate Kinase-R in Glycolysis



Normal Red Cell

PEP

wtPKR

Pyruvate

Cellular Demand

ATP Production Meets Demand

Mutant PKR Pyruvate Kinase Deficiency

PEP

mPKR

Pyruvate

Cellular Demand

Inadequate Production of ATP

- PKR mutations decrease PK stability, ATP generation and RBC membrane integrity and increase RBC destruction, leading to chronic hemolytic anemia

Wildtype PKR Other Hemolytic Anemias

PEP

wtPKR

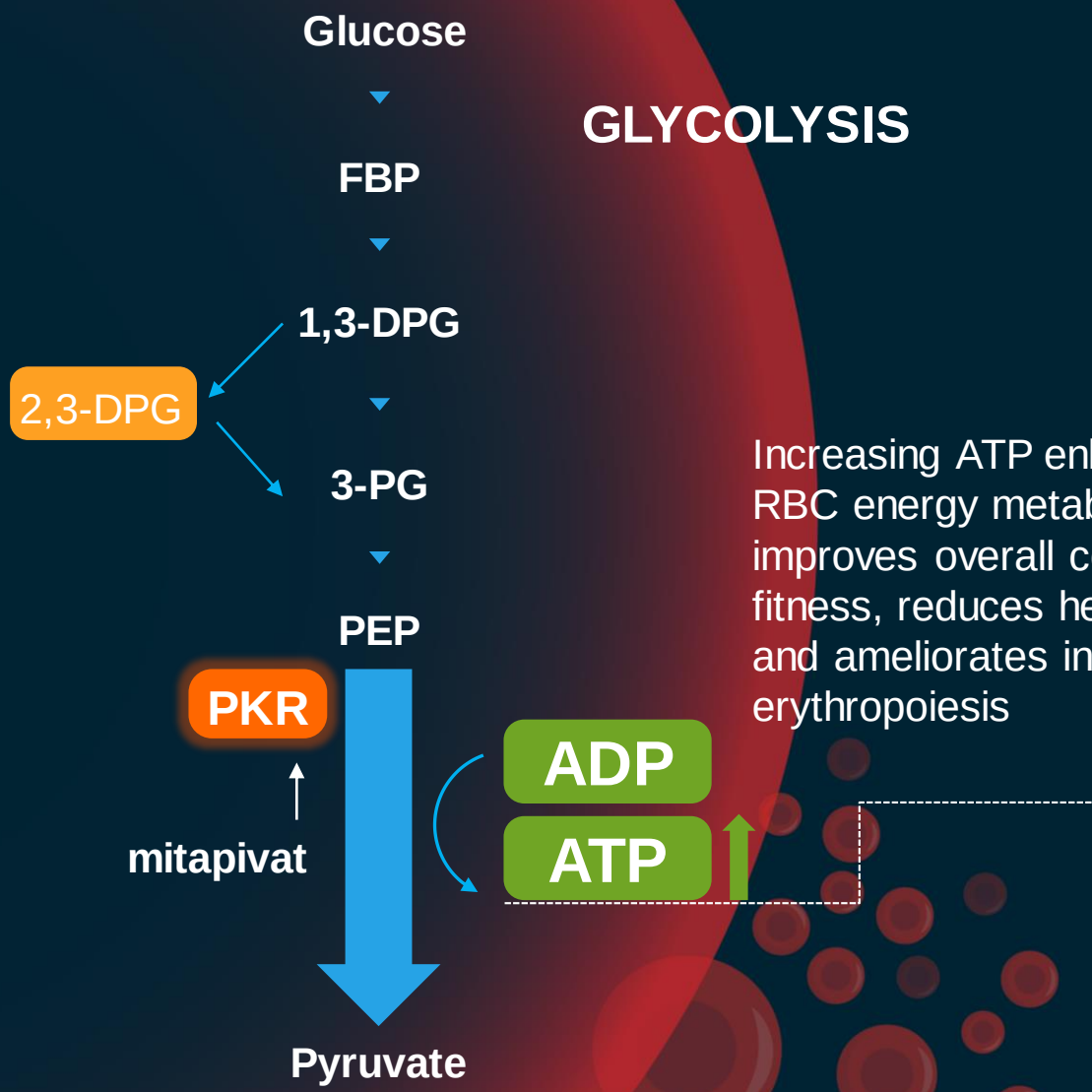
Pyruvate

Cellular Demand

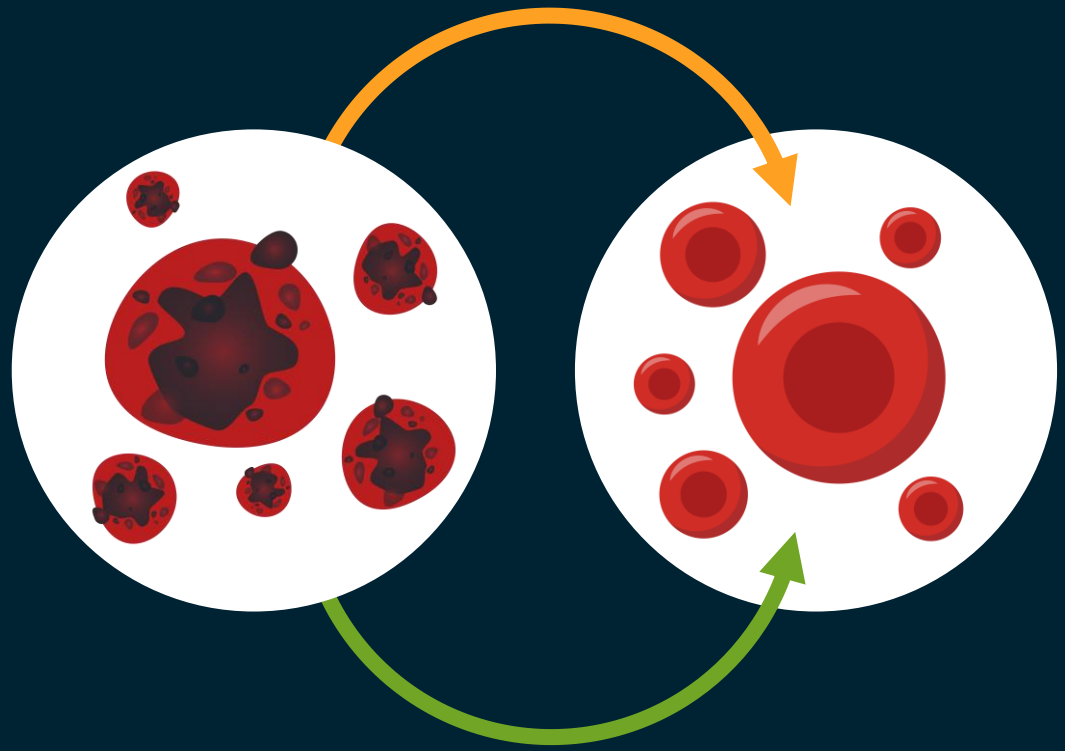
Increased Demand of ATP

- In other hemolytic anemias, there is an increase in ATP demand and impaired ATP production, leading to damage and premature death of RBCs, hemolysis and anemia

