

Q1 2026 Financial Results and Business Highlights

Conference call for investors and analysts

29 April 2026



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Q1 2026 earnings call agenda

- 1 Introduction** Morgan Sanford, VP Investor Relations
- 2 CEO Opening Remarks** Brian Goff, Chief Executive Officer
- 3 Financial Results** Cecilia Jones, Chief Financial Officer
- 4 Commercial Highlights** Tsveta Milanova, Chief Commercial Officer
- 5 R&D Highlights** Sarah Gheuens, MD, PhD, Chief Medical Officer, Head of R&D
- 6 CEO Closing Remarks and Q&A**

CEO Opening Remarks

Brian Goff, Chief Executive Officer

Continued focus on delivering on 2026 strategic priorities



Execute high-impact U.S. launch for AQVESME™ (mitapivat) in thalassemia



Potential to expand PK activation franchise into sickle cell disease and LR-MDS



Unlock future value in hematology and other rare disease by advancing early-stage pipeline



Ensure long-term sustainability through disciplined capital allocation and operational efficiency

Q1 2026 – early progress toward growth inflection

Strong commercial execution

- Q1 2026 Net Revenues \$20.7M, \$8.7M in Q1 2025
- 2026 OpEx expected to be approximately flat compared to prior year
- Strong cash position – over \$1B

Progress against 2026 strategic priorities



Strong initial AQVESME thalassemia launch progress – 242 prescriptions¹



Mitapivat sNDA filing for U.S. accelerated approval in sickle cell disease in Q2 2026

Agios – positioned for near-term growth inflection and sustained longer-term value creation



Maximizing PKa franchise

- AQVESME launch execution
- Mitapivat sNDA filing in sickle cell disease



Differentiated next-gen PKa

- Tebapivat – upcoming catalysts
 - LR-MDS Phase 2b topline data H1 2026
 - Sickle cell disease Phase 2 topline data H2 2026



Pipeline diversification to drive sustained growth

- Progress early-stage programs
- Expansion in rare hematology and other rare diseases

Near-term value

Mid-term value

Long-term value

Financial Results

Cecilia Jones, Chief Financial Officer

Q1 2026 Financial Results

Statement of Operations (\$M)	Q1 2026	Q1 2025
Mitapivat Net Revenue	\$20.7	\$8.7
US Net Revenue	\$18.8	\$8.7
Ex-US Net Revenue	\$1.9	-
Cost of Sales	\$1.3	\$1.1
Research & Development Expense	\$81.1	\$72.7
Selling, General & Administrative Expense	\$48.3	\$41.5
Net (Loss) Income	(\$99.1)	(\$89.3)

Balance Sheet	Q1 2026	Q4 2025
Cash, Cash Equivalents and Marketable Securities	\$1.0B	\$1.2B

Disciplined capital allocation to drive sustained long-term value creation

Focused investment to **maximize AQVESME U.S. launch** in thalassemia

Operating expense management aligned with driving sustained long-term growth

Strategic pipeline diversification through disciplined internal and external innovation

