Q1 2026 Financial Results and Business Highlights

Conference call for investors and analysts

29 April 2026

agios

Forward Looking Statements

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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

LR-MDS = lower-risk myelodysplastic syndrome; PK = pyruvate kinase.

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

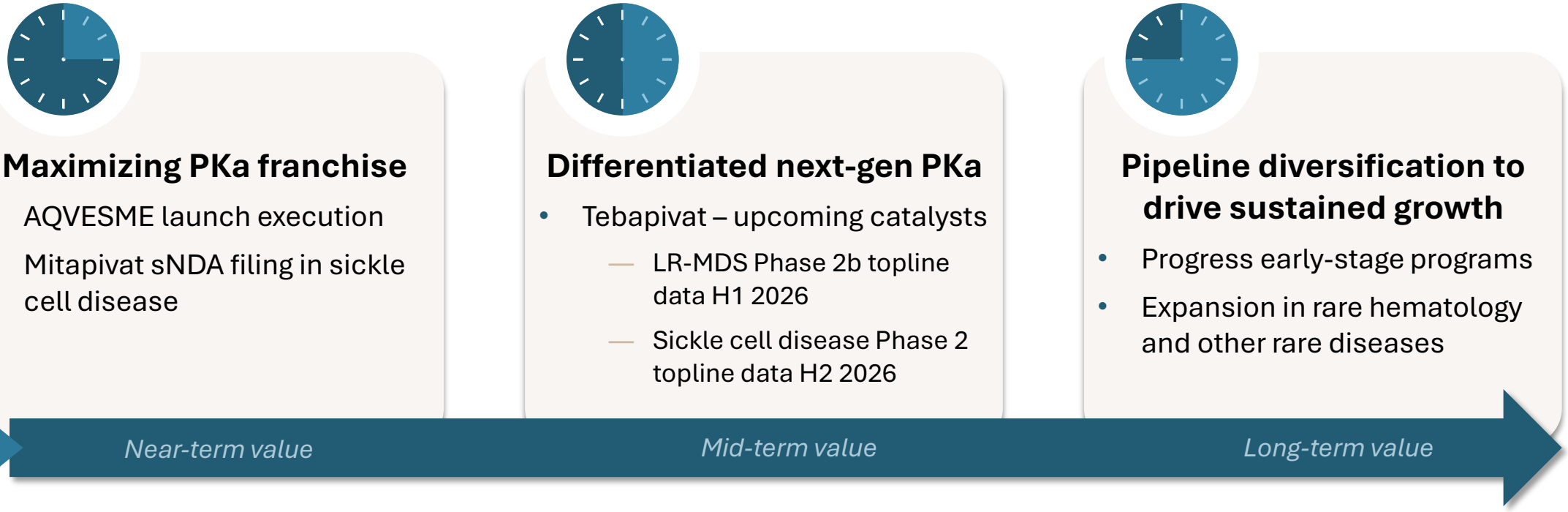
Progress against 2026 strategic priorities

Strong initial AQVESME thalassemia launch progress – 242 prescriptions[1]

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

1. As of March 31st; reflects prescriptions from Risk Evaluation and Mitigation Strategy (REMS)-certified physicians. sNDA = supplemental new drug application.

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



PKa = pyruvate kinase activator/activation; sNDA = supplemental new drug application; LR-MDS = lower-risk myelodysplastic syndrome.

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

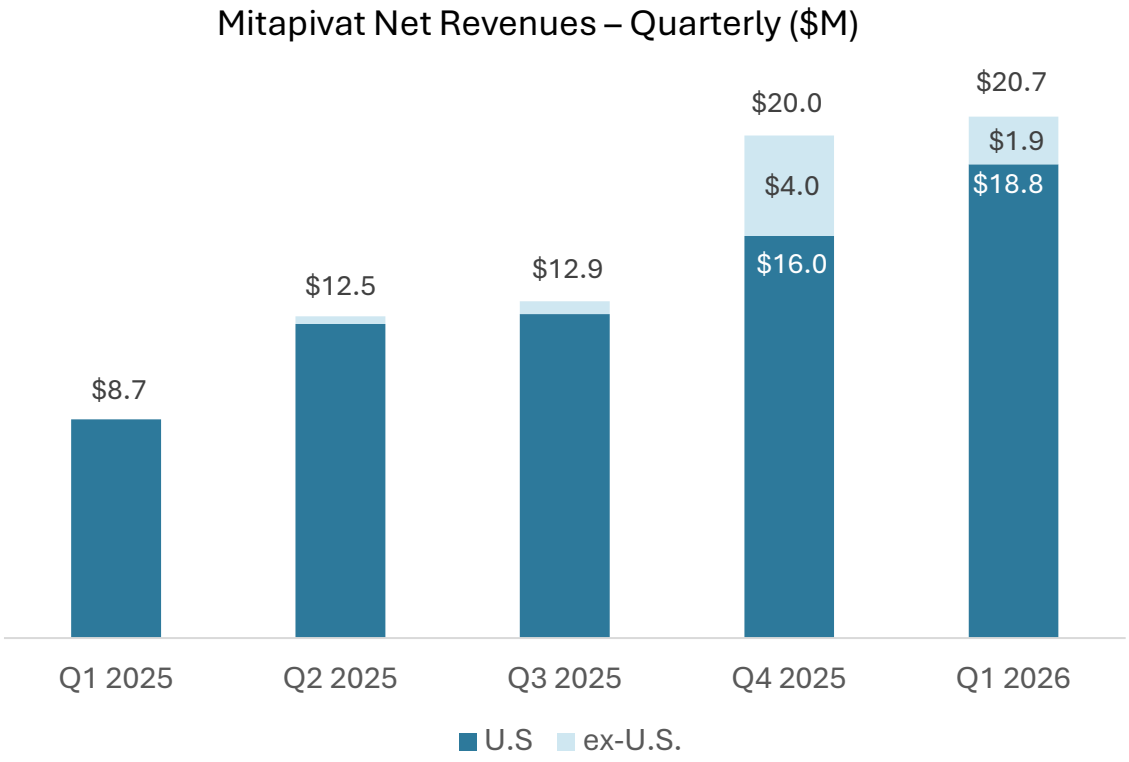
Strategic pipeline diversification through disciplined internal and external innovation

