Agios 2019 ASH Investor Reception

December 9, 2019

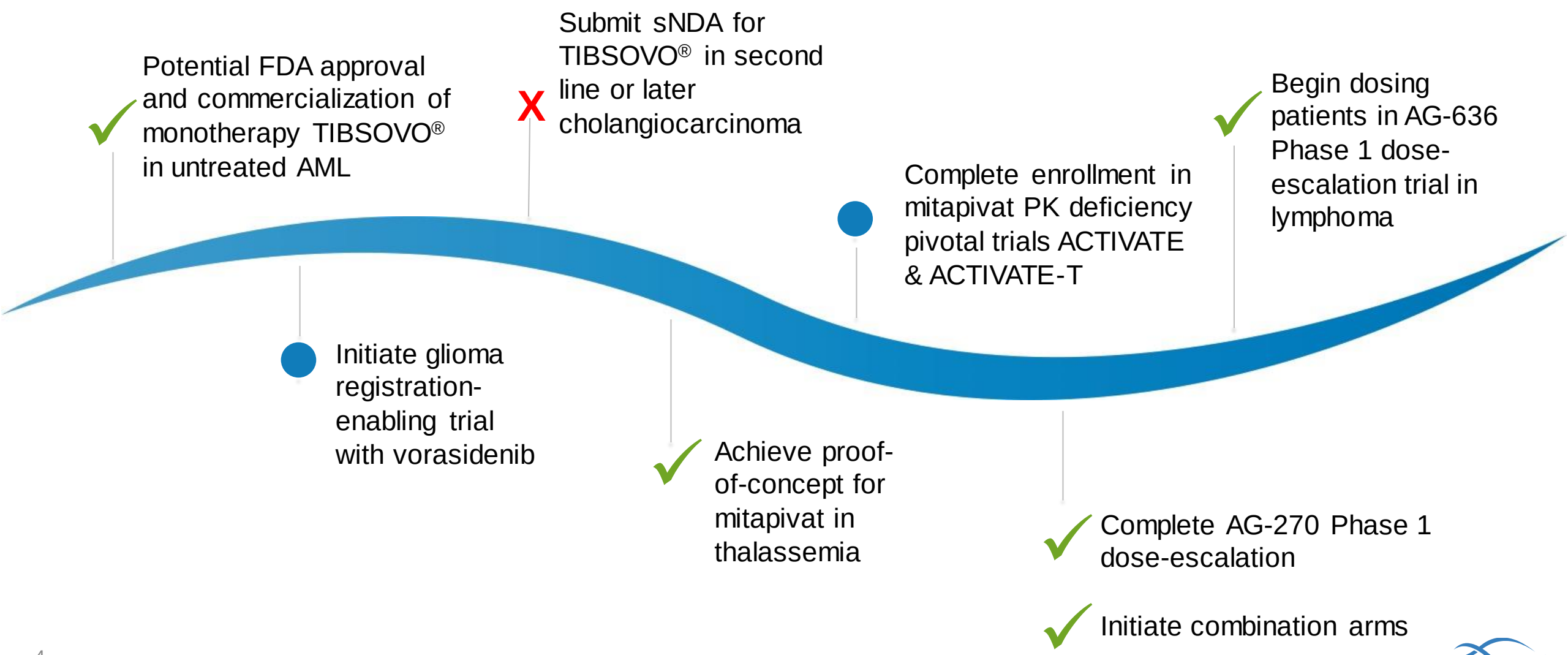
Forward Looking Statements

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' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including TIBSOVO® (ivosidenib) and mitapivat; the potential benefits of Agios' product candidates; its key milestones for 2019; and the potential benefit of its strategic plans and focus. The words "anticipate," "expect," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," 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: 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.

Today's Agenda

- Opening Remarks – **Andrew Hirsch,** Chief Financial Officer & Head of Corporate Developement
- Overview IDH Data – **Chris Bowden, M.D.,** Chief Medical Officer
- Overview of DRIVE PK and PK Deficiency Data – **Eduard van Beers, M.D., Ph.D.**, University Medical Center Utrecht
- Mitapivat Proof-of-Concept in Thalassemia – **Chris Bowden**
- Q&A – **Eduard van Beers, Chris Bowden & Andrew Hirsch**

2019 Key Milestones Position Agios for Long-term Value Creation



Today's Key Takeaways

Agios is leading the science behind IDH mutations in AML

- Multiple pathways can predict for response (JAK2) or resistance (NRAS, PTPN11) to IDH1 inhibitors in AML
- Both IDH-related and non-IDH related pathways potentially contributing to relapse to IDH1 inhibition as a monotherapy further underscoring the complexity of treating AML
- Combination therapy of TIBSOVO® + azacitidine demonstrates high rates of mutation clearance suggesting deep and durable remissions including in patients with baseline mutational profile suggestive of resistance

Mitapivat has the potential to be the first disease-modifying treatment in PK deficiency, a chronic, lifelong hemolytic anemia

- Updated DRIVE PK data demonstrate mitapivat can provide a sustained hemoglobin response and is well tolerated with chronic treatment for more than 3 years
- Significant complications and comorbidities associated with PK deficiency regardless of transfusion status

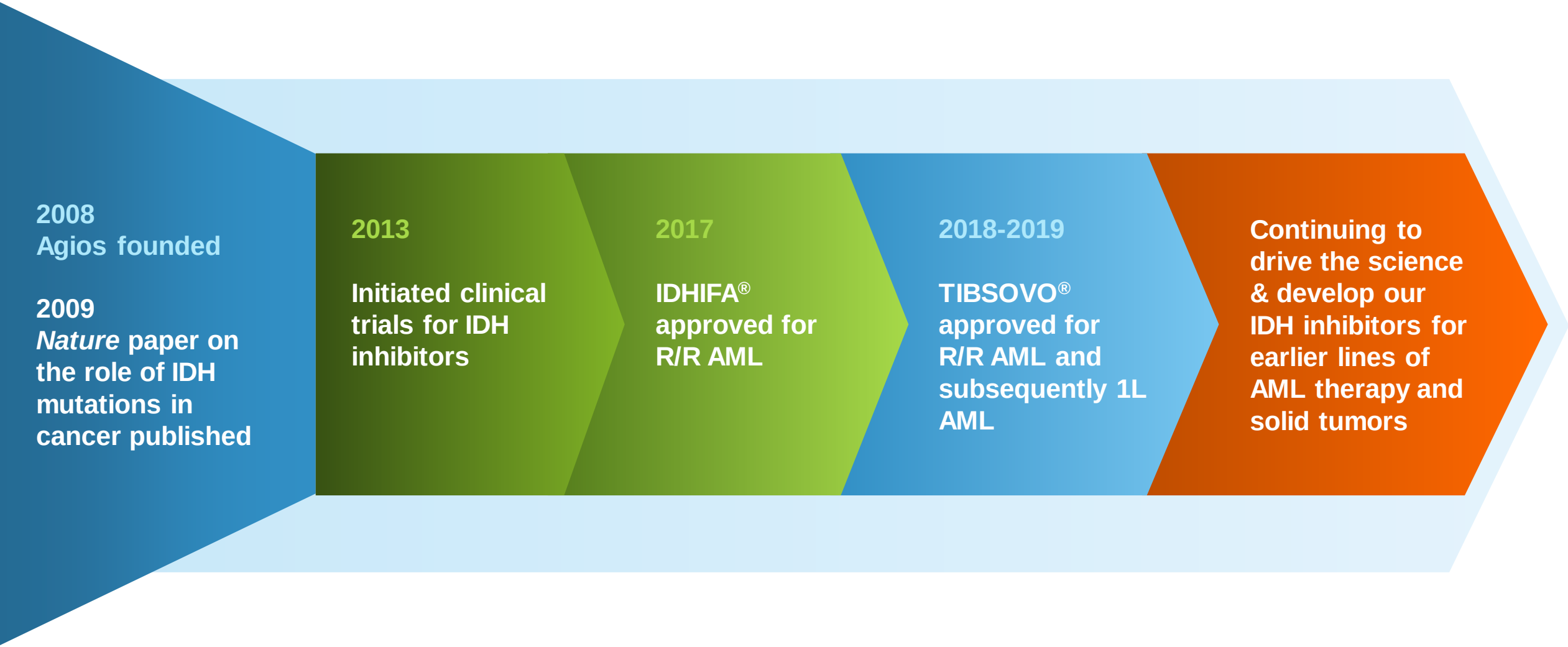
Proof-of-concept achieved for mitapivat in thalassemia

- Treatment with mitapivat induced hemoglobin increase of ≥ 1.0 g/dL in 7 of 8 evaluable patients
- Safety profile consistent with previously published Phase 2 data for mitapivat in patients with PK deficiency

Overview of ASH IDH Presentations

Dr. Chris Bowden, Chief Medical Officer

Leading the Science for IDH Mutations in Cancer



Four IDH-focused Presentations at ASH Highlight Our Scientific and Clinical Leadership in This Space