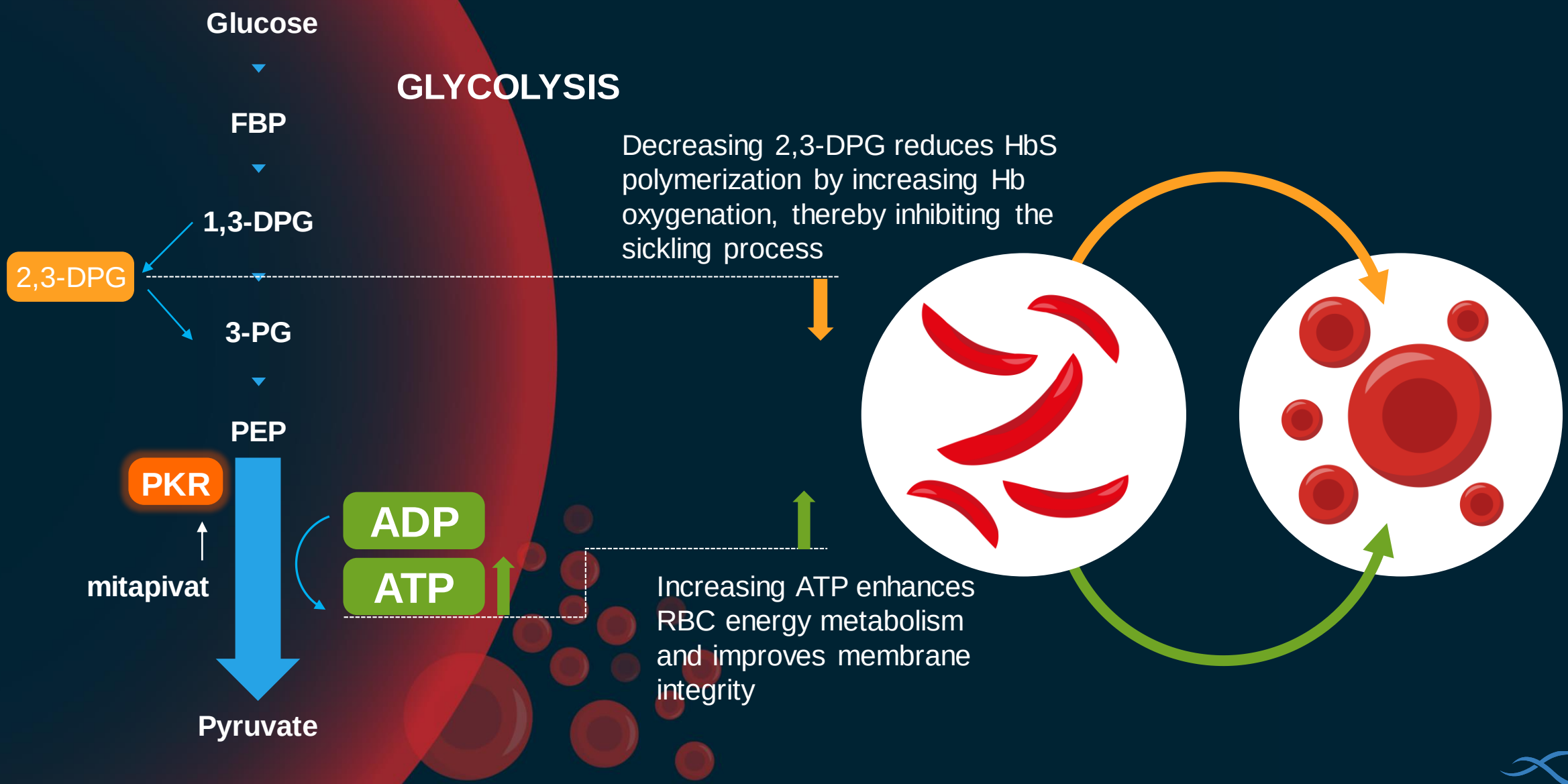
PKR Activators May Improve Thalassemic RBC Production and Survival by Increasing ATP Production



Increasing ATP enhances RBC energy metabolism, improves overall cell fitness, reduces hemolysis and ameliorates ineffective erythropoiesis



PKR Activation in Sickle Cell Disease Modulates 2,3-DPG and ATP to Improve Anemia and Reduce Sickling



PKR Activation Has Potential Broad Utility Across Hemolytic Anemias

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

Pyruvate Kinase Deficiency

NTD Adult PKD	Phase 3 enrollment complete; Topline data expected YE 2020 – mid 2021
TD Adult PKD	Phase 3 enrollment complete; Topline data expected YE 2020 – mid 2021
Pediatric PKD	Pivotal plan expected by YE

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

β- and α-Thalassemia

NTD β- and α-Thalassemia	Phase 2 enrollment complete
Thalassemia	Pivotal plan expected by YE and initiation in 2021

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

Sickle Cell Disease

Adult SCD	NIH CRADA; data to be submitted to ASH
Adult SCD	Pivotal study expected to initiate in 2021

Source: Agios estimates, market research

Thalassemia Overview

Dr. Kevin Kuo, University Health Network, University of Toronto

What is Thalassemia?

Thalassemia is an inherited blood disorder that reduces the production of functional hemoglobin, the protein in red blood cells that carries oxygen. This causes a shortage of red blood cells and low levels of oxygen in the bloodstream, leading to a variety of health problems.

Two main types of thalassemia, **alpha** thalassemia and **beta** thalassemia

Alpha thalassemia is caused by mutations in alpha globin

Beta thalassemia is caused by mutations in beta globin

~18-23K thalassemia patients in the U.S. and EU

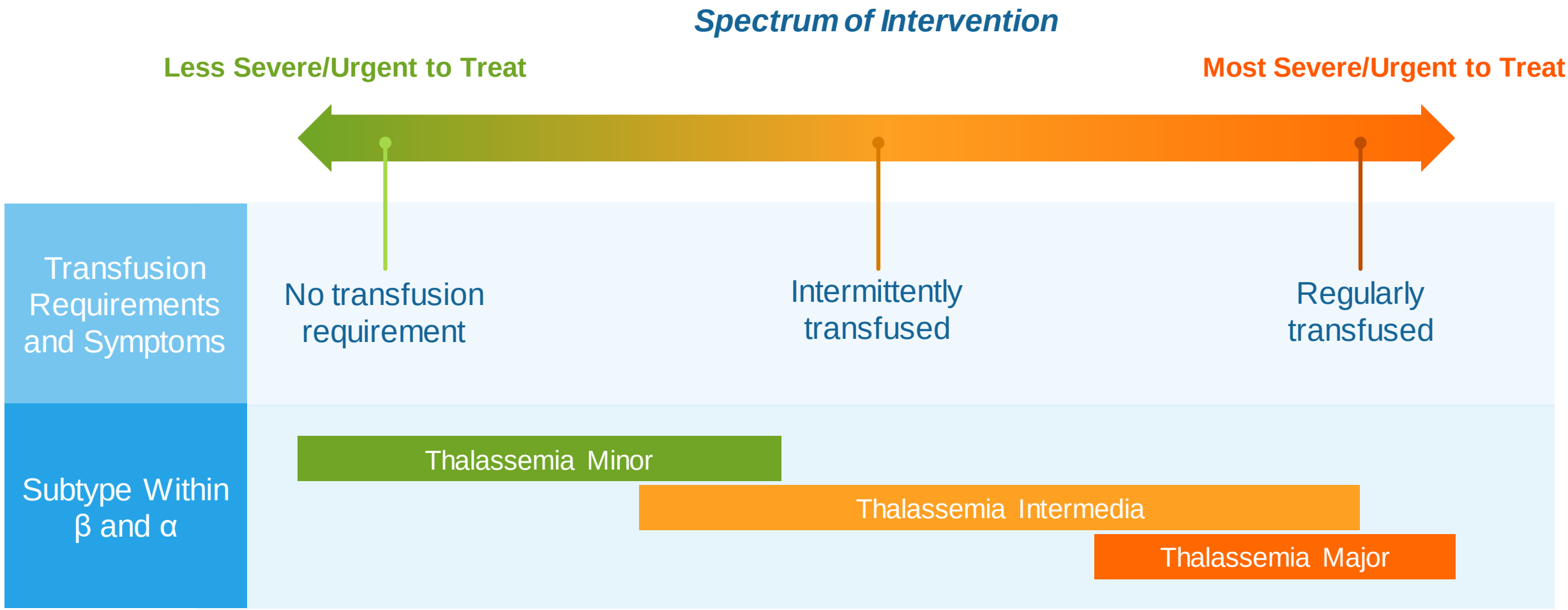
~70% of beta thalassemia is in EU vs. ~30% in the US

Alpha thalassemia is split ~50% US vs. ~50% EU

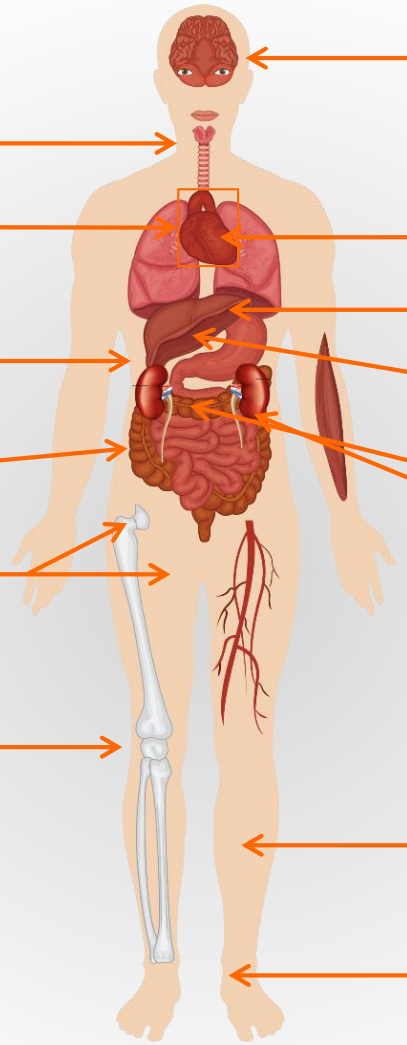
Beta thalassemia is split approximately 60/40 transfusion dependent (TD) / non-transfusion dependent (NTD) with the TD patients reflecting the most severe phenotype