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



$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

PK = pyruvate kinase; GtN = Gross to Net.

Aqvesme™ (mitapivat) tablets 100mg – early U.S. launch reflects high-quality demand

242 prescriptions[1]
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

1. reflects prescriptions from risk evaluation and mitigation strategy certified physicians as of March 31, 2026 from date of FDA approval, December 23, 2025. NTDT = non-transfusion dependent thalassemia.

Aqvesme™ (mitapivat) tablets 100mg – 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

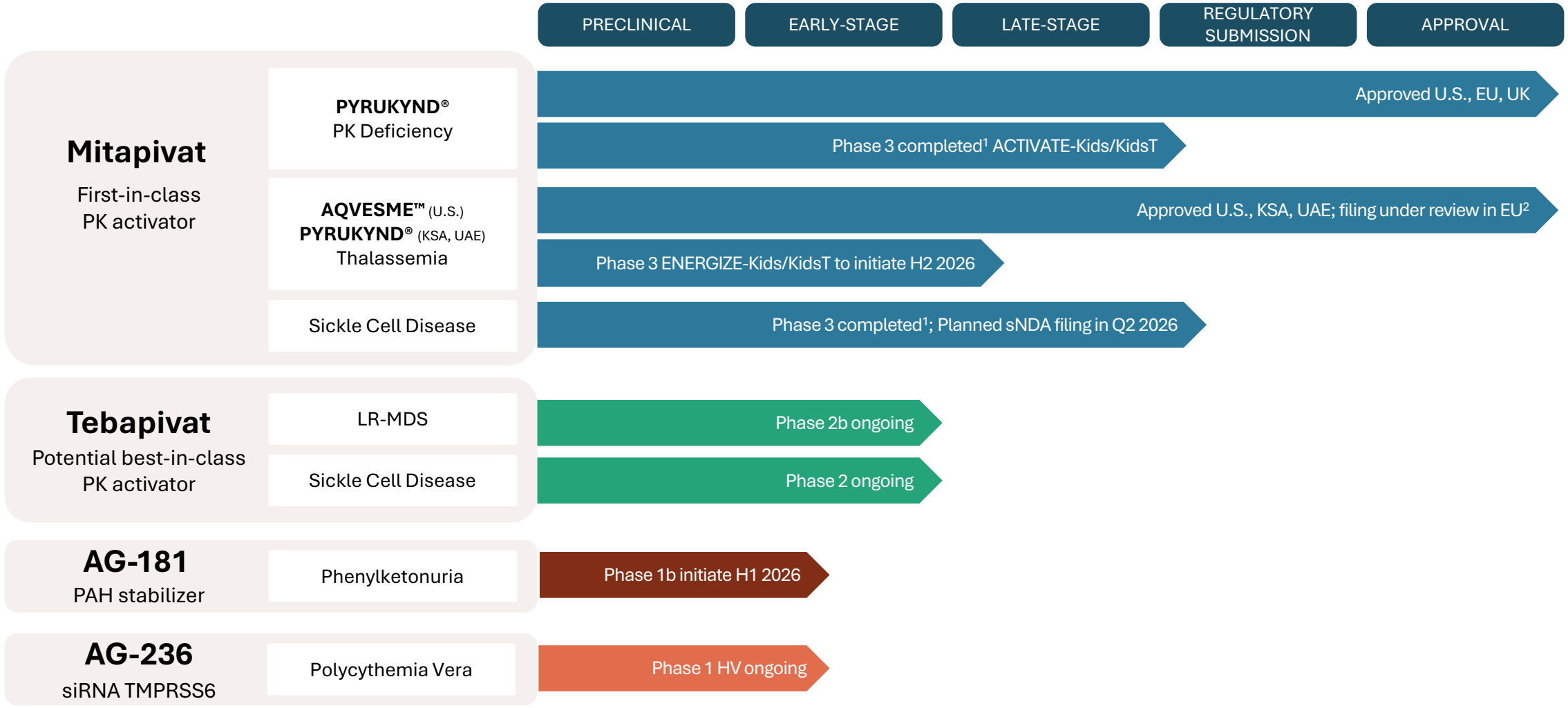
- Expand prescriber breadth across community and academic practices
- Broaden adoption into NTD patients (~2/3 of adult diagnosed population in U.S.)
- Payer engagement and education to further shape coverage policy

REMS = risk evaluation and mitigation strategy; NTD = non-transfusion dependent.