High rate of IDH1 mutation clearance and measurable residual disease negativity in patients with IDH1-mutant newly diagnosed acute myeloid leukemia treated with ivosidenib (AG-120) and azacitidine	Presented: Sunday, Dec. 8, 8 AM by Scott Daigle
Complex Polyclonal Resistance Mechanisms to Ivosidenib Monotherapy in IDH1-Mutant Relapsed or Refractory Acute Myeloid Leukemia Revealed by Single Cell Sequencing Analyses	Presented: Monday, Dec. 9, 7 AM by Dr. Lynn Quek
Molecular mechanisms mediating relapse following ivosidenib monotherapy in patients with IHD1-mutant relapsed or refractory acute myeloid leukemia	Presented: Monday, Dec. 9, 8 AM by Dr. Rich Stone
Enasidenib plus azacitidine significantly improves complete remission and overall response compared with azacitidine alone in patients with newly diagnosed acute myeloid leukemia (AML) with isocitrate dehydrogenase 2 (IDH2) mutations: interim phase II results from an ongoing, randomized study	Presented: Monday, Dec. 9, 10:30 AM by Dr. Courtney DiNardo

TIBSOVO® Results in Durable Remissions as a Monotherapy in R/R AML

	R/R AML 500 mg (n=179)
CR+CRh rate, n (%) [95% CI]	57 (31.8) [25.1, 39.2]
Time to CR/CRh, median (range) months	2.0 (0.9–5.6)
Duration of CR/CRh, median [95% CI] months	8.2 [5.6, 12.0]
CR rate, n (%) [95% CI]	43 (24.0) [18.0, 31.0]
Time to CR, median (range) months	2.8 (0.9–8.3)
Duration of CR, median [95% CI] months	10.1 [6.5, 22.2]
CRh rate, n (%)	14 (7.8)
Duration of CRh, median [95% CI] months	3.6 [1.0, 5.5]
Overall response rate, n (%) [95% CI]	75 (41.9) [34.6, 49.5]
Time to first response, median (range) months	1.9 (0.8–4.7)
Duration of response, median [95% CI] months	6.5 [5.5, 10.1]
Best response, n (%)	
CR	43 (24.0)
CRi or CRp	21 (11.7)
MLFS	11 (6.1)
SD	68 (38.0)
PD	15 (8.4)
NA	21 (11.7)

CRh includes 9 patients with investigator-assessed responses of CRi/CRp and 5 with MLFS.
Overall response rate includes CR, CRi/CRp, MLFS, and PR.
NA = not assessed; PD = progressive disease.
Source: U.S. Prescribing Information

Objective of AML Translational Research

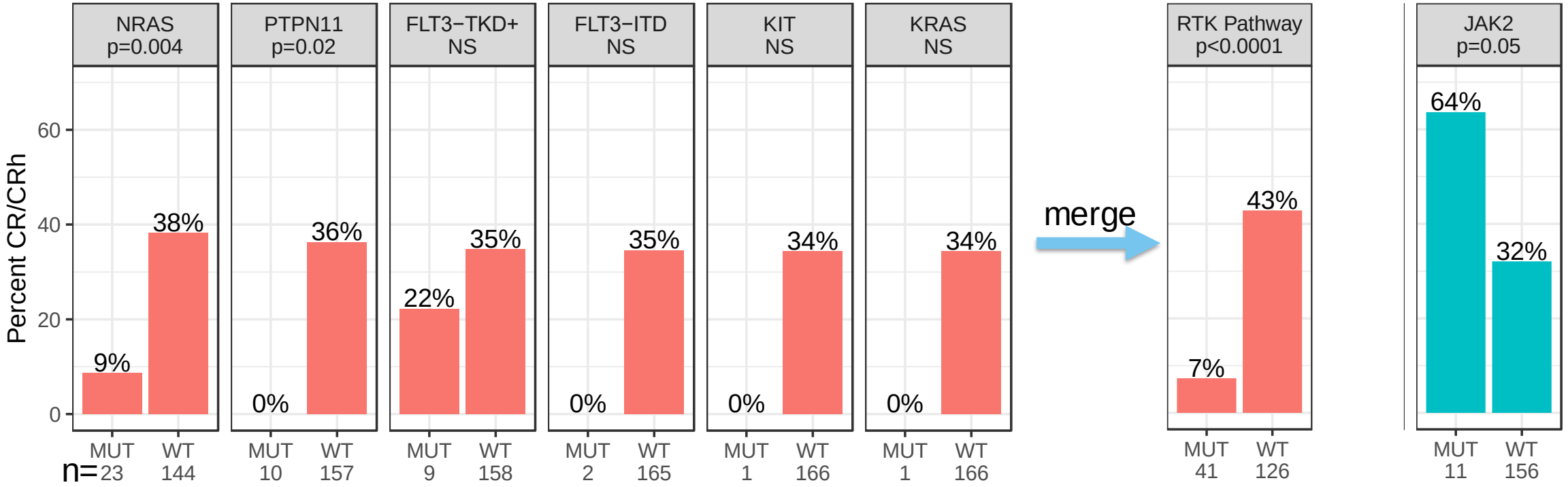
Characterize the molecular predictors of response and mechanisms of relapse to ivosidenib monotherapy in mutant *IDH1* relapsed/refractory AML using comprehensive genomic profiling

- Hypothesis 1: Pre-therapy genetic profile predicts response
  - Mutations in single genes and pathways
  - Clonal vs subclonal status of mIDH1

- Hypothesis 2: Relapse is due to both IDH-dependent and independent mechanisms
  - Assess for IDH2 and/or novel IDH1 mutations at relapse[1,2]
  - Compare mutational profile pre-therapy and at relapse

1. Harding JJ et al. *Cancer Discov* 2018;8:1540-47. **2.** Intlekofer AM et al. *Nature* 2018;559:125-29.; Data presented at ASH 2019

Translational Work Highlights Multiple Pathways at Baseline that Could Predict Response or Resistance to IDH1 Inhibitors



- RTK pathway mutations, and mutations in the individual genes *NRAS* and *PTPN11*, are significantly associated with a lack of CR or CRh response
- 64% of patients with JAK2 mutations achieved CR or CRh

P-values: Fisher's exact test. NS, Not Significant. FLT3-TKD+: FLT3 TKD or JXD
Data Source: 167 patients with baseline NGS data from whole bone marrow; Data presented at ASH 2019

Both IDH-related and Non-IDH-related Pathways Identified at Relapse Further Underscoring Heterogeneity of R/R AML

Pathway	Patients with mutations emerging at relapse or progression by pathway, n (%)	
	All n = 74	Best response of CR or CRh n = 26
***IDH*-related**	17 (23%)	9 (35%)
IDH1 second-site	10 (14)	5 (19)
IDH2-R140Q	9 (12)	6 (23)
Non-IDH-related		
RTK pathway	20 (27%)	9 (35%)
Differentiation	13 (18)	8 (31)
Chromatin	9 (12)	8 (31)
Epigenetics	6 (8)	2 (8)

Data Source: 74 patients with NGS data at baseline and at relapse or disease progression (BMMC/PBMC, all evaluable response groups); Data presented at ASH 2019

Four IDH-focused Presentations at ASH Highlight Our Scientific and Clinical Leadership in This Space

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TIBSOVO® and Azacitidine Combination Results in Deep and Durable Remissions in Newly Diagnosed AML

Response parameter	All patients N=23
CR, n (%) [95% CI]	14 (60.9) [38.5, 80.3]
Time to CR, median (range), months	3.7 (0.8–15.7)
Duration of CR, median [95% CI], months	NE [9.3, NE]
CR+CRh,[a] n (%) [95% CI]	16 (69.6) [47.1, 86.8]
Time to CR+CRh, median (range), months	2.8 (0.8–11.5)
Duration of CR+CRh, median [95% CI], months	NE [12.2, NE]
CRh, n (%)	2 (8.7)
ORR, n (%) [95% CI]	18 (78.3) [56.3, 92.5]
Time to response, median (range), months	1.8 (0.7–3.8)
Duration of response, median [95% CI], months	NE [10.3, NE]
Best response[b]	
CR, n (%) [95% CI]	14 (60.9) [38.5, 80.3]
CRi/CRp, n (%)	2 (8.7)
MLFS, n (%)	2 (8.7)
SD, n (%)	4 (17.4)
NA, n (%)	1 (4.3)
OS, 12-month rate, % [95% CI][c]	82.0 [58.8, 92.8]
Duration of follow-up, median (range), months	16.1 (1.3–31.7)

Multiple orthogonal measures of MRD in bone marrow samples demonstrate high molecular remissions