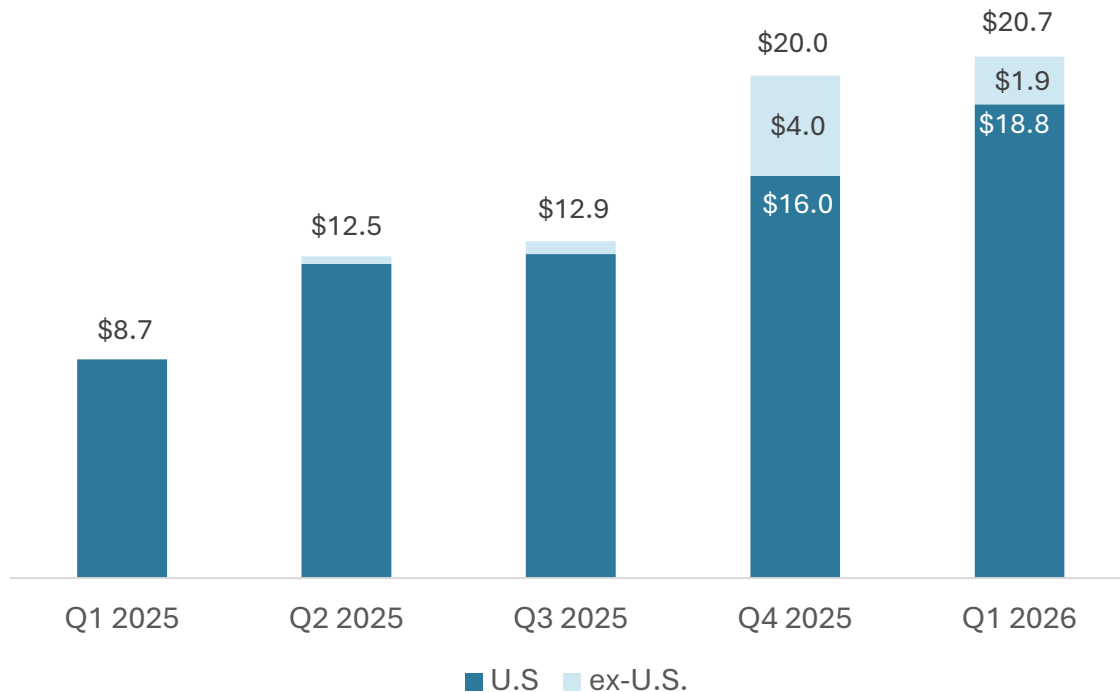
Focused on delivering 2026 strategic priorities balanced against driving long-term growth

Commercial Highlights

Tsveta Milanova, Chief Commercial Officer

Q1 2026 – robust performance driven by strong commercial execution

Mitapivat Net Revenues – Quarterly (\$M)



\$20.7M Q1 2026 Net Revenue, +138% vs prior year

Key Q1 2026 dynamics

- **\$18.8M U.S. Net Revenues**
 - Strong initial AQVESME U.S. launch execution in thalassemia
- **\$1.9M ex-U.S. Net Revenues**
 - Driven by thalassemia uptake in Saudi Arabia, reflects early market access dynamics
- Continued quarter-on-quarter variability due to ordering patterns, inventory dynamics and GtN



– early U.S. launch reflects high-quality demand



242 prescriptions¹
as of March 31st

- Strong early adoption among highly-engaged patients, exiting Q1 with growing NTDT patients
- Broadening community-based prescriber uptake
- Shorter-than-expected time to treatment, reflecting patient engagement and readiness
 - Early in launch, still anticipate 10-12 weeks on average time-to-treatment initiation



– early U.S. launch unlocking path to sustained adoption

AQVESME profile addresses unmet need in broad thalassemia patient population

- Compelling clinical profile and broad indication statement driving early adoption
- Efficient REMS onboarding, after operationalization in late January
- Comprehensive patient services supporting continuity of treatment