Agios market research; Thuret, et.al., Haematologica 2010;95:724-729

Thalassemia Patients Typically Categorized by Degree of Transfusion Dependence



Complications from Thalassemia Occur Regardless of Transfusion Status



Hypothyroidism
Hypoparathyroidism

Cardiac siderosis
Left-sided heart failure

Hepatic failure
Viral hepatitis

Diabetes mellitus

Hypogonadism

Osteoporosis

Transfusion reactions
Alloimmunization

Silent cerebral ischemia

PHT
Right-sided heart failure

Extramedullary
hematopoietic pseudotumors

Hepatic fibrosis, cirrhosis, and cancer

Gallstones

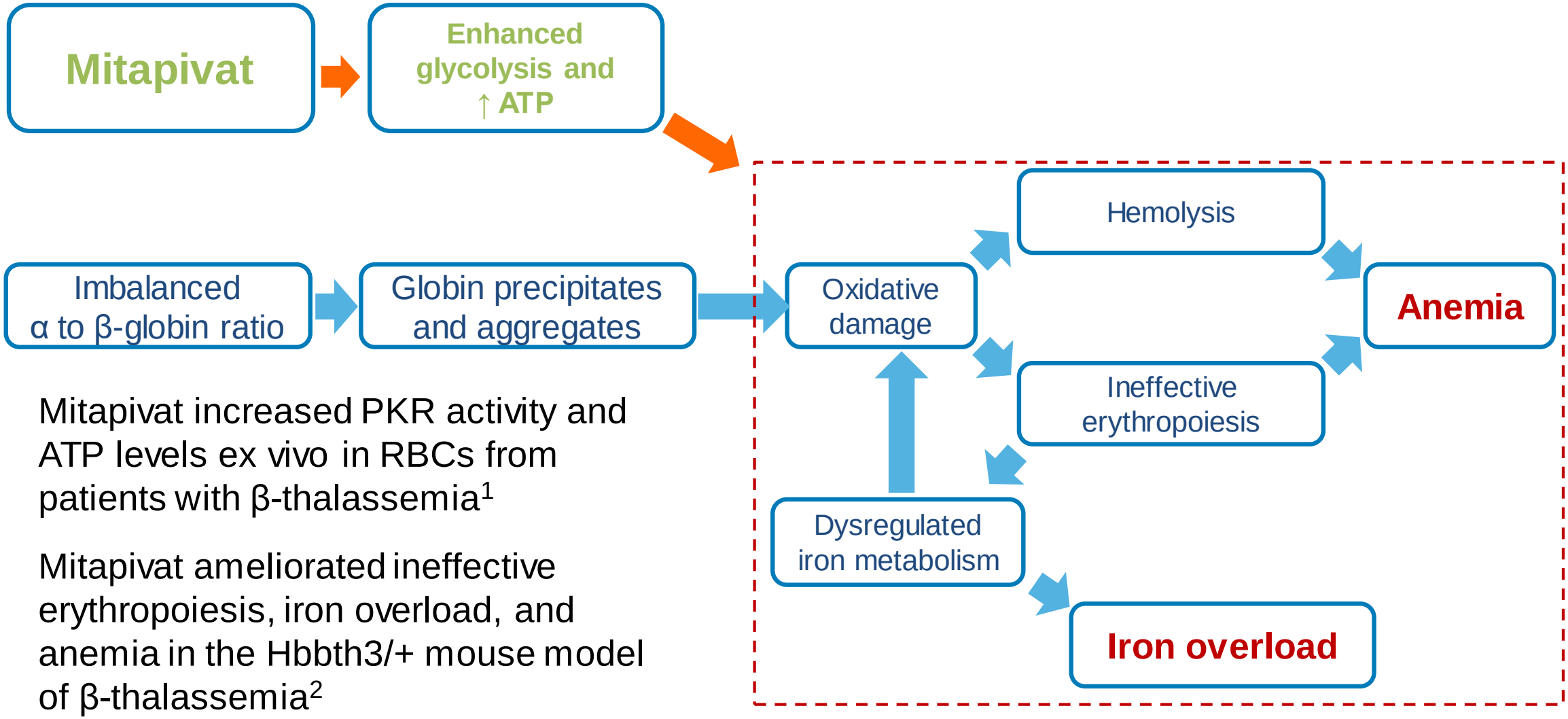
Splenomegaly

Venous thrombosis

Leg ulcers

Musallam. Acta Haematol. 2013;130:64. Musallam. Haematologica. 2013;98:833.

Hypothesis: Mitapivat Mechanism in Thalassemia



- Mitapivat increased PKR activity and ATP levels ex vivo in RBCs from patients with β-thalassemia[1]
- Mitapivat ameliorated ineffective erythropoiesis, iron overload, and anemia in the Hbbth3/+ mouse model of β-thalassemia[2]

1. Rab MAE et al. *ASH Congress* 2019, Abstract 3506.
2. Matte A et al. *EHA Congress* 2016, Abstract S135.

Proof of concept for the oral pyruvate kinase activator mitapivat in adults with non–transfusion-dependent thalassemia: Interim results from an ongoing, phase 2, open-label, multicenter study

Kevin HM Kuo1, D Mark Layton2, Ashutosh Lal3, Hanny Al-Samkari4, Feng Tai5,

Megan Lynch5, Katrin Uhlig5, Elliot P Vichinsky3

1Toronto General Hospital, University Health Network, Toronto, ON, Canada; 2Hammersmith Hospital, Imperial College Healthcare NHS Trust, London, UK; 3UCSF Benioff Children's Hospital Oakland, Oakland, CA, USA; 4Massachusetts General Hospital, Harvard Medical School, Boston, MA, USA; 5Agios Pharmaceuticals, Inc., Cambridge, MA, USA

Disclosures

This study was funded by Agios Pharmaceuticals, Inc. We would like to thank the patients taking part in this study.

Kevin HM Kuo – Agios, Apellis, Bluebird Bio, Celgene, Pfizer – consultant; Alexion, Novartis – consultant, honoraria; Bioverativ – data safety monitoring board member; Pfizer – research support. **D Mark Layton** – Agios, Novartis – consultant and advisory board member; Cerus – data safety monitoring board member. **Ashutosh Lal** – none. **Hanny Al-Samkari** – Agios, Dova, Moderna – consultant; Agios, Dova, Amgen – research funding. **Feng Tai, Megan Lynch,** and **Katrin Uhlig** – Agios – employment and stockholder. **Elliot P Vichinsky** – GBT, Pfizer, Novartis, Bluebird Bio, Agios – consultant and research funding

Editorial assistance was provided by Christine Ingleby, PhD, CMPP, Excel Medical Affairs, Horsham, UK, and supported by Agios