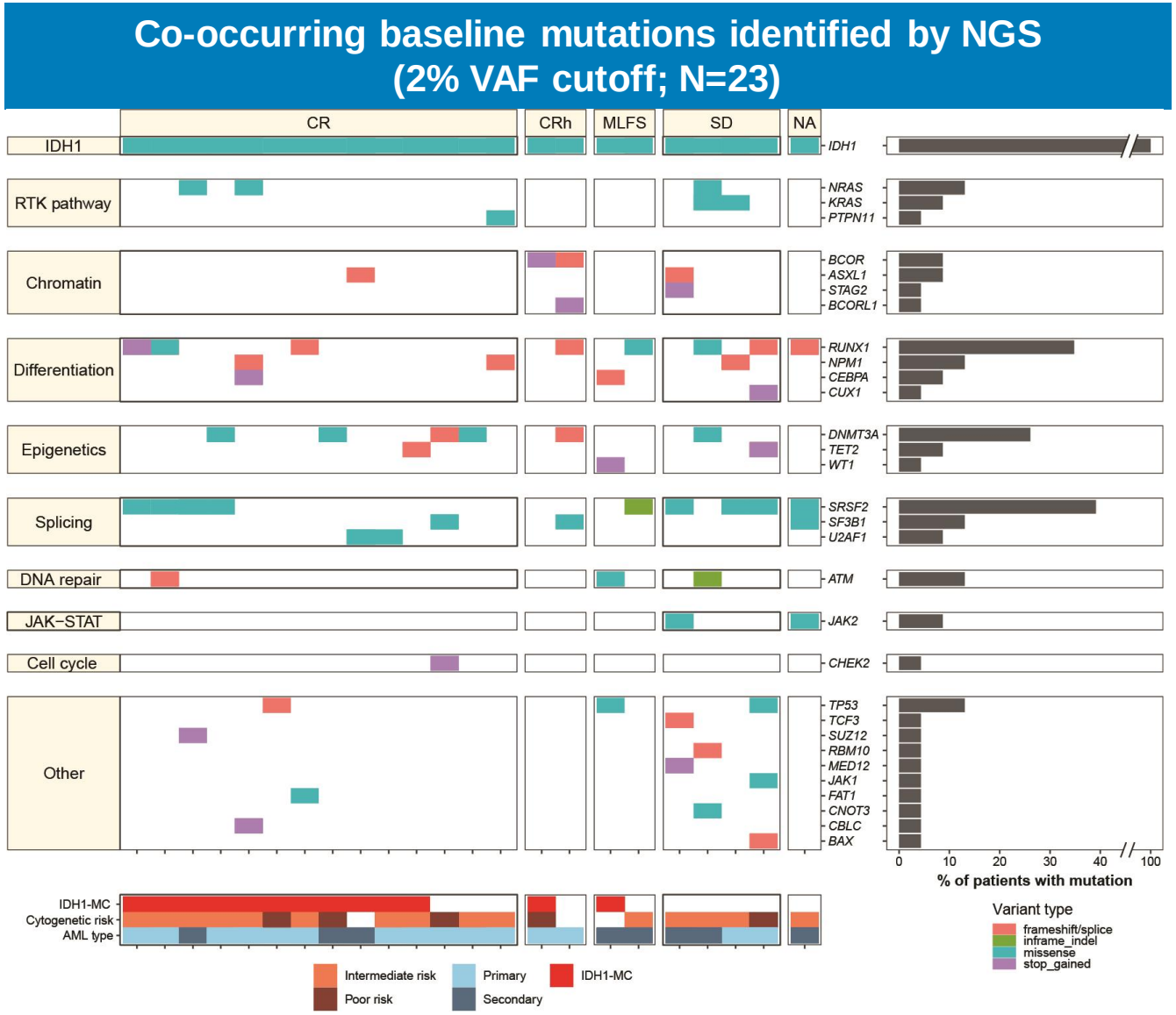
	Mutation clearance		
	m*IDH1*, BEAMing digital PCR (n=21)	≥2 non-DTA[a] genes, NGS (n=17)	Flow MRD (n=14)
Assay cutoff	0.02–0.04%	2%	1.25%[b]
CR+CRh	11/16 (69)	9/13 (69)	10/12 (83)
CR	10/14 (71)	8/11 (73)	8/10 (80)
CRh	1/2 (50)	1/2 (50)	2/2 (100)
Non-CR+CRh responders	1/2 (50)	0/2 (0)	0/1 (0)
Nonresponders	0/3 (0)	0/2 (0)	0/1 (0)

Combination of enasidenib + azacitidine demonstrated similarly high remission rates with ORR: 72%, CR rate of 53%

[a]Mutation clearance of all baseline co-mutations identified by NGS, excluding genes involved in clonal hematopoiesis (*DNMT3A/TET2/ASXL1* – "DTA" genes) [b]The average sensitivity is 1.25%, with a range from 0.13% to 1.84% owing to variability in the surface markers and LAIPs detected

DTA = *DNMT3A/TET2/ASXL1; Data presented at ASH 2019; TIBSOVO® is not approved in any country for the treatment of patients with newly diagnosed AML in combination with azacitidine.*

TIBSOVO® and Azacitidine Combination Induced Response in Newly Diagnosed Patients with RTK Pathway Mutations



- No statistically significant relationship between baseline genetic variants and clinical response or primary resistance was observed.
- CR/CRh was achieved in four of five patients with poor-risk karyotypes.
- CR/CRh was achieved in three of five patients with receptor tyrosine kinase (RTK) pathway mutations (KRAS, NRAS, PTPN11).

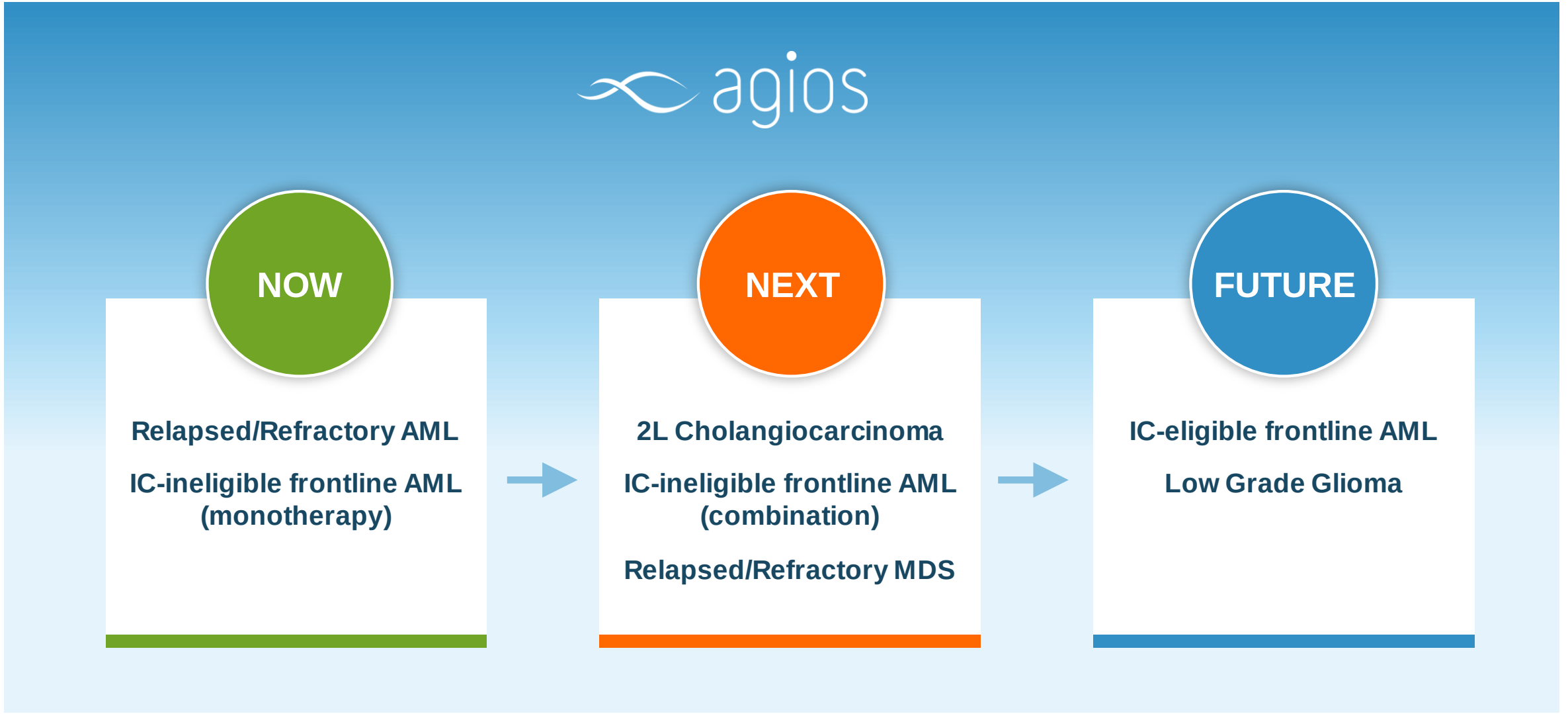
Known or likely oncogenic mutations detected in BMMCs or PBMCs are shaded by variant type. In this heatmap, each column corresponds to a single patient, arranged by best overall response.
IDH1-MC = *IDH1* mutation clearance; Data presented at ASH 2019; *TIBSOVO® is not approved in any country for the treatment of patients with newly diagnosed AML in combination with azacitidine.*

Conclusions

- TIBSOVO® as a monotherapy or in combination results in durable remissions in relapsed/refractory and newly diagnosed AML
- AML is a heterogeneous disease, and in relapsed/refractory disease, multiple pathways at baseline can predict for response (JAK2) or resistance (NRAS, PTPN11) to IDH1 inhibitors
- Translational work has further elucidated mechanisms potentially contributing to relapse to IDH1 inhibition as a monotherapy which include both IDH-related and non-IDH related pathways further underscoring the complexity of treating AML and the need for mutational testing at relapse
- Combination therapy of TIBSOVO® + azacitidine demonstrates high rates of mutation clearance suggesting both deep and durable remissions including in newly diagnosed patients with baseline mutational profile suggestive of resistance (e.g. Ras pathway mutations)
  - Ongoing Phase 3 AGILE trial aims to further validate the combination hypothesis

Data presented at ASH 2019; *TIBSOVO® is not approved in any country for the treatment of patients with newly diagnosed AML in combination with azacitidine.*