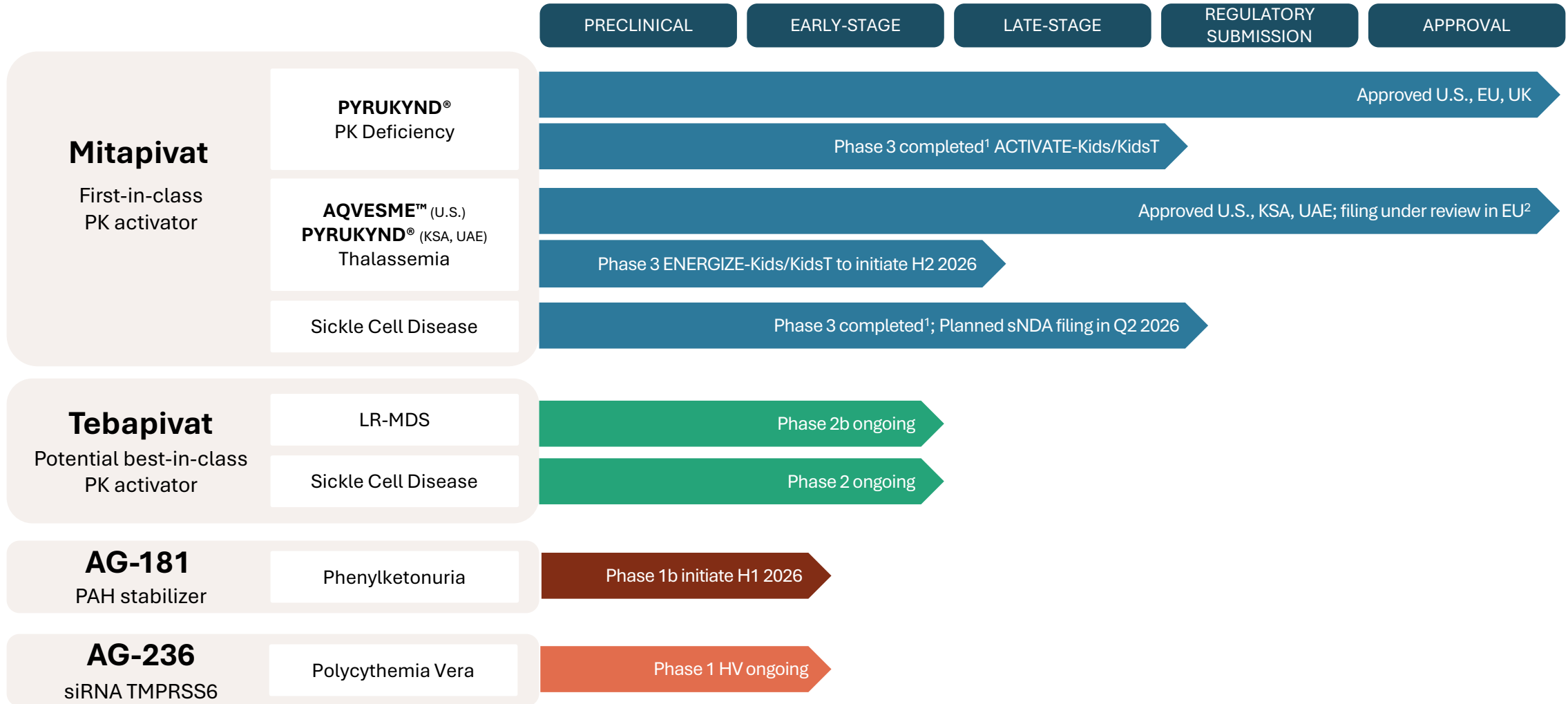
Priorities in coming quarters to drive sustained AQVESME adoption

- ▶ Expand prescriber breadth across community and academic practices
- ▶ Broaden adoption into NTD patients (~2/3 of adult diagnosed population in U.S.)
- ▶ Payer engagement and education to further shape coverage policy