12 June 2020
S297: New therapeutic approaches for thalassemia

Study design: Open-label, phase 2, multicenter study

Key Inclusion Criteria

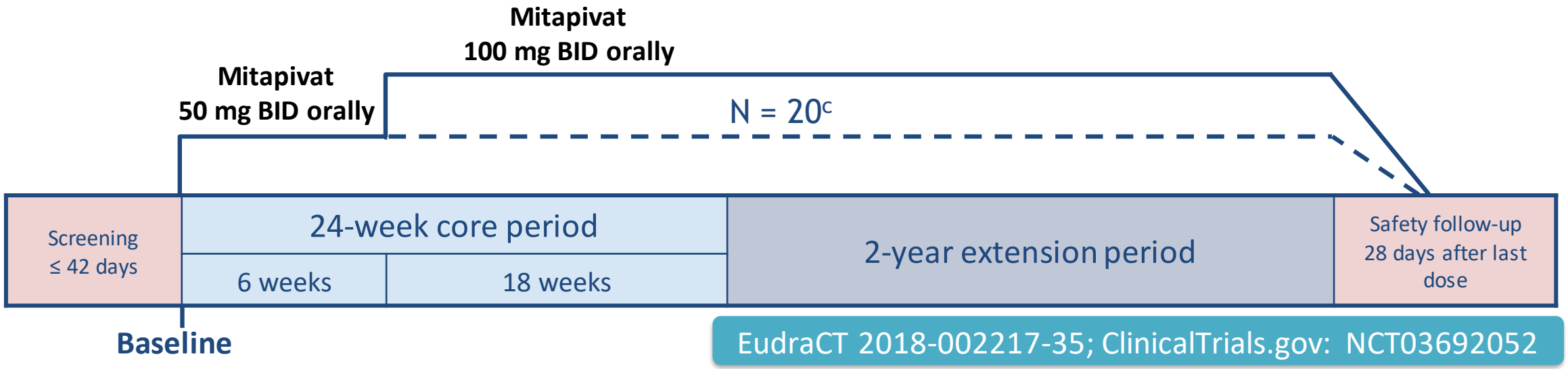
- β-thalassemia ± α-globin gene mutations, HbE β-thalassemia, or α-thalassemia (HbH disease)
- Hb ≤ 10.0 g/dL
- Non–transfusion-dependent[a]

Primary Endpoint[b]

- Hb response, defined as increase of ≥ 1.0 g/dL from baseline at any time between weeks 4–12, inclusive

Secondary/Exploratory Endpoints

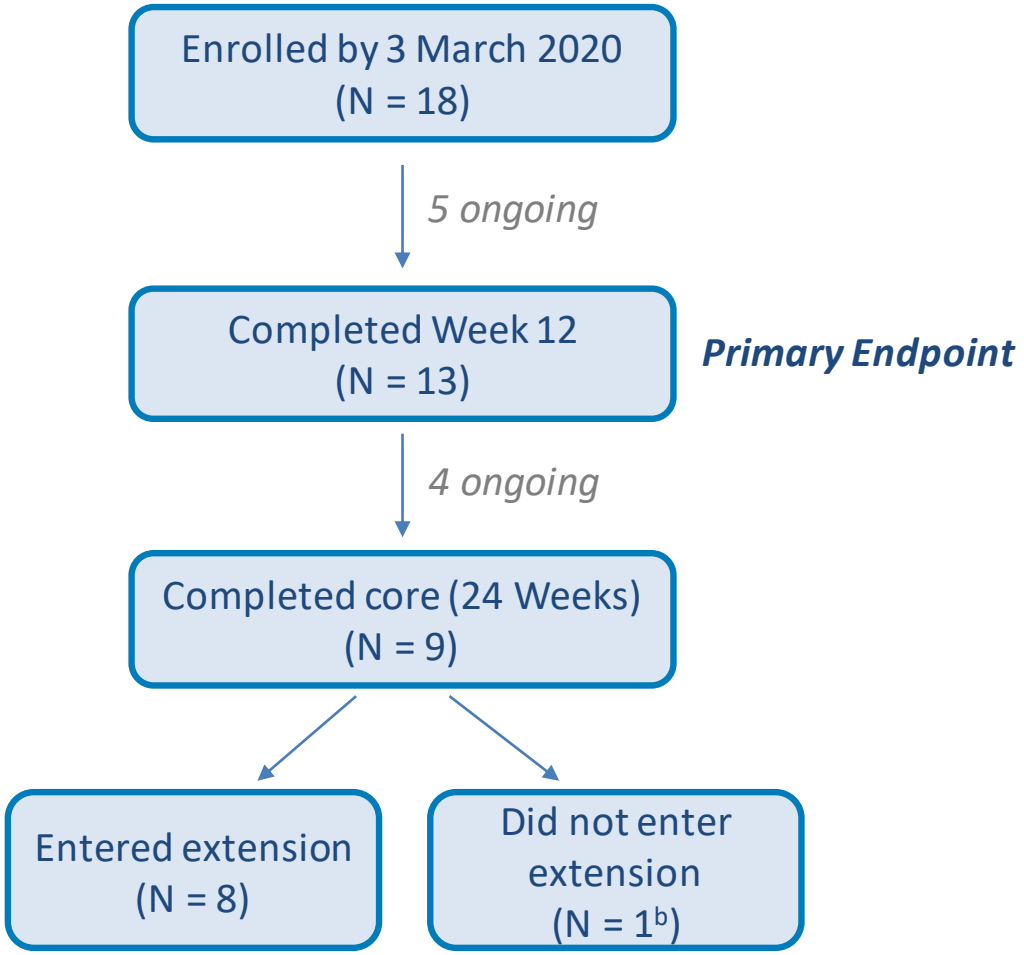
- Sustained Hb response; delayed Hb response; markers of hemolysis; hematopoietic activity; safety



[a]≤ 5 RBC units transfused in the preceding 24 weeks and none in the 8 weeks prior to study drug. [b]With the originally planned sample size of 17 patients enrolled, the study would have 80% power to reject a ≤ 30% response rate at a one-sided 0.05 type 1 error rate. [c]Fully enrolled. BID = twice daily

Demographics and disposition

Baseline characteristics		Total (N = 18)
Median (range) duration of treatment, weeks		20.6 (1.1–50.0)
Male/female, n		5/13
Age at informed consent, median (range), years		43.5 (29–67)
Race, n (%)	Asian	9 (50.0)
	White	4 (22.2)
	Native Hawaiian or other Pacific Islander	1 (5.6)
	Other[a]	4 (22.2)
Thalassemia type, n (%)	α	5 (27.8)
	β	13 (72.2)
Hb baseline, median (range), g/dL		8.43 (5.6–9.8)
Indirect bilirubin, median (range), mg/dL		1.17 (0.31–5.52)
Lactate dehydrogenase, median (range), U/L		249 (126–513)
Erythropoietin, median (range), mU/mL		70.5 (15–11,191)



Hydroxyurea, splenectomy, and prior transfusions were reported in two patients each at baseline
[a]Includes patients who reported more than one category, and one not reported. [b]Investigator decision

Key efficacy results

Primary endpoint was met in 92.3% of patients

Endpoint	Genotype	N/N	%	90% CI
Hb responders during weeks 4–12 among those who completed 12 weeks	All	12/13	92.3	68.4, 99.6
	α	4/4	100	47.3, 100
	β	8/9	88.9	57.1, 99.4
Hb responders during weeks 12–24 among those who completed 24 weeks	β[a]	8/9	88.9	57.1, 99.4
Sustained responders: primary response and ≥ 2 Hb responses during weeks 12–24	β[a]	7/8	87.5	52.9, 99.4