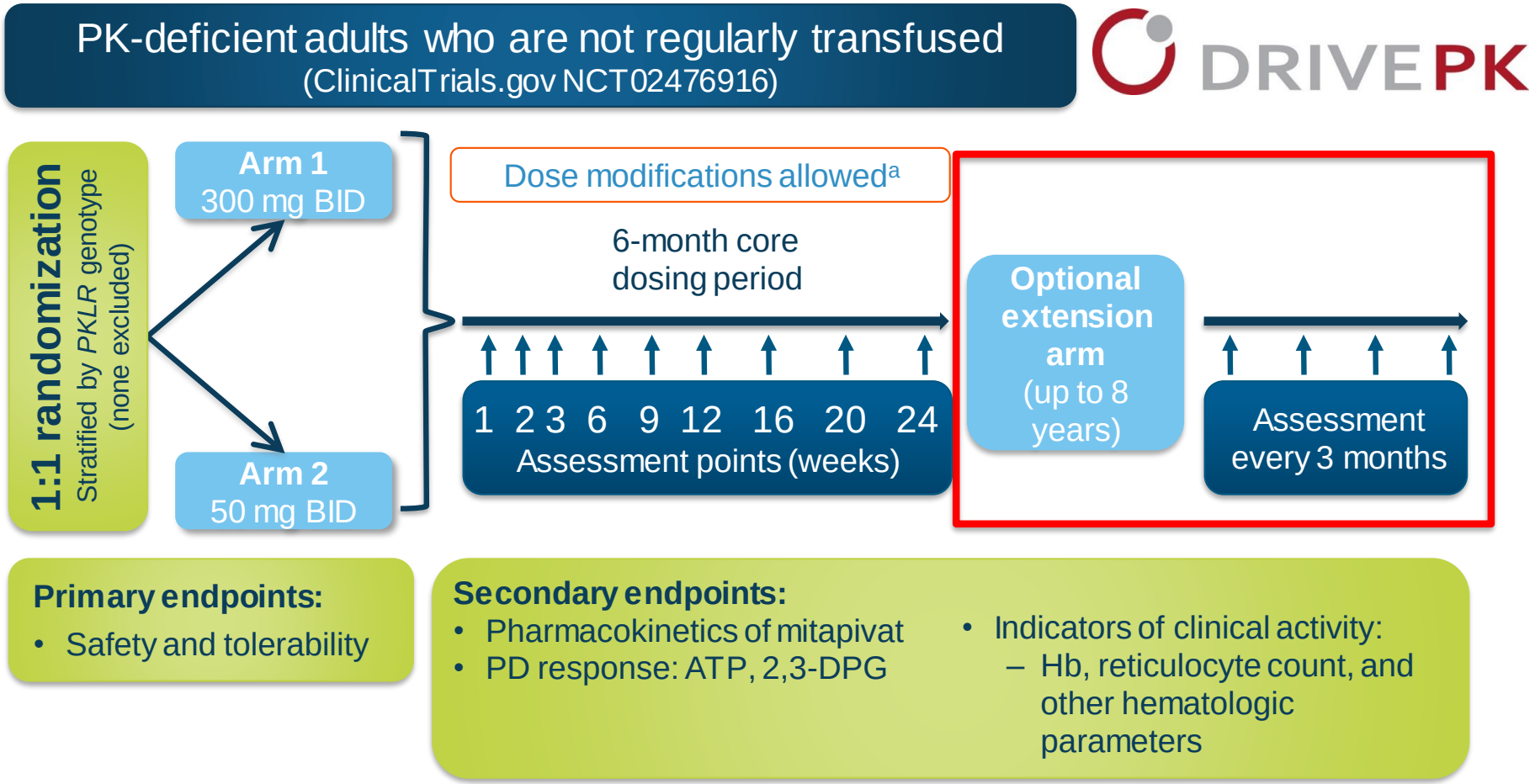
What's Possible for IDHm Patients



Overview of ASH PKR Presentations

Dr. Eduard van Beers, University Medical Center Utrecht

Long-term Safety & Efficacy Data from DRIVE PK Study of Mitapivat in Adults in PK Deficiency

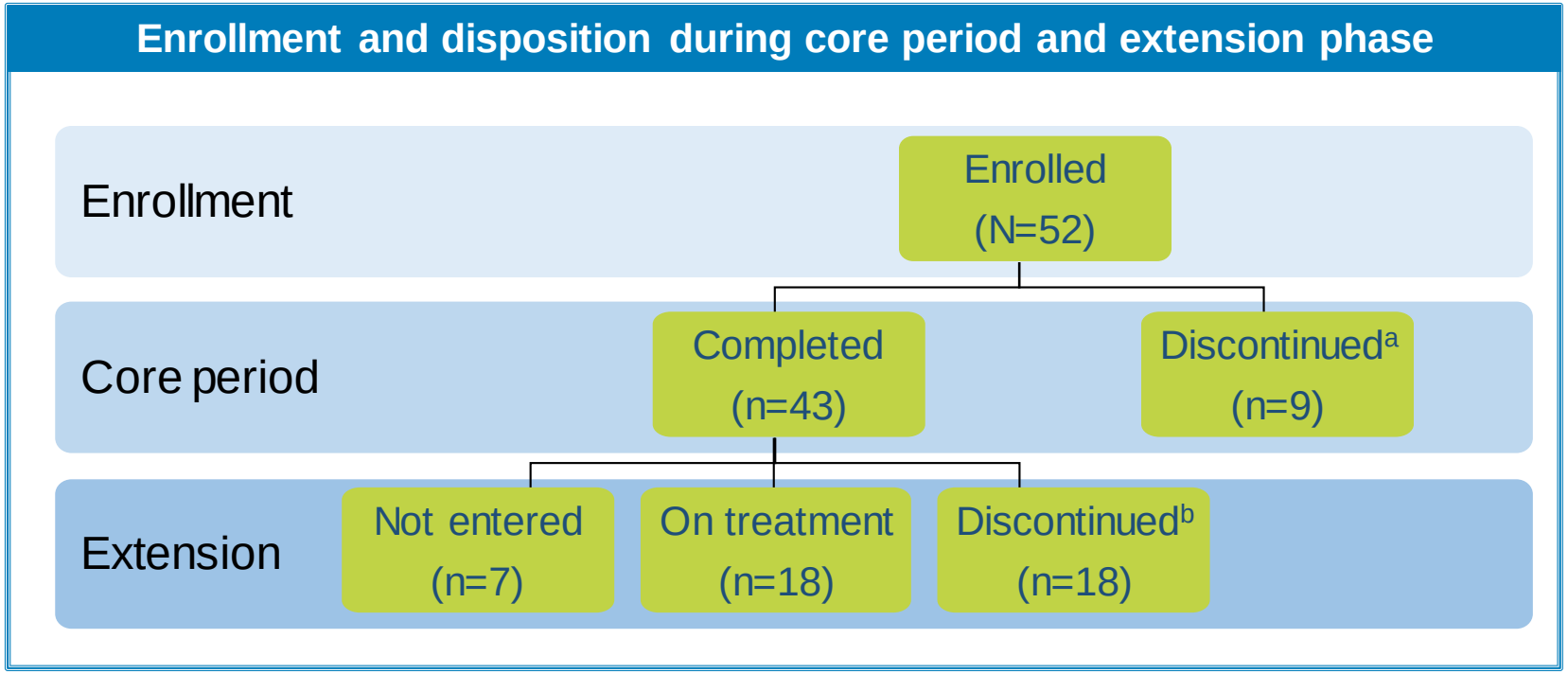


Not regularly transfused = no more than 3 units of red blood cells transfused in the 12 months prior to the first day of study dosing and no transfusions within 4 months of the first day of study dosing; 2,3-DPG = 2,3-diphosphoglycerate; BID = twice daily; PD = pharmacodynamic; Data presented at ASH 2019

[a]Dose adjustments were allowed in the core period on the basis of safety, side-effect profile, and Hb response

18 Patients Remain in Extension Phase of Study

- Of the 52 patients enrolled in the study, 43 completed the core period (24 weeks) and 18 remained in the study as of March 27, 2019



[a]Adverse events (AEs), n=4 (pharyngitis and nausea, hemolytic anemia, hypertriglyceridemia, and pleural effusion); investigator decision, n=2; patient withdrew consent, n=3
[b]Investigator decision, n=7; other reasons, n=7; patient withdrew consent, n=1; AEs, n=2 (elevated alanine aminotransferase and nonalcoholic steatohepatitis, and renal cell carcinoma); non-compliance, n=1; Data presented at ASH 2019

Demographics Similar for Those in Extension Compared to the Total Cohort

- No differences in age, race, or sex were observed between patients who continued in the extension period and those in the total cohort
- All continuing patients had at least one missense PKLR mutation

Characteristic	Total N=52	Continued n=18
Male, n (%)	32 (61.5)	10 (55.6)
Age at randomization, median (range), years	34 (18–61)	33.5 (19–61)
White, n (%)	43 (82.7)	17 (94.4)
Hb baseline, median (range), g/dL	8.9 (6.5–12.3)	9.7 (7.9–12.0)
Splenectomy, n (%)	43 (82.7)	11 (61.1)
Cholecystectomy, n (%)	38 (73.1)	14 (77.8)
Iron chelation prior to enrollment, n (%)	25 (48.1)	5 (27.8)
Osteoporosis, n (%)	8 (15.4)	2 (11.1)
Mutation category, n (%) Missense/missense Missense/non-missense Non-missense/non-missense	 32 (61.5) 10 (19.2) 10 (19.2)	 14 (77.8) 4 (22.2) 0 (0)