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 process[1]

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 dosing[1]
- +1.9 g/dL mean Hb increase at 5 mg QD in SCD[1]
- Dose-dependent ↓2,3-DPG / ↑ATP with PD durability up to 4 weeks post-dose[1]
- 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 independence[3]
- ~50% lower exposure vs NH, supporting evaluation of higher doses in broader LR-MDS population[3]
- Ex vivo RBC data show ↑PK activity, ↑ATP, and improved red cell function[4]

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

H1 2026

Sickle Cell Disease
Mitapivat
Pre-sNDA meeting Q1

Lower-Risk MDS
tebapivat
Phase 2b topline data

Polycythemia Vera
AG-236 (TMPRSS6i)
Phase 1 HV topline data

H2 2026

Sickle Cell Disease
tebapivat
Phase 2 topline data

Phenylketonuria
AG-181 (PAH stabilizer)
Phase 1b PoM data

AQVESME (mitapivat) U.S. launch underway in thalassemia

sNDA = supplemental new drug application; MDS = myelodysplastic syndrome; TMPRSS6i = transmembrane protease, serine 6 inhibitor; HV = healthy volunteer; PAH = phenylalanine hydroxylase; PoM = proof of mechanism.

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

Foundation in rare hematology → Other rare

	PK deficiency	Thalassemia	Sickle Cell Disease	LR-MDS	Polycythemia Vera	PKU
	PYRUKYND	**AQVESME/ PYRUKYND[1]**	**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+

>$10B total estimated global market size for current pipeline indications by 2030*

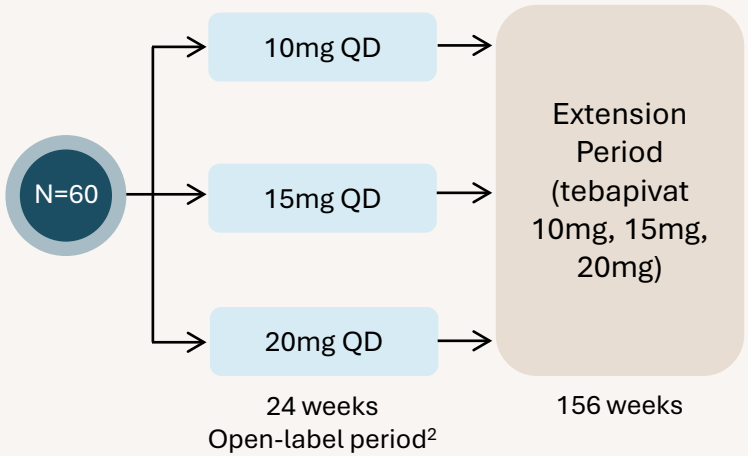
*EvaluatePharma forecasted U.S. market value in 2030; LR-MDS represents a subset of the provided market size and prevalence is estimated ~70% of total MDS patients. 1. Mitapivat approved and marketed in U.S. under brand name AQVESME for thalassemia; approved and marketed under brand name PYRUKYND outside 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-MDS[1]



Phase 2b topline data expected H1 2026

Key Inclusion Criteria

- Lower-risk MDS[3]
- Transfusion dependent[4]
- 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 weeks[5] 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

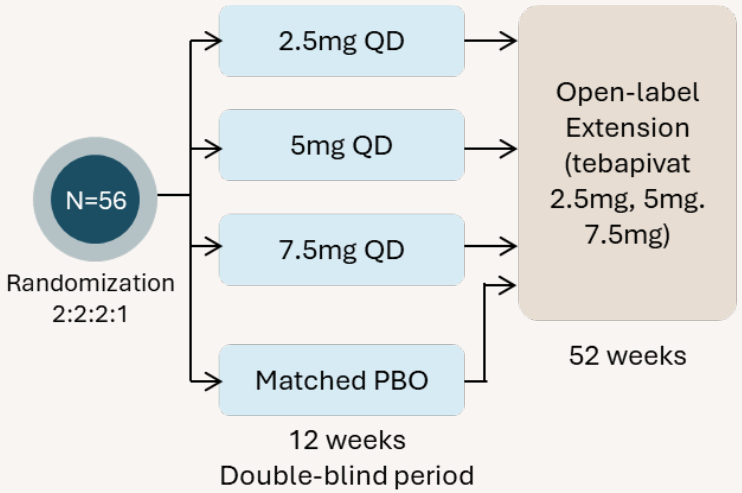
Secondary Endpoints

- 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; 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 Disease[1]



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 randomization[2]

Primary Endpoint

- Hb response[3] 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; 2. Discontinuation of hydroxyurea requires a 90-day washout before providing informed consent; 3. Hb response defined as ≥1.0g/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.