Hb responder defined as a ≥ 1.0 g/dL Hb increase from baseline at least once

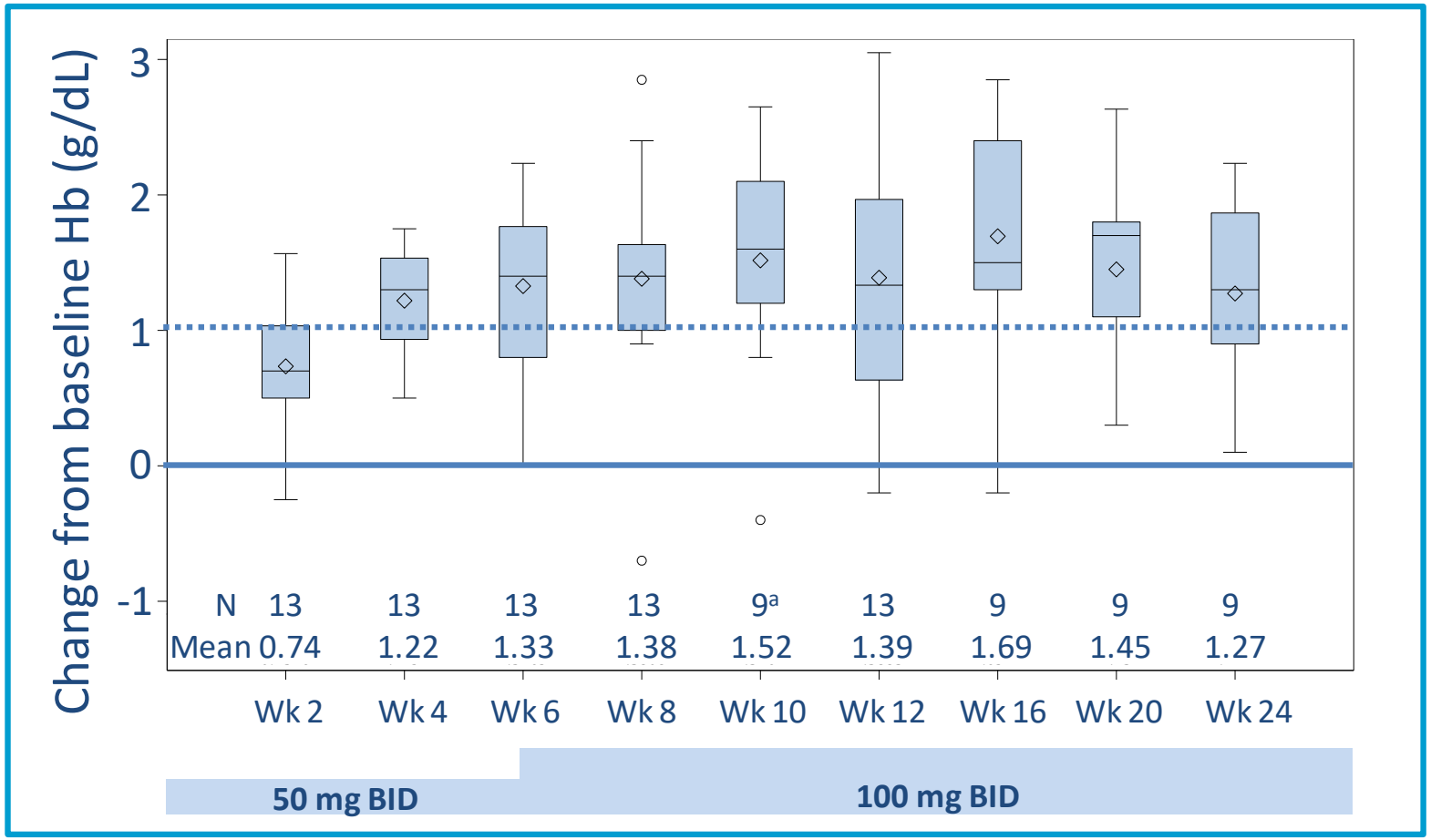
[a] Only patients with β-thalassemia had completed 24 weeks of treatment at the time of datacut

Hemoglobin change from baseline

Patient population	N	Weeks	Mean (SD) change from baseline Hb, g/dL
All patients	13	4–12	1.34 (0.7)
α-thalassemia	4	4–12	1.17 (0.4)
β-thalassemia	9	4–24	1.43 (0.8)

Median (range) time to Hb increase of ≥ 1 g/dL among responders was 3.1 (1.4–7.1) weeks

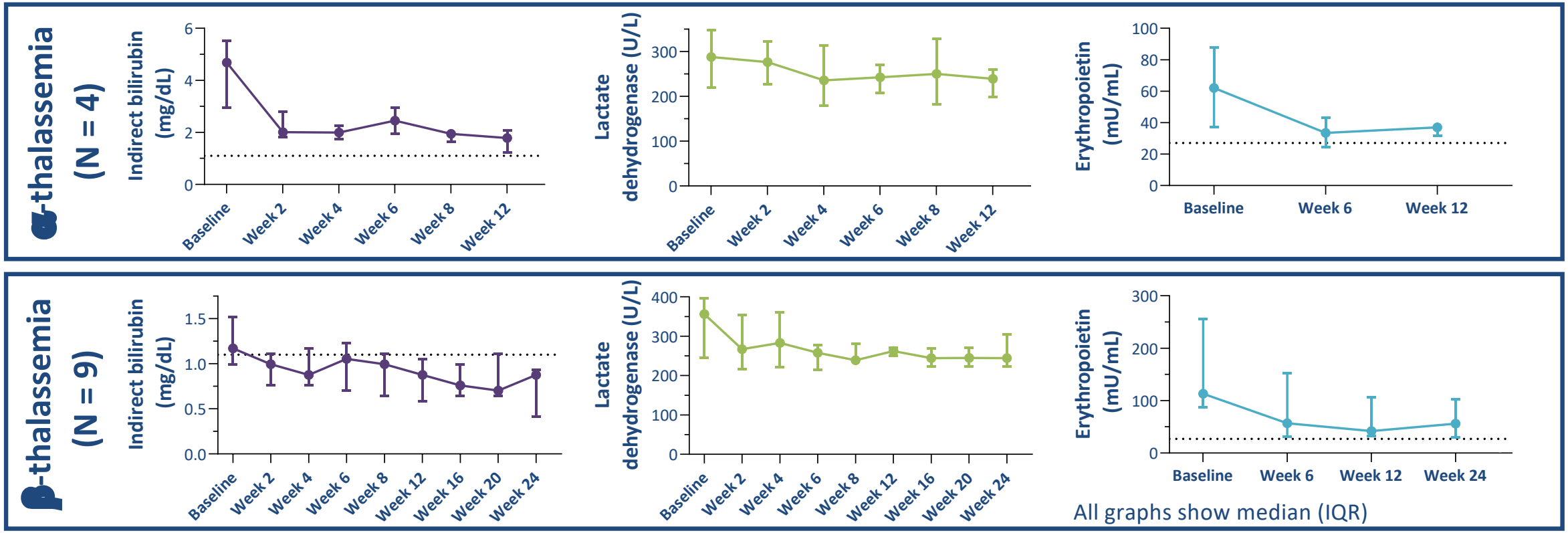
Hemoglobin change over time



Solid blue line indicates baseline, dashed blue line indicates Hb change required for primary endpoint. Boxes represent inter quartile range, lines indicate medians, diamonds indicate means, whiskers and outliers (circles) calculated with Tukey's method. [a]4 patients were not evaluated at Week 10 due to a protocol amendment eliminating this visit
Wk = week

Markers of hemolysis and erythropoiesis

– The improvements in these markers correlated with the Hb increases



Dashed lines indicate upper limit of normal range. For α-thalassemia: N = 4 for lactate dehydrogenase and erythropoietin, for indirect bilirubin N = 3 at baseline, weeks 2, 8 and 12, and N = 2 at weeks 4 and 6; for β-thalassemia: N = 9 for erythropoietin, for lactate dehydrogenase N = 9 at baseline, weeks 6, 8, 12, and 20 and N = 8 at weeks 2, 4, 16, and 24, for indirect bilirubin N = 9 at baseline and N = 7 at the remaining times. IQR = interquartile range (25^{th}–75^{th} centiles)

ATP change with mitapivat

- Mean ATP percent increase from baseline was similar to that previously observed with mitapivat in healthy volunteers[1]

Treatment dose	Visit	Mean (CV%) ATP change from baseline in blood, %
50 mg	Week 6 (n = 9)	82.7 (85.8)
100 mg	Week 8 (n = 12)	76.8 (62.7)
100 mg	Week 12 (n = 12)	86.7 (68.7)
100 mg	Week 24 (n = 5)	92.3 (71.6)

1. Yang H et al. *Clin Pharmacol Drug Dev* 2019;8:246.
CV% = coefficient of variation, percent

Safety summary[a]

	Total (N = 18)
Patients with any AE, n (%)	13 (72.2)
Patients with any related AE, n (%)	11 (61.1)
Patients with AEs by maximum severity, n (%)	
Grade 1	4 (22.2)
Grade 2	7 (38.9)
Grade 3[b]	2 (11.1)

- There were no serious adverse events (AEs) and no AEs leading to treatment discontinuation as of the datacut
- Dose escalation to 100 mg BID was well tolerated and not associated with an increase in AEs
- Reported in one patient each:
  - AE leading to treatment interruption (grade 3, postural vertigo, not related)
  - AE leading to treatment modification (grade 2, bloating and heartburn, related)
- Post-datacut, one serious AE of renal dysfunction was reported, which resolved upon treatment discontinuation (grade 3, judged related by investigator)

AEs coded using MedDRA, version 22.0
[a]As of datacut of 3 March 2020
[b]Neither were judged related

Most common AEs[a]

AEs in ≥ 2 patients, number of patients (%)	Total (N = 18)
Insomnia	8 (44.4)
Dizziness	5 (27.8)
Cough	4 (22.2)
Dyspepsia	4 (22.2)
Fatigue	4 (22.2)
Headache	4 (22.2)
Nasal congestion	4 (22.2)
Nausea	4 (22.2)
Upper respiratory tract infection	4 (22.2)
Abdominal distension	3 (16.7)
Diarrhea	3 (16.7)
Ocular icterus	3 (16.7)
Oropharyngeal pain	3 (16.7)
Pain	3 (16.7)
Abdominal pain upper	2 (11.1)
Back pain	2 (11.1)
Pain in extremity	2 (11.1)
Pyrexia	2 (11.1)
Rash	2 (11.1)