Data presented at ASH 2019

Mitapivat Was Generally Well Tolerated; No New Safety Signals Were Identified in Extension

Most common AEs in patients who continued in the extension period[a]

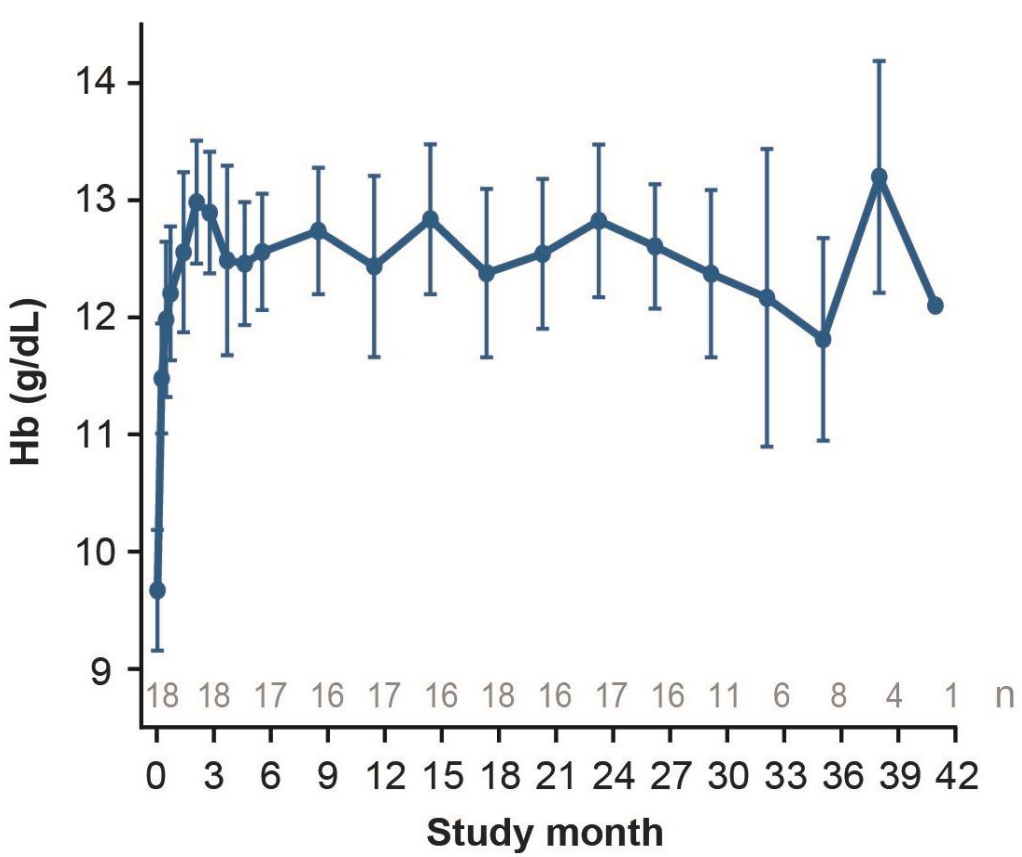
AE, n (%)	Continued n=18	
	Core (n=18)	Extension (n=18)
Headache	10 (55.6)	7 (38.9)
Insomnia	5 (27.8)	5 (27.8)
Fatigue	4 (22.2)	5 (27.8)
Nasopharyngitis	1 (5.6)	5 (27.8)
Dizziness	3 (16.7)	3 (16.7)
Gastroenteritis	2 (11.1)	3 (16.7)
Pyrexia	2 (11.1)	3 (16.7)
Hypertriglyceridemia	1 (5.6)	3 (16.7)
Nausea	6 (33.3)	2 (11.1)
Influenza	4 (22.2)	2 (11.1)
Arthralgia	4 (22.2)	2 (11.1)
Cough	3 (16.7)	2 (11.1)
Vomiting	4 (22.2)	1 (5.6)
Diarrhea	4 (22.2)	1 (5.6)
Dysmenorrhoea	4 (22.2)	1 (5.6)
Hot flush	5 (27.8)	0 (0.0)
Chest discomfort	3 (16.7)	0 (0.0)

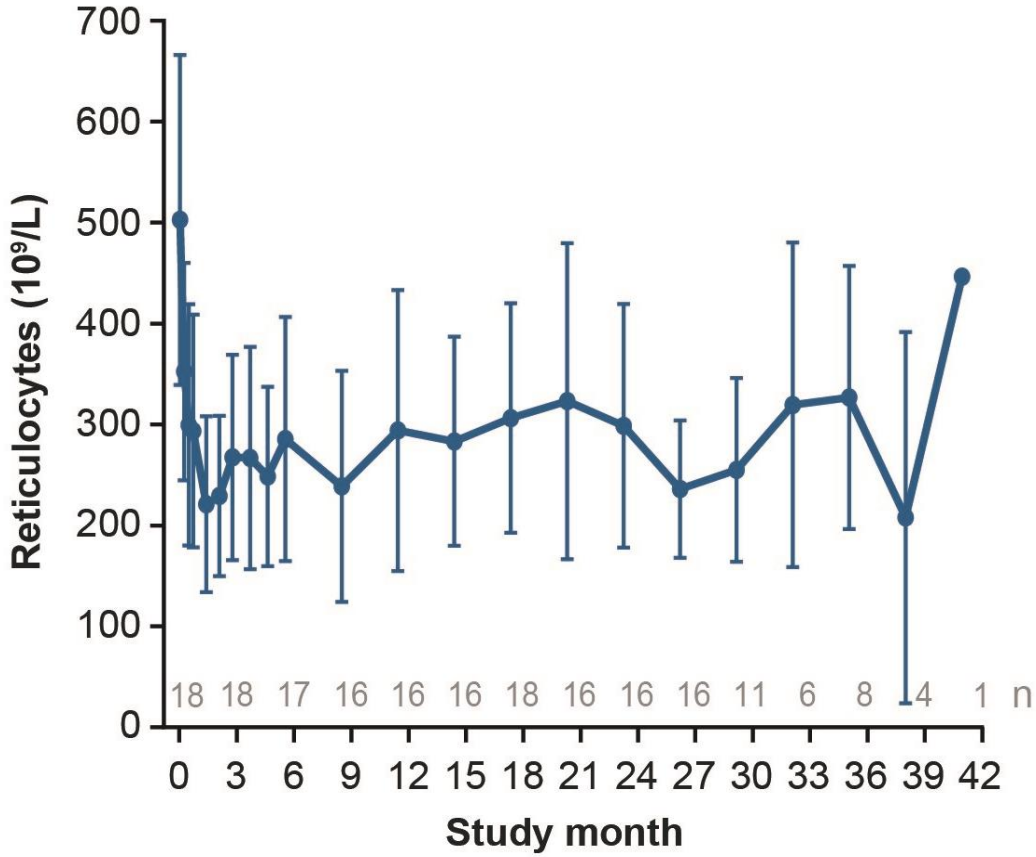
AEs coded using MedDRA, version 21.0; Data presented at ASH 2019

[a]AEs occurring in >15% of the 18 continuing patients in either the core or extension periods

Hemoglobin Response Maintained for More Than 3 Years

- Improvements in hemoglobin and other markers of hemolysis including reticulocytes, indirect bilirubin, and haptoglobin achieved during the core period were sustained during the extension period (up to 42 months)





Data presented at ASH 2019

Conclusions

- Mitapivat is a novel, first-in-class, PK-R activator with potential to be the first disease-altering therapy for patients with PK deficiency
- Patients who responded to mitapivat had long-term clinically meaningful responses:
  - Improvements in hemoglobin and other hemolysis markers were sustained at optimized individual doses during the extension period
- Chronic daily dosing with mitapivat for a median of 3 years and up to 42 months was well tolerated:
  - Consistent safety profile over the duration of treatment with no new safety signals observed
- Two phase 3 trials are ongoing to further study the effect of mitapivat in patients with PK deficiency

ACTIVATE N=76

A PHASE 3 CLINICAL TRIAL INVESTIGATING
A NOVEL ENZYME ACTIVATOR'S TREATMENT
EFFECT IN PATIENTS WITH PK DEFICIENCY

ACTIVATE-T N=20–40

A PHASE 3 CLINICAL TRIAL INVESTIGATING
A NOVEL ENZYME ACTIVATOR'S TREATMENT
EFFECT IN PK DEFICIENCY PATIENTS WITH
HIGH TRANSFUSION BURDEN

Prevalence of Comorbidities and Complications in Adults with Pyruvate Kinase Deficiency

Dr. Eduard van Beers, University Medical Center Utrecht

Patients with PK Deficiency Have Higher Rates of Comorbidities & Complications Regardless of Transfusion Status

Results from the PK deficiency National History Study show that patients with PK deficiency, regardless of transfusion status, have higher rates of select comorbidities and complications than age-and gender-matched individuals from the general population

For many conditions, a gradient is seen across PK deficiency transfusion cohorts, with the highest rates observed for patients who are regularly transfused

Even patients with PK deficiency who have never been transfused are at increased risk of complications of the disease and its treatment

Study Population and Inclusion Criteria

Patients with PK deficiency: Sufficient data on transfusion history to enable classification into one of three mutually exclusive cohorts:

- Ever Regularly Transfused (ERT): Received transfusions, ≥6 in any year
- Never Regularly Transfused (NRT): Received transfusions, ≤4 per year
- Never Transfused (NT): Never received a transfusion

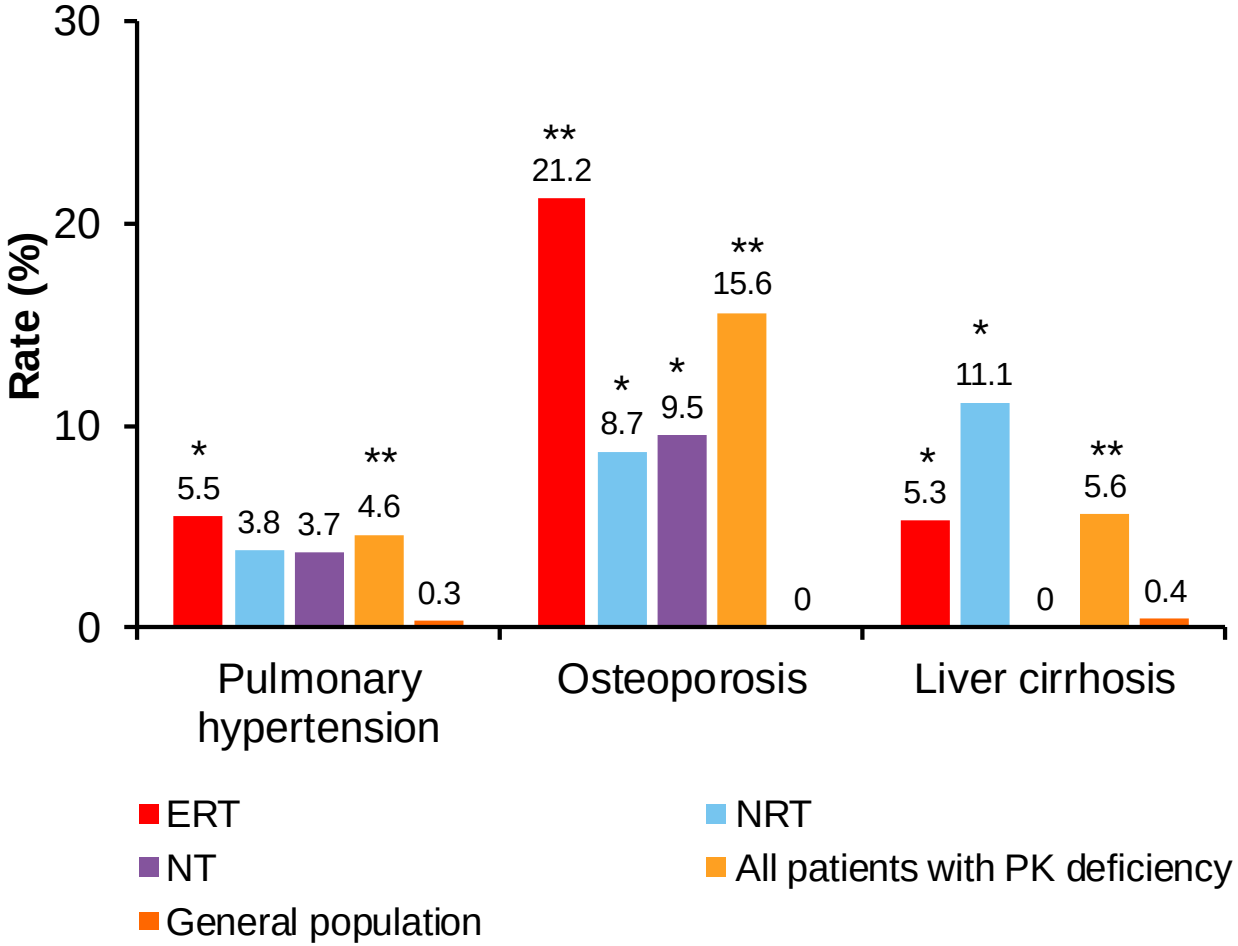
General population: No hemolytic anemia diagnoses; ≥5 years of continuous enrollment in the MarketScan® databases.

Parameter	ERT (n=65)	NR (n=30)	NT (n=27)	p-value ERT vs NRT	p-value ERT vs NT
Male, n (%)	30 (46.2)	17 (56.7)	16 (59.3)	0.383	0.360
Age, mean (SD) years	34.2 (11.0)	39.5 (14.7)	37.2 (16.3)	0.083	0.383
White, n (%)	63 (96.9)	30 (100)	27 (100)	>0.999	>0.999
Hispanic or Latino, n (%)	2 (3.1)	1 (3.3)	1 (3.7)	>0.999	>0.999
Genotype, n (%)				0.085	0.003
Amish (R479H/R479H)	20 (30.8)	4 (13.3)	3 (11.1)		
Missense/missense	21 (32.3)	14 (46.7)	19 (70.4)		
Non-missense/missense	11 (16.9)	8 (26.7)	5 (18.5)		
Non-Missense/Non-missense	12 (18.5)	2 (6.7)	0 (0)		
Unknown	1 (1.5)	2 (6.7)	0 (0)		

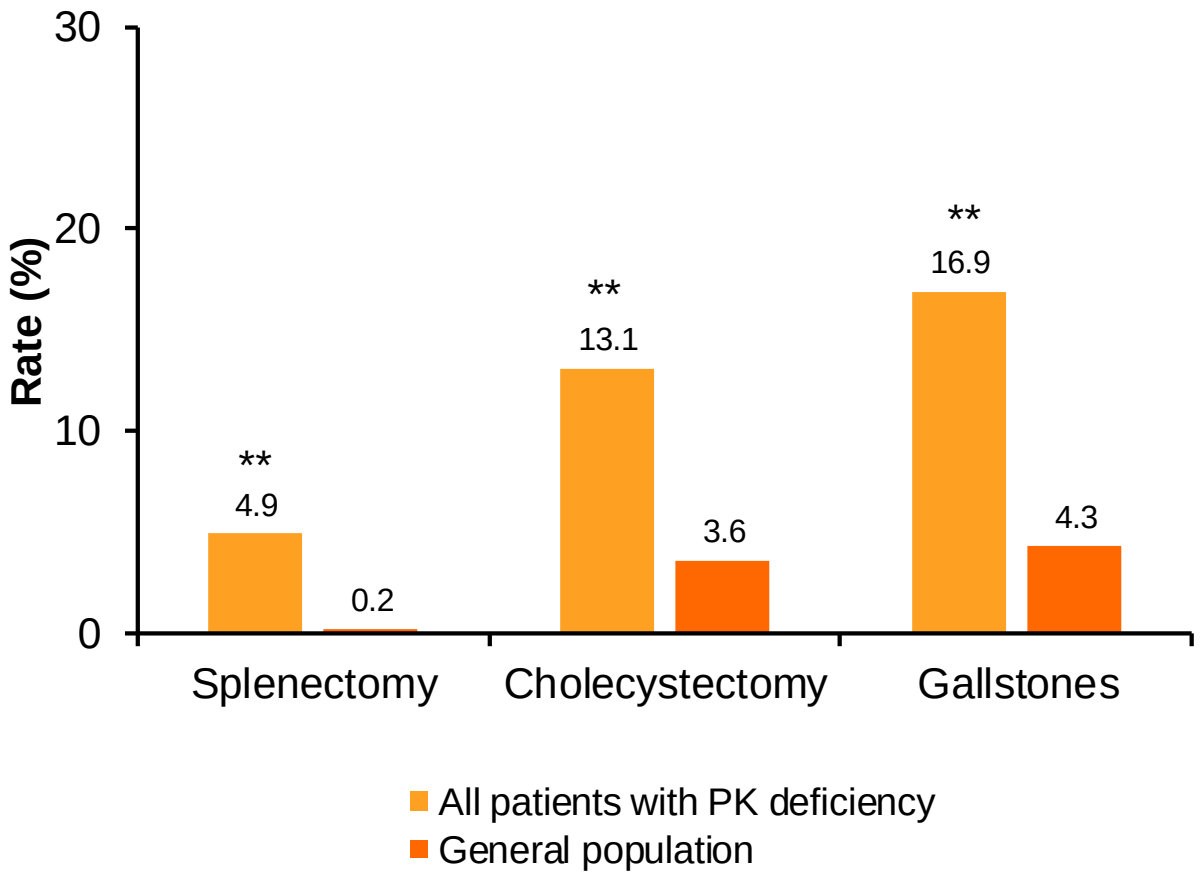
The general population (N=1220) was matched by age and gender to the PK deficiency population. For sex, race, and ethnicity, the p-value is based on a two-sided Fisher's exact test; for age, the p-value is based on a two-sample t-test; for genotype, the p-value is based on a Chi-square test; Data presented at ASH 2019

Adults with PK Deficiency Had Higher Rates of Select Comorbidities & Complications

Adults with PK deficiency had higher lifetime rates of pulmonary hypertension, osteoporosis, and liver cirrhosis



Adults with PK deficiency had higher rates of splenectomy, cholecystectomy, and gallstones over the preceding 8 years