R&D Highlights

Sarah Gheuens, MD, PhD,
Chief Medical Officer, Head of R&D

Continued pipeline momentum

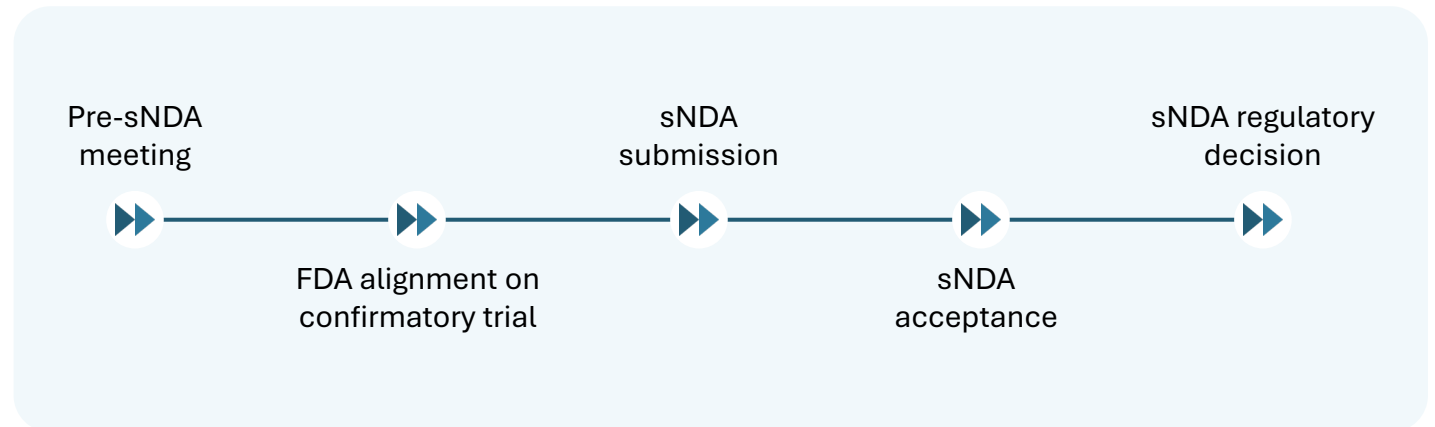


1. Defined as completion of double-blind randomized portion of the trial. 2. Positive Committee for Medicinal Products for Human Use (CHMP) opinion disclosed 17 October 2025. PK = pyruvate kinase; EU = European Union; UK = United Kingdom; KSA = Kingdom of Saudi Arabia; UAE = United Arab Emirates; LR-MDS = lower-risk myelodysplastic syndrome; PAH = Phenylalanine Hydroxylase; siRNA = small interfering RNA; TMPRSS6 = transmembrane protease serine 6; HV = healthy volunteer; sNDA = supplemental new drug application.

Mitapivat advancing toward U.S. accelerated approval in sickle cell disease

- Pre-sNDA meeting completed
- Confirmatory trial designed to demonstrate clinical benefit, balancing operationally feasible design and execution
- Plan to share additional RISE UP data at upcoming medical congress

U.S. accelerated approval pathway regulatory process¹



Pleased with progress of FDA engagement and now expect to file sNDA in Q2 2026

1. Figure is illustrative only. PK = pyruvate kinase; FDA = food and drug administration; sNDA = supplemental new drug application.

Tebapivat – next-generation PK activator – distinct design supporting potential for differentiated clinical profile

Next-generation PK activator

Structurally differentiated,
next-generation PK activator

Potent dual PKR/PKM2 activator

PK/PD properties support QD
dosing without taper

Early data supports potential differentiation vs first-generation PKa

Sickle Cell Disease



- Studied in healthy volunteers and sickle cell disease patients (Phase 1)
- Long half-life (~87–93 h), enabling once-daily dosing¹
- +1.9 g/dL mean Hb increase at 5 mg QD in SCD¹
- Dose-dependent ↓2,3-DPG / ↑ATP with PD durability up to 4 weeks post-dose¹
- Potent PKM2 activation with antifibrotic effects (preclinical)²

Lower Risk-MDS



- ≥1.5 g/dL mean Hb increase achieved in one patient (Weeks 8–16)³
- 40% of low-transfusion-burden patients achieved transfusion independence³
- ~50% lower exposure vs NH, supporting evaluation of higher doses in broader LR-MDS population³
- Ex vivo RBC data show ↑PK activity, ↑ATP, and improved red cell function⁴