- The safety profile was consistent with prior studies in healthy volunteers and patients with PK deficiency

[a]As of datacut of 3 March 2020
AEs coded using MedDRA, version 22.0

Conclusions

- This is the first clinical study evaluating PKR activation as a therapeutic option in α- and β-thalassemia, and is the first drug trial aimed at treating α-thalassemia
- Proof-of-concept was demonstrated
  - > 90% of patients met the primary endpoint showing a clinically significant Hb increase
  - All four α-thalassemia patients and eight of nine β-thalassemia patients were responders
  - A sustained Hb response was observed over time in patients with longer follow-up
  - Improvements in markers of hemolysis and erythropoiesis were consistent with mitapivat's mechanism of action
- Mitapivat was generally well tolerated; the safety profile was consistent with previous studies

These data indicate that activation of wild-type PKR by the oral agent mitapivat improved Hb and associated markers of hemolysis and erythropoiesis in patients with both α-and β-thalassemia, and that further investigation is warranted. Pivotal trials are in development.

Sickle Cell Disease Overview

Dr. Swee Lay Thein, National Institutes of Health

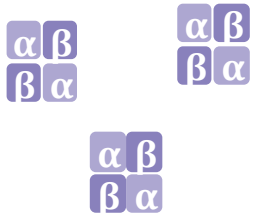
HbS Mutation Causes Hb Polymerization, Leading to RBC Sickling

- Sickle cell disease (SCD) is a rare blood disorder characterized by 2 key features: recurrent acute clinical events (most common is acute pain) and chronic anemia
- These pathological features are a direct result of sickled RBCs which are rigid, adhesive, and highly fragile
- Sticky sickled RBCs, together with other blood components cause microvascular occlusion - ischemia and depletion of the oxygen supply to tissues
- Fragile sickled cells cause chronic anemia
- There are ~100K patients in the U.S. and 35K patients in the EU5

Healthy

β-globin subunit from wild-type gene

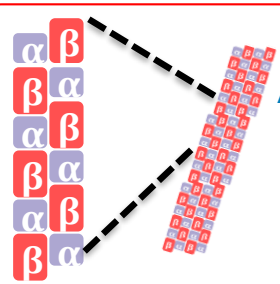
HbA
HbA molecules do not polymerize



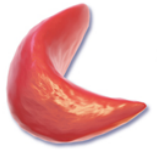
Healthy RBC
Retain flexible, biconcave shape
Full of individual HbA molecules

Sickle Cell Anemia

β globin subunit from mutant gene



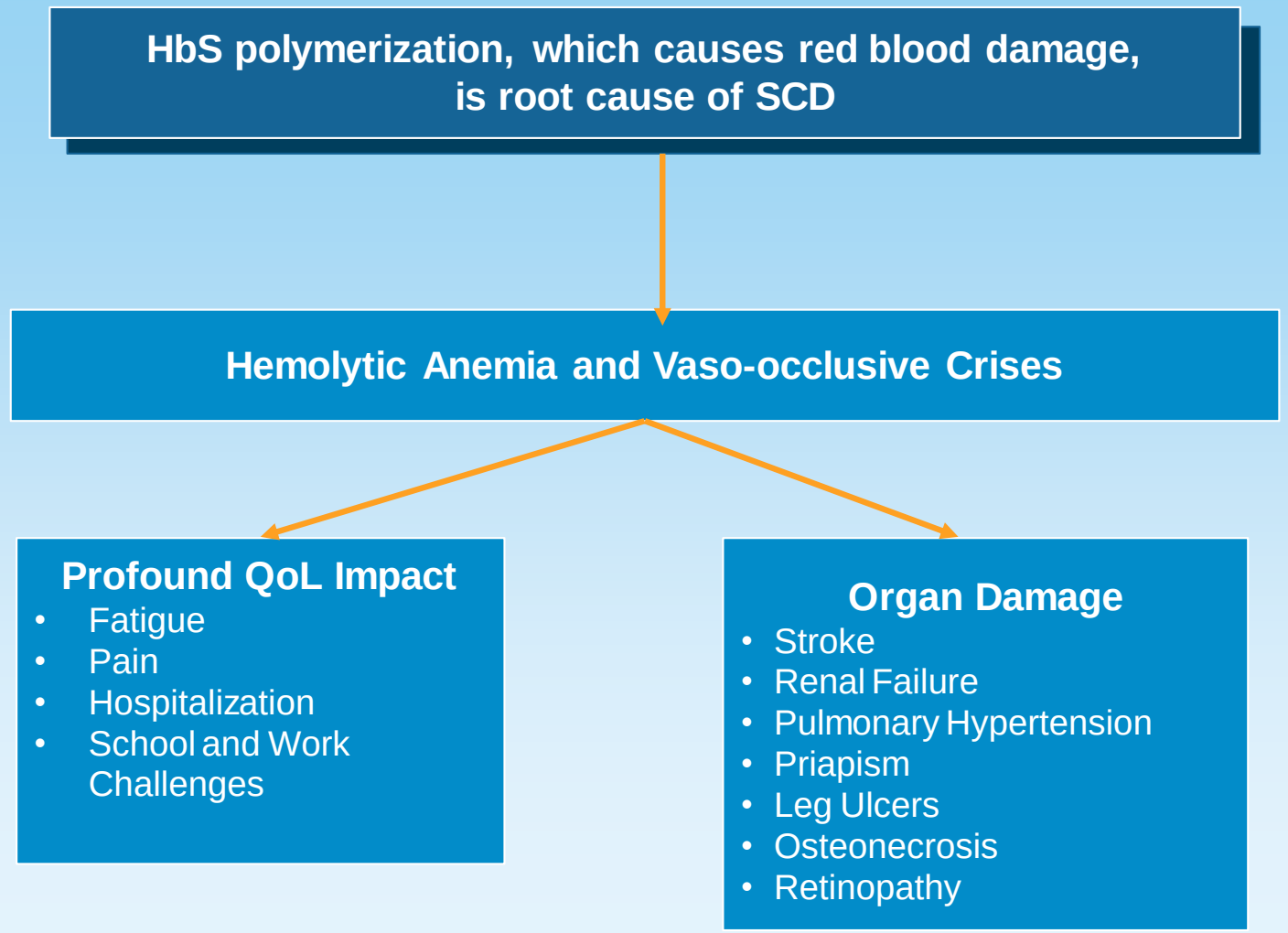
Abnormal 'sickle' HbS
When deoxygenated, molecules polymerize into a fiber



Sickled RBC
When deoxygenated, have elongated, rigid shape due to abnormal Hb fibers

Ware RE, et al. *Lancet*. 2017;390(10091):311-33.; RBC image: BruceBlaus [CC BY-SA 4.0 (https://creativecommons.org/licenses/by-sa/4.0)]; Agios Market Research

SCD Results in Morbidity and Mortality Via Distinct Pathways – Opportunity for Therapy That Addresses Hemolytic Anemia and Vaso-occlusion



HbS, sickle hemoglobin; SCD, sickle cell disease. Eaton, W. and Bunn, F. Targeting HbS Polymerization. *Blood.* 2017