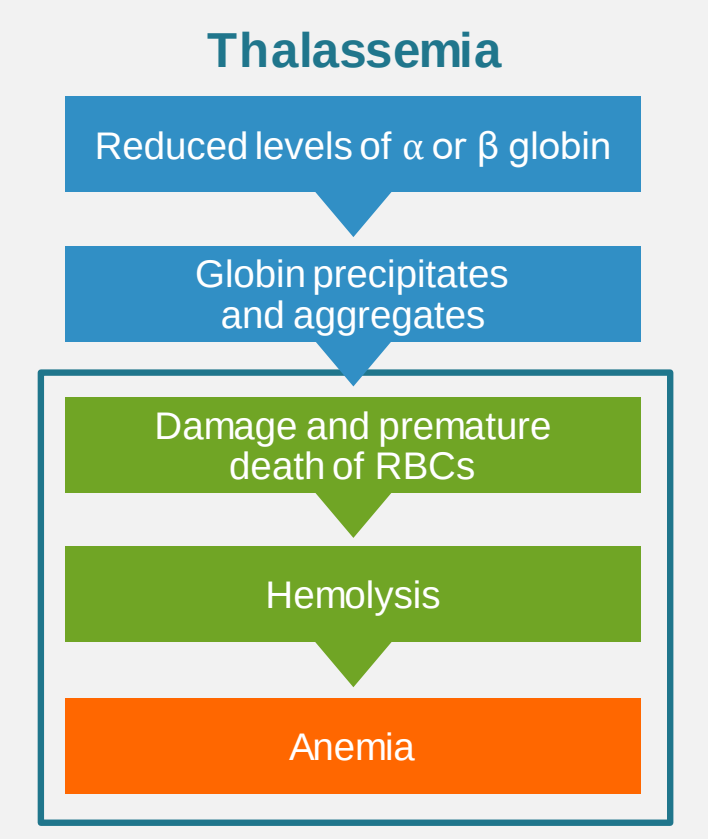
All comparisons are based on a two-sided Fisher's exact test; *p<0.05 for PK Deficiency NHS population versus matched general population; **p<0.001 for PK Deficiency NHS population versus matched general population; Data presented at ASH 2019

Mitapivat Development in Thalassemia

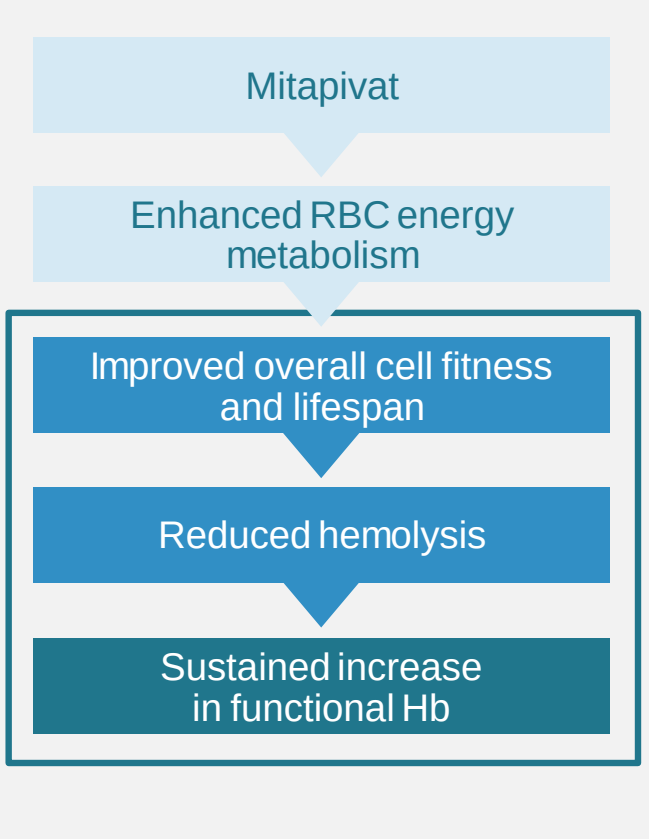
Dr. Chris Bowden, Chief Medical Officer

Mitapivat Activates Both Wild-type and Mutant PKR

Hypothesis: Activation of wild-type PKR may improve RBC cell fitness and survival by increasing ATP levels in hemolytic anemias such as thalassemia



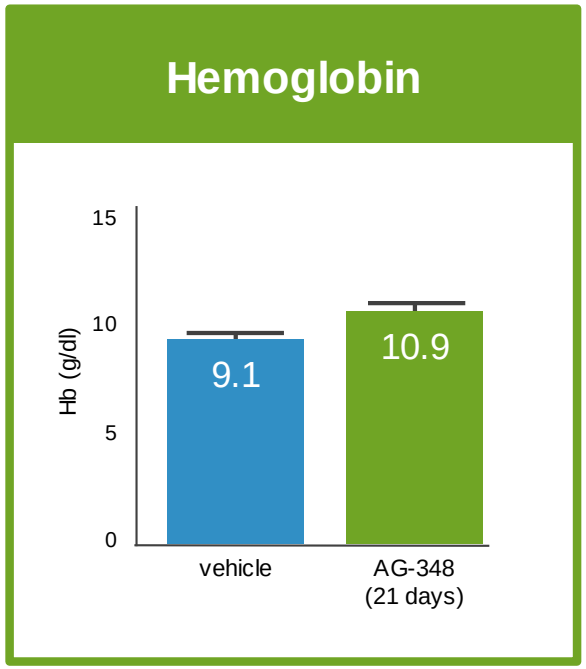
Thalassemic RBCs have impaired ATP production and increased ATP demand to maintain cell fitness[1-4]

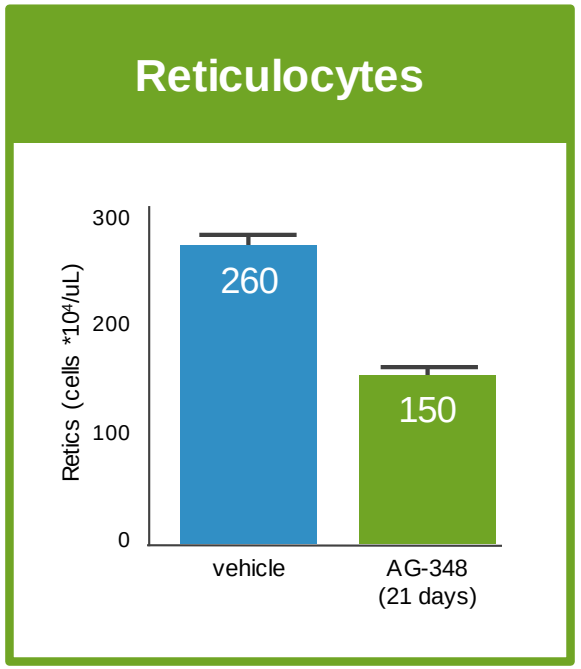


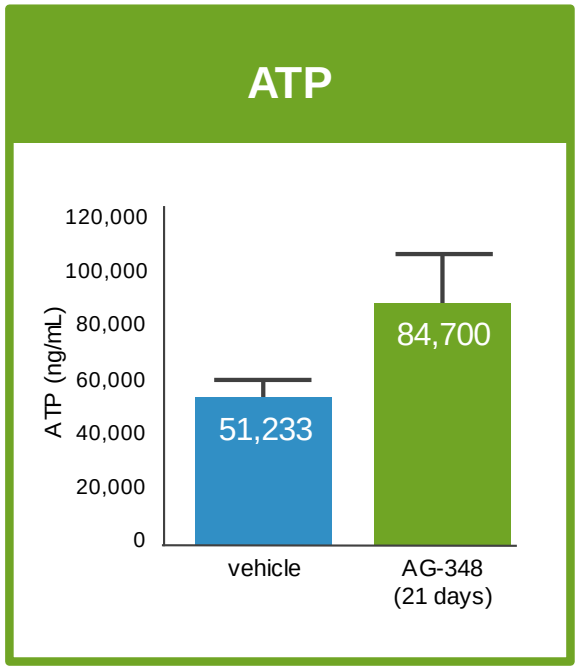
ATP, adenosine triphosphate; RBC, red blood cell.

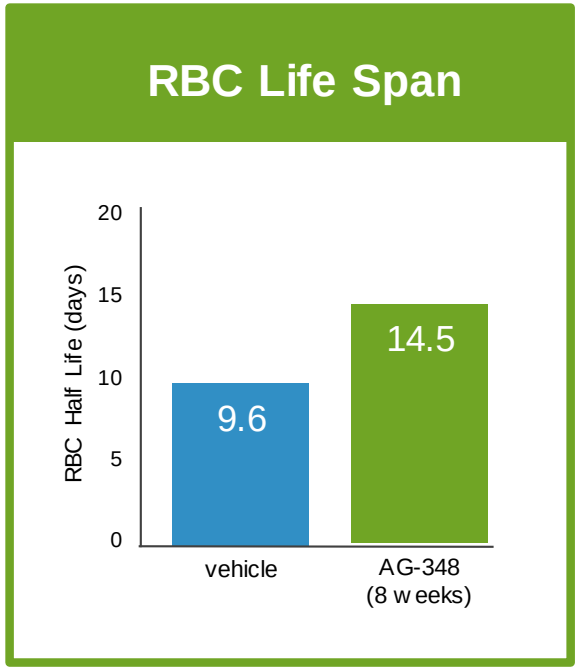
1. Ting YL et al. *Br J Haematol*. 1994;88:547-54. **2.** Kung C et al. *Blood* 2017;130:1347-56. **3.** Chakraborty I et al. *Arch Med Res*. 2012;43:112-6. **4.** Gunn RB et al. *J Clin Invest*. 1972;51:1043-50. **5.** Scott GL, et al. *Br J Haematol*. 1970;18:13-28.