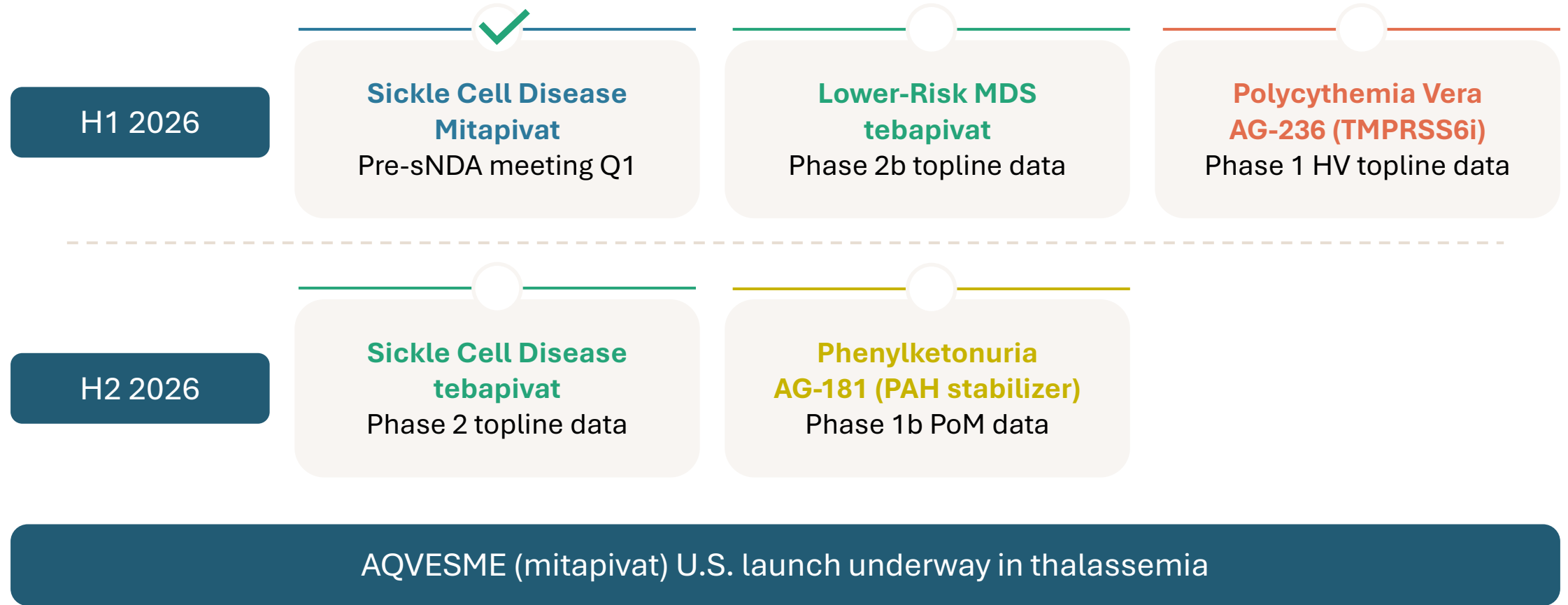
Phase 2b LR-MDS topline data in H1 2026; Phase 2 Sickle Cell Disease topline data in H2 2026

1. Xu JZ et al. Results from a Phase 1 Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Tebapivat (AG-946) in Patients with Sickle Cell Disease. 2. Nguyen et al., Blood (ASH 2024), Abstract #1107. Preclinical mouse models of nephritis (anti-GBM) demonstrating PKM2-mediated antifibrotic effects of PK activation. 3. Phase 2a LR-MDS trial, data on file. 4. Fattizzo et al. American Journal of Hematology (2024). Glycolytic activity and in vitro effect of the pyruvate kinase activator AG-946 in red blood cells from low-risk myelodysplastic syndromes patients. DOI: 10.1002/ajh.27300. PK = pyruvate kinase; PK/PD = pharmacokinetic/pharmacodynamic; QD = once-daily; SCD = sickle cell disease; 2,3-DPG = 2,3-diphosphoglycerate; ATP = Adenosine Triphosphate; Hb = hemoglobin; NH = normal healthy volunteer; LR-MDS = lower risk myelodysplastic syndrome; RBC = red blood cell; NHV = normal healthy volunteers;