Significant Impact of SCD on Patients

50%

Of patients have at least 1 VOC per year

<10 g/dL

Hb level of almost all patients

10-25%

Of patients are regularly transfused

~30 years

Shortened life expectancy

53%

Of adults have had a cerebral silent infarction

10-20 days

Sickle red cell life vs. 90-120 days for normal red cells

24%

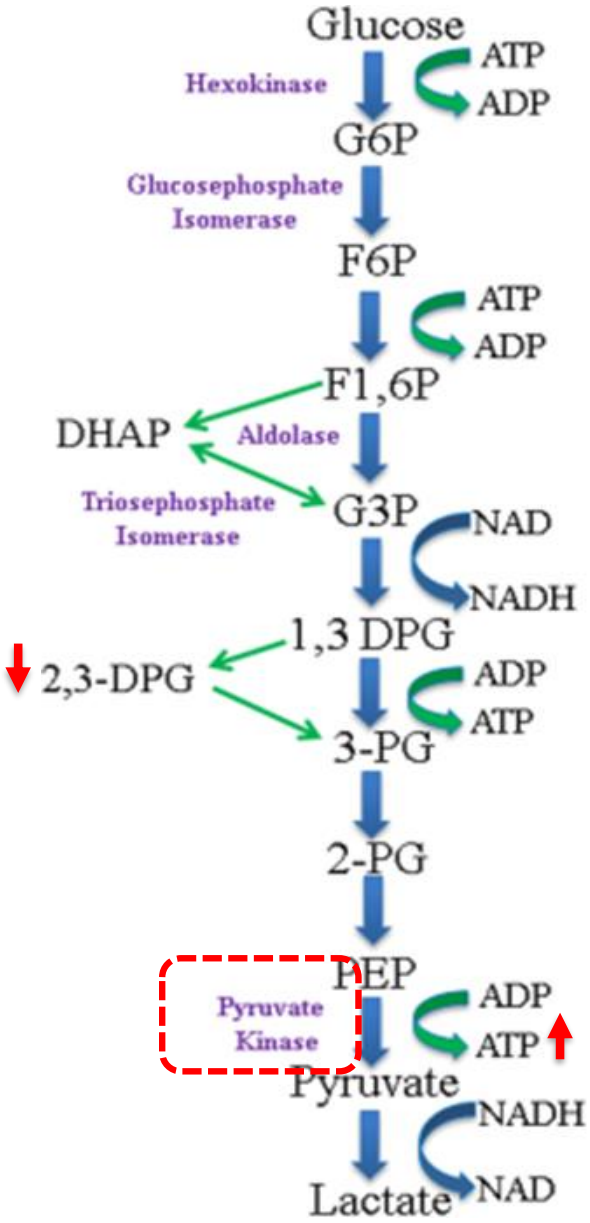
Patients have a stroke by 45 years

N Shah, et al, PLoS One. 2019;14(7): e0214355; Lanzkron, S. et al. Public Health Rep. Mar-Apr 2013; 128(2): 110-6; Kassim AA, et al. Blood. 2016; 127(16):2038-2040; Kanter J, Kruse-Jarres R. Blood Rev. 2013;27(6):279-287; Vichinsky, E. Hematology. 2017(1):435-439.; Agios market researh

Proof-of-concept Study of Mitapivat in Sickle Cell Disease

Dr. Swee Lay Thein, National Institutes of Health

Glycolytic Pathway within RBC



- Red cell longevity is dependent on ATP
- Glycolysis is the only source of ATP production in red blood cell (RBCs)
  - Pyruvate Kinase (PK), a key enzyme in the final step of glycolysis, generates 50% of the total red cell ATP
- RBCs in PK deficiency (PKD) are characterized by:
  - Increased levels of 2,3-DPG among other upstream glycolytic intermediates
  - Less intracellular ATP, leading to shortened RBC lifespan (increased hemolysis)
- PKD patients exhibit clinical manifestations of chronic hemolysis

NHLBI & Agios CRADA Study of Mitapivat in SCD: Study Design

Primary

Safety and tolerability

- Frequency and severity of adverse events
- Changes in laboratory parameters (including reticulocyte counts and levels of hemoglobin, bilirubin, and lactate dehydrogenase)

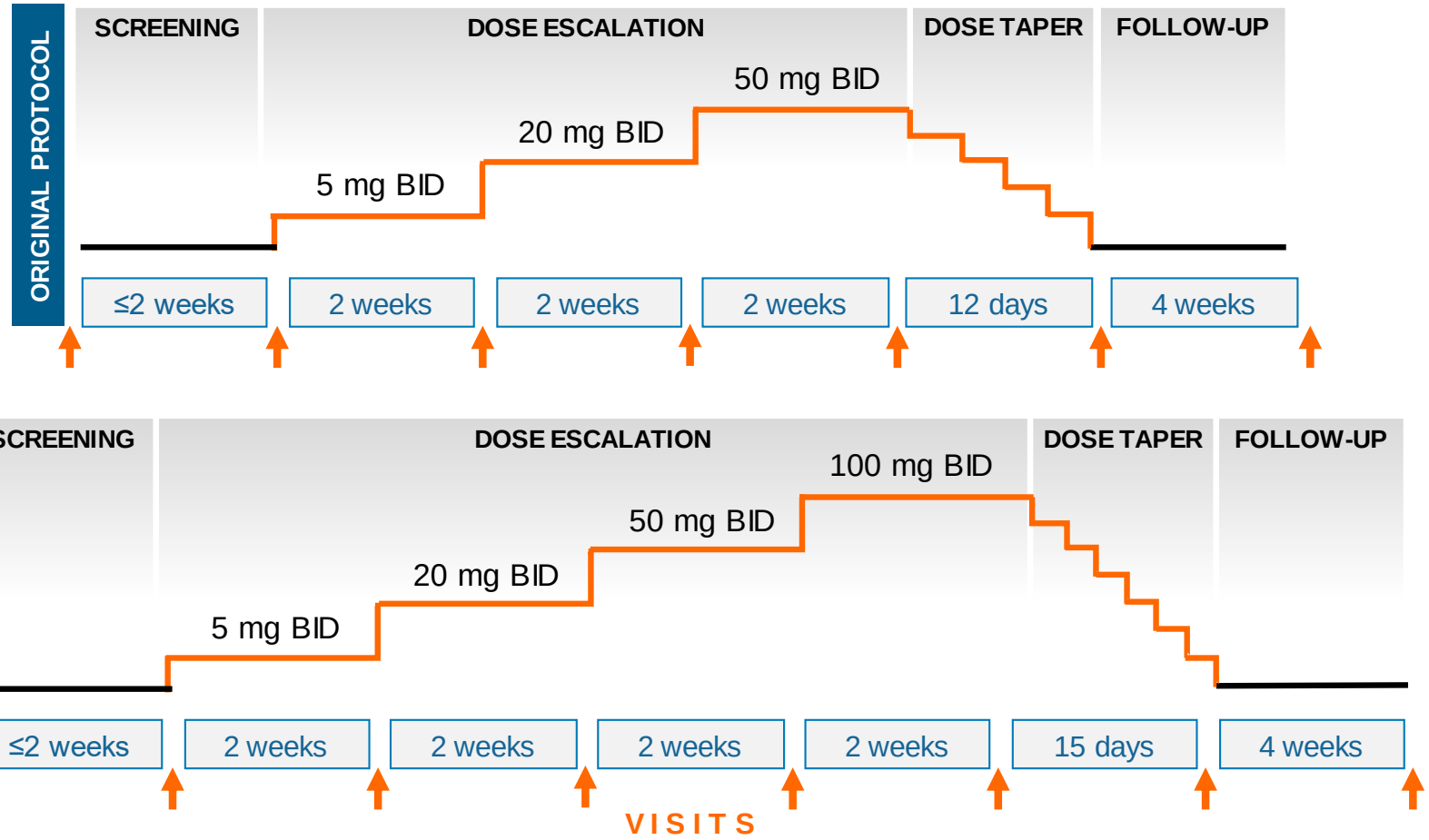
Secondary

Pharmacokinetics/pharmacodynamics

- Pharmacokinetics of mitapivat (AG-348)
- Levels of 2,3-DPG, PK-R, and ATP, and oxygen dissociation sickling in RBCs
- Relationship between mitapivat pharmacokinetics and safety

Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Escalating Multiple Oral Doses of Mitapivat in Subjects With Stable SCD

ClinicalTrials.gov NCT04000165: Nonrandomized, open-label, phase 1 study; N ≈ 15–25



CRADA, Cooperative Research and Development Agreement; NHLBI, National Heart, Lung, and Blood Institute; SCD, sickle cell disease; BID, twice daily.

Clinical Proof-of-concept for Mitapivat Established in Sickle Cell Disease

7 of 8 (88%) efficacy evaluable patients experienced a Hb increase, and 5 of 8 (63%) patients achieved a Hb increase of ≥1.0 g/dL from baseline (range 1.0-2.7 g/dL) at doses of 50 mg BID or lower.