Mitapivat Improves Red Cell Parameters in a β-thalassemia Mouse Model







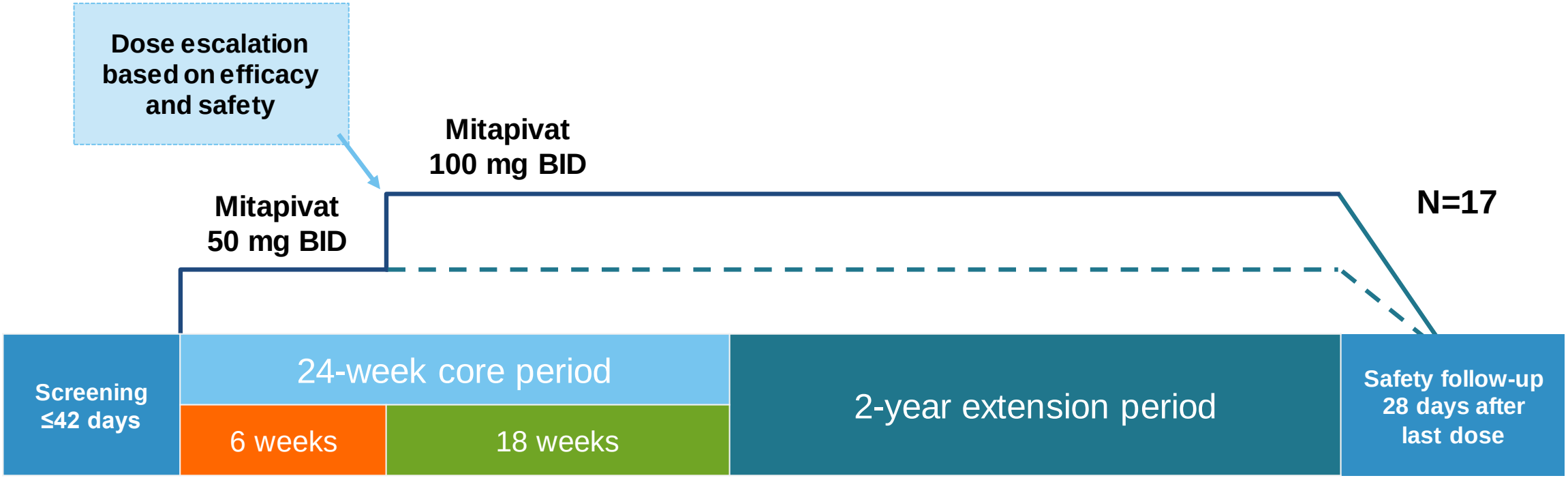


Vehicle
Mitapivat (AG-348)
(Hb, reticulocytes & ATP, 21 days RBC half life, 8 weeks)

- Treatment with mitapivat for up to 2 months shows sustained improvement in hematological parameters
- Sharp reduction in circulating immature red cells suggests amelioration of ineffective erythropoiesis
- ~50% increase in lifespan of peripheral RBCs

Source: Lucia de Franceschi et al, EHA 2016

Design of Phase 2 Study of Mitapivat in Adults with Non-transfusion-dependent β- and α-thalassemia



Primary endpoint: Hb response (≥1.0 g/dL increase in Hb from baseline in at least one assessment [W4-12])

Secondary endpoints: Safety, other markers of clinical efficacy, markers of hemolysis and erythropoetic activity

Exploratory: Markers of iron metabolism and iron overload

EudraCT 2018-002217-35; ClinicalTrials.gov NCT03692052
BID, twice daily

Clinical Proof-of-concept for Mitapivat Established in Non-transfusion-dependent Thalassemia

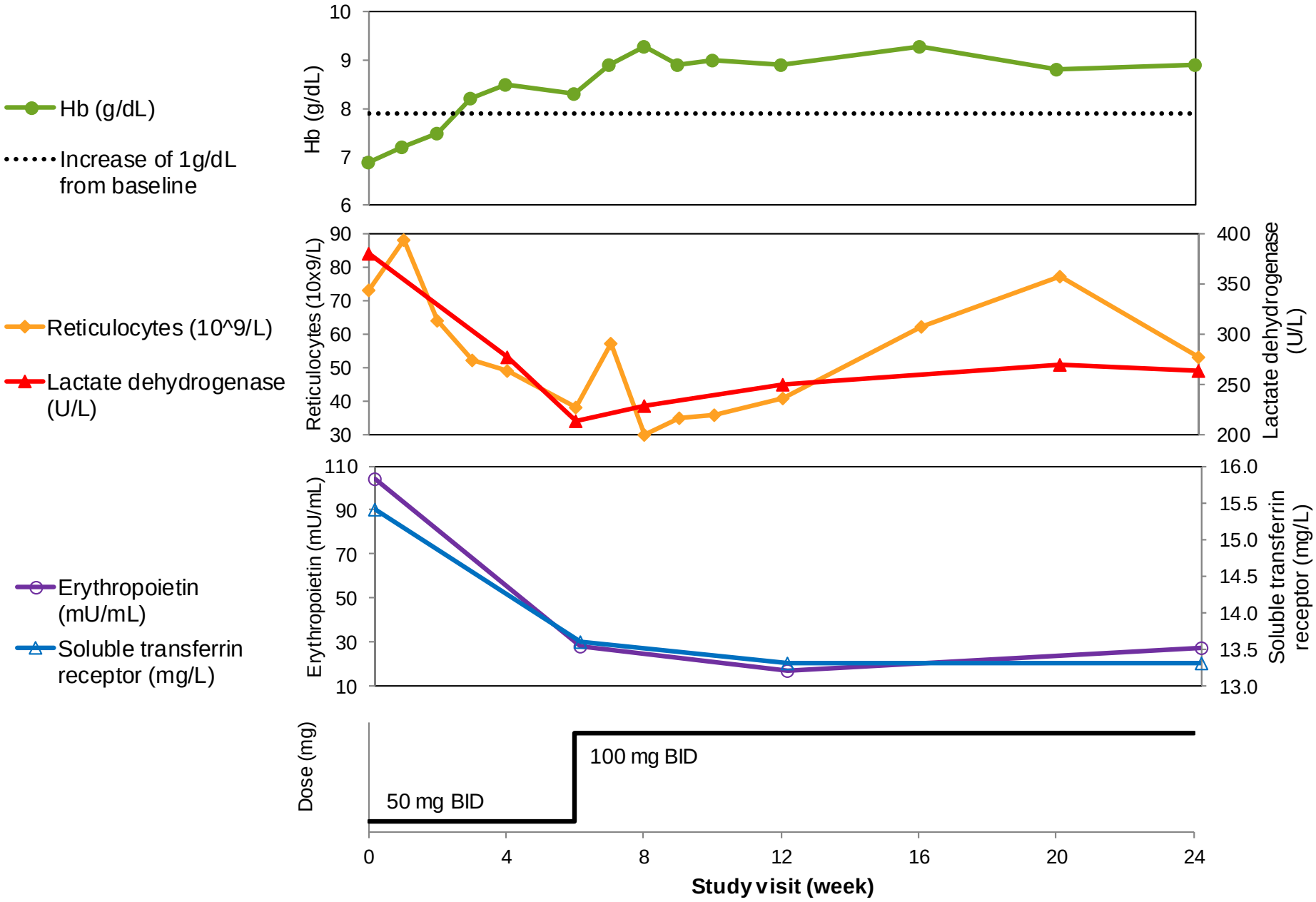
7 of 8 efficacy evaluable patients achieved a hemoglobin increase of ≥1.0 g/dL from baseline in at least one assessment (weeks 4-12)

In responding patients, the mean hemoglobin increase from baseline was 1.76 g/dL (range, 0.9–3.3 g/dL)

Majority of adverse events were Grade 1 or 2 and consistent with previously published Phase 2 data for mitapivat in patients with PK deficiency

Updated results from the Phase 2 thalassemia study will be presented at a medical meeting in the first half of 2020

Illustrative Patient Case Study: Male, 44 years old



What's Possible for PKR Activators

Today's Key Takeaways

Agios is leading the science behind IDH mutations in AML

- Multiple pathways can predict for response (JAK2) or resistance (NRAS, PTPN11) to IDH1 inhibitors in AML
- Both IDH-related and non-IDH related pathways potentially contributing to relapse to IDH1 inhibition as a monotherapy further underscoring the complexity of treating AML
- Combination therapy of TIBSOVO® + azacitidine demonstrates high rates of mutation clearance suggesting deep and durable remissions including in patients with baseline mutational profile suggestive of resistance

Mitapivat has the potential to be the first disease-modifying treatment in PK deficiency, a chronic, lifelong hemolytic anemia

- Updated DRIVE PK data demonstrate mitapivat can provide a sustained hemoglobin response and is well tolerated with chronic treatment for more than 3 years
- Significant complications and comorbidities associated with PK deficiency regardless of transfusion status

Proof-of-concept achieved for mitapivat in thalassemia

- Treatment with mitapivat induced hemoglobin increase of ≥ 1.0 g/dL in 7 of 8 evaluable patients
- Safety profile consistent with previously published Phase 2 data for mitapivat in patients with PK deficiency

Q&A