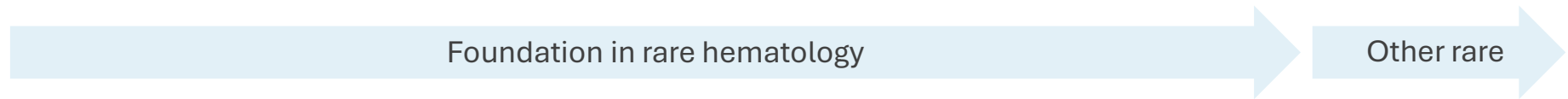
CEO Closing Remarks

Brian Goff, Chief Executive Officer

Strong catalyst flow across pipeline in 2026



Agios – pipeline advancement driving leadership in rare hematology and other rare diseases



	PK deficiency	Thalassemia	Sickle Cell Disease	LR-MDS	Polycythemia Vera	PKU
	PYRUKYND	AQVESME/ PYRUKYND¹	Mitapivat/ Tebapivat	Tebapivat	AG-236	AG-181
Mechanism of action	PK activator	PK activator	PK activator	PK activator	TMPRSS6i siRNA	PAH stabilizer
Development stage	Approved	Approved	Registration/ Phase 2	Phase 2b	Phase 1 HV	Phase 1b in PKU patients
Total market opportunity by 2030	Ultra-rare	\$1B+	\$3B+	\$4.5B+	\$1B+	\$1B+

Agios pipeline >\$10B in potential PYS opportunity in 2030

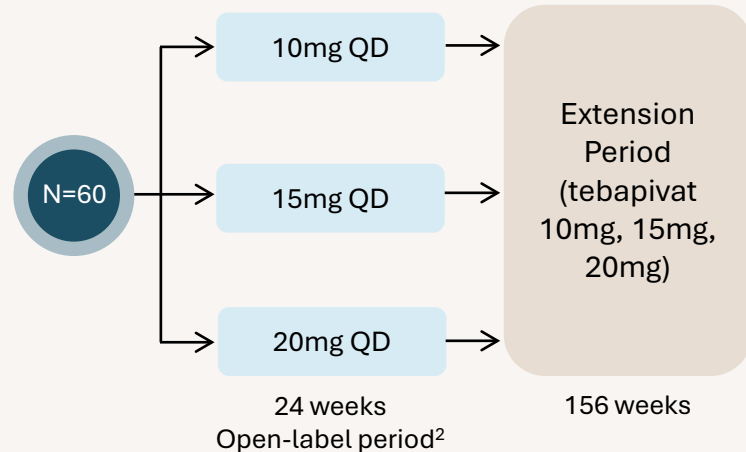
*EvaluatePharma forecasted U.S. market value in 2030; LR-MDS represents a subset of the provided market size and prevalence is estimated ~70% of total MDS patients. 1. Mitapivat approved and marketed in U.S. under brand name AQVESME for thalassemia; approved and marketed under brand name PYRUKYND outside the U.S. PK = pyruvate kinase; sNDA = supplemental new drug application; LR-MDS = lower risk myelodysplastic syndrome; TMPRSS6i = transmembrane protease, serine 6 inhibitor; PAH = phenylalanine hydroxylase; PKU = Phenylketonuria.

Q&A session

Appendix

Appendix – tebapivat Phase 2b Lower Risk-MDS trial

Tebapivat Phase 2b trial for LR-MDS¹



Phase 2b topline data expected
H1 2026

Key Inclusion Criteria

- Lower-risk MDS³
- Transfusion dependent⁴
- Hb <10.0 g/dL
- Up to 2 prior therapies, including ESAs and/or luspatercept

Primary Endpoint

- Proportion of participants with transfusion independence for at least 8 consecutive weeks⁵ during the core 24 week treatment period