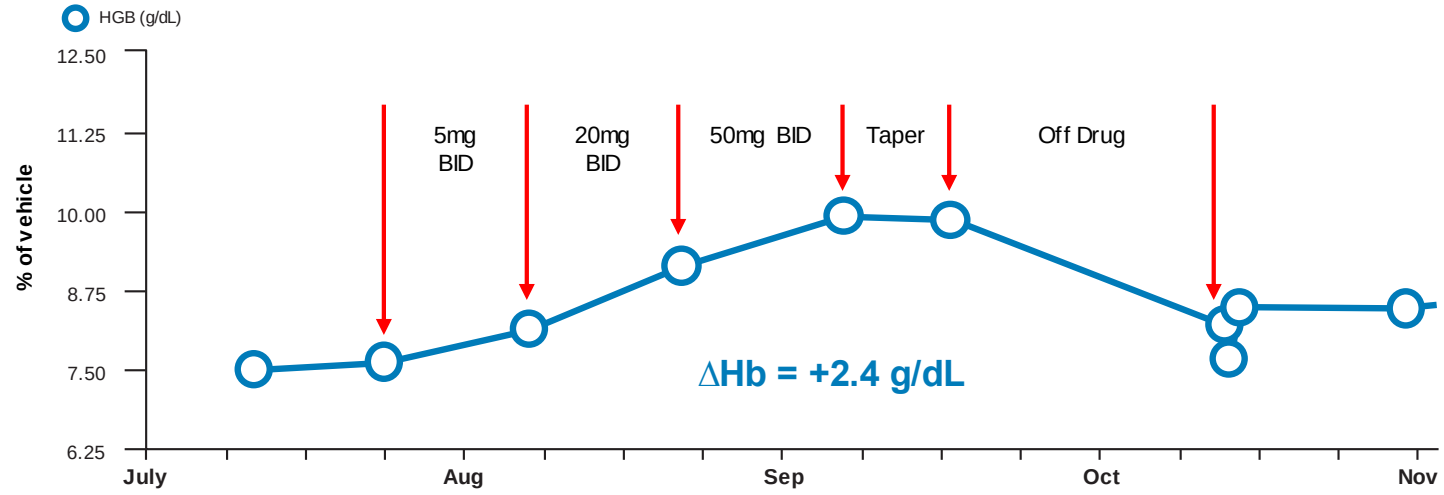
Treatment with mitapivat was associated with decreases in hemolytic markers such as bilirubin, LDH and reticulocytes.

2,3-DPG decreases and increases in ATP levels were observed. Sickling curves (t50) and oxygen dissociation curves (p50) consistent with decreases in both sickling and HbS polymerization.

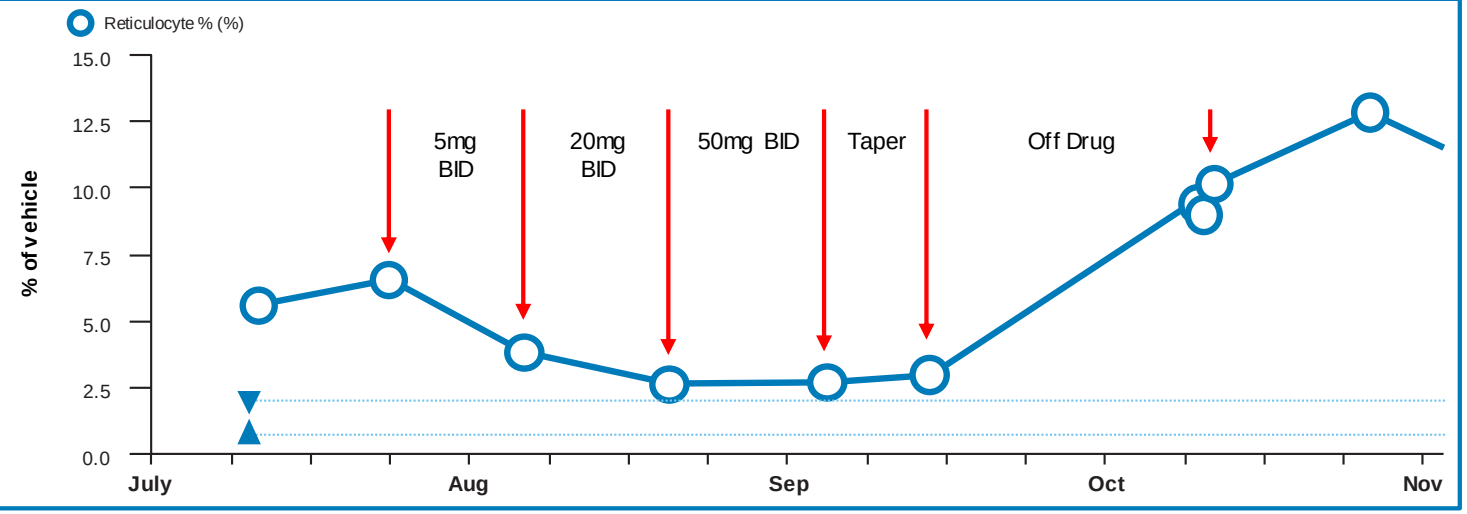
AEs generally consistent with previously reported data with mitapivat treatment or are to be expected in the context of SCD. One SAE, a VOC, occurred during drug taper and was possibly attributed to mitapivat.

Illustrative Sickle Cell Patient Case Study: Male, 39 Years Old

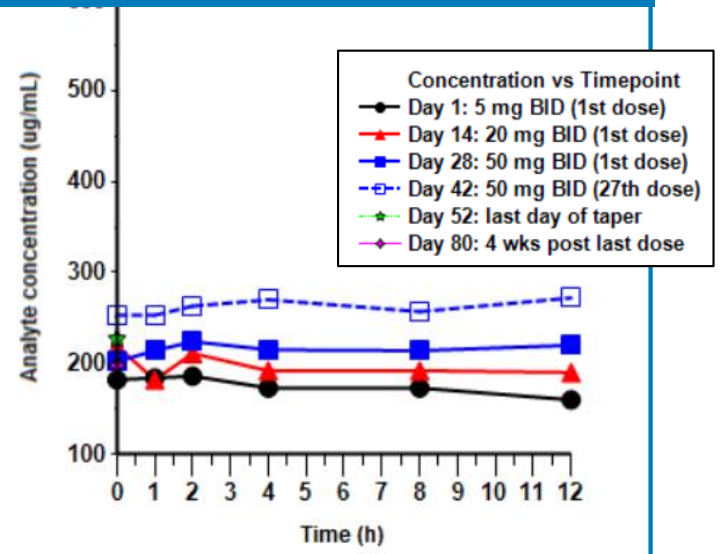
HEMOGLOBIN



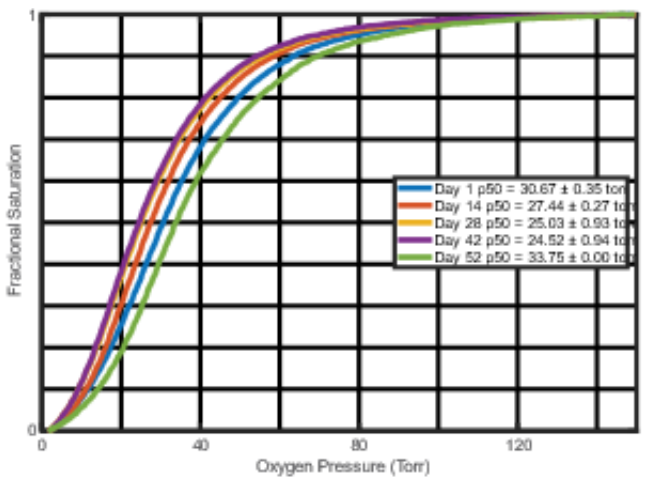
RETICULOCYTE %



ATP



OXYGEN PRESSURE



Closing Remarks

Today's Key Takeaways

Mitapivat data in thalassemia support broad pivotal development

- Treatment with mitapivat induced Hb increase of ≥1.0 g/dL in 92% evaluable patients, including 100% α-thalassemia patients
- 7 of 8 evaluable patients achieved sustained Hb response
- Thalassemia pivotal development plan expected to be finalized by YE 2020 and initiated in 2021

Proof-of-concept established for mitapivat in sickle cell disease

- 63% patients achieved a Hb increase of ≥1.0 g/dL
- Safety profile consistent with previously reported mitapivat data or expected in the context of SCD
- PD and biomarker data support mitapivat's proposed MOA
- Data support pivotal development in SCD; mitapivat pivotal study to initiate in 2021

Monetization of IDHIFA royalty provides $255M non-dilutive capital

- Additional capital supports pivotal development of mitapivat in both thalassemia and SCD


agios
Q&A