Key Exclusion Criteria

- Known history or AML or secondary MDS
- Prior exposure to a PK activator, IDH inhibitors, IST, stem cell transplant
- Currently receiving imetelstat, iMiDs, HMAs

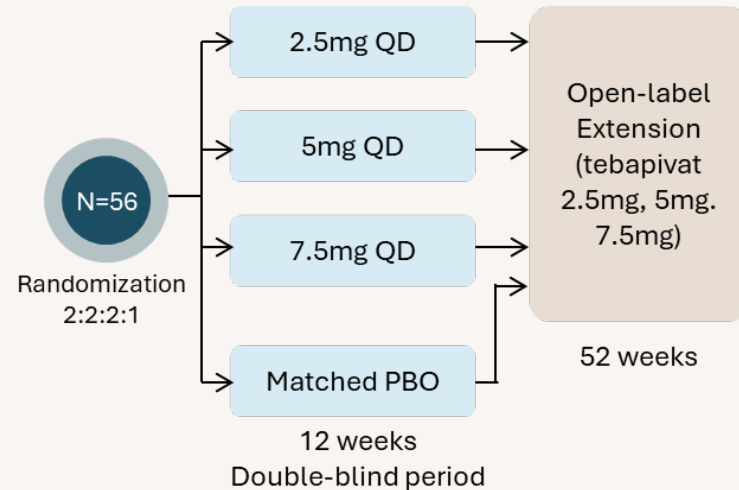
Secondary Endpoints

- Change in hemoglobin
- Transfusion independence for 12 weeks
- Additional measures of anemia
- PK/PD biomarkers
- Safety

1. Full trial details, including participation criteria and study plan, can be found at [clinicaltrials.gov NCT05490446](https://clinicaltrials.gov/NCT05490446); 2. Enrollment completion of one cohort triggers opening of enrollment in the next cohort; 3. Risk score: ≤ 3.5 according to IPSS-R classification (WHO classification; Arber et al, 2016); 4. LTB or HTB according to revised IWG 2018 criteria; 5. Transfusion independence defined as transfusion-free for ≥ 8 consecutive weeks during core period. LR-MDS = lower risk myelodysplastic syndrome; QD = once-daily; ESAs = Erythropoiesis-stimulating agents; AML = acute myeloid leukemia; PK = pyruvate kinase; IST = immunosuppressive therapy; iMiDs = Immunomodulatory Imide drugs; HMAs = Hypomethylating Agents; PK/PD = pharmacokinetics/pharmacodynamics.

Appendix – tebapivat Phase 2 Sickle Cell Disease trial

Tebapivat Phase 2 trial for Sickle Cell Disease¹



Phase 2 topline data expected
H2 2026

Key Inclusion Criteria

- Hb ≥ 5.5 - ≤ 10.5 g/dL
- HU use permitted, if dose has been stable for at least 90 days before randomization²

Primary Endpoint

- Hb response³ at baseline, and from Weeks 10 - 12

Key Exclusion Criteria

- >10 SCPCs in the past 12 months
- Receiving treatment with voxelotor, crizanlizumab, L-glutamine, or hematopoietic stimulating agents within 90 days before randomization

Secondary Endpoints

- Average change from baseline in Hb concentration
- Average change from baseline in markers of hemolysis and erythropoiesis
- PROs: PROMIS Fatigue, PROMIS Pain, ASCQ-Me
- Safety

1. Full study details, including participation criteria and study plan can be found at [clinicaltrials.gov NCT06924970](https://clinicaltrials.gov/NCT06924970); 2. Discontinuation of hydroxyurea requires a 90-day washout before providing informed consent; 3. Hb response defined as ≥ 1.0 g/dL increase in hemoglobin from Weeks 10-12. QD = once-daily; PBO = placebo; Hb = hemoglobin; HU = hydroxyurea; SCPCs = sickle cell pain crises; PROs = patient reported outcomes; PROMIS = Patient-Reported Outcomes Measurement Information System; ASCQ-Me = adult sickle cell quality of life measure.