Q2 2026 Financial Results and Business Highlights

Conference call for investors and analysts

30 July 2026

agios

Forward Looking Statements

This presentation and various remarks we make during this presentation contains 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), AQVESME™ (mitapivat), tebapivat, cevidoplenib, AG-236 and AG-181; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including mitapivat, tebapivat, cevidoplenib, AG-236 and AG-181; Agios' expectations for the review of marketing applications for mitapivat by regulatory agencies, including the FDA; Agios' strategic vision and goals; 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 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 press release 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 royalty payments related to the sale of its oncology business or any milestone or royalty payments related to its in-licensing of AG-236 and cevidoplenib, 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 press release 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.

Q2 2026 earnings call agenda

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2	**CEO Opening Remarks**	Brian Goff, Chief Executive Officer
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4	**Commercial Highlights**	Tsveta Milanova, Chief Commercial Officer
5	**R&D Highlights**	Sarah Gheuens, MD, PhD, Chief Medical Officer, Head of R&D
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CEO Opening Remarks

Brian Goff, Chief Executive Officer

Agios – advancing a diversified rare disease portfolio with multiple growth drivers

Thalassemia	Sickle Cell Disease	Immune Thrombocytopenia	Polycythemia Vera	Phenylketonuria
First medicine approved for broad thalassemia population	Strong anti-hemolytic profile, addressing key driver of mortality	Next-gen SYK inhibitor, improved tolerability may drive durability	TMPRSS6i designed for sustained hepcidin control, extended dosing	PAH enzyme stabilizer, potential for durable phenylalanine control
AQVESME™/PYRUKYND® approved U.S., EU, KSA, UAE	**Mitapivat** sNDA accepted with Priority Review, PDUFA goal date November 1, 2026	**Cevidoplenib** Phase 3 planned for H1 2028	**AG-236** Phase 2/3 trial planned for H2 2026	**AG-181** proof-of-mechanism data in PKU patients H2 2026

U.S. = United States; EU = European Union; KSA = Kingdom of Saudi Arabia; UAE = United Arab Emirates; sNDA = supplemental New Drug Application; PDUFA = Prescription Drug User Fee Act; SYK = spleen tyrosine kinase; TMPRSS6i = transmembrane serine protease 6 inhibitor; PAH = phenylalanine hydroxylase; Phe = phenylalanine; PKU = phenylketonuria.

Q2 2026 – commercial growth, pipeline expansion and financial discipline

Commercial launch momentum

- $44.7M worldwide net revenue, including $40.9M in U.S. net revenues
- 442 cumulative AQVESME prescriptions from REMS-certified HCPs[1]

Near-term growth catalyst

- Mitapivat sNDA accepted under accelerated approval pathway with Priority Review in SCD
- PDUFA goal date November 1, 2026

Strategic pipeline diversification

- In-licensed cevidoplenib, highly-selective, next-gen SYK inhibitor in ITP
- Advancing AG-236 into operationally seamless Phase 2/3 program

Strong capital position

- ~$965B in cash, cash equivalents and marketable securities
- Well capitalized to support commercial growth and pipeline execution

1. Prescriptions from REMS-certified HCPs linked to completed patient start forms. REMS = Risk Evaluation and Mitigation Strategy; HCPs = health care physicians; sNDA = supplemental new drug application; SCD = sickle cell disease; PDUFA = Prescription Drug User Fee Act; SYK = spleen tyrosine kinase; ITP = immune thrombocytopenia.

Financial Results

Cecilia Jones, Chief Financial Officer

Q2 2026 Financial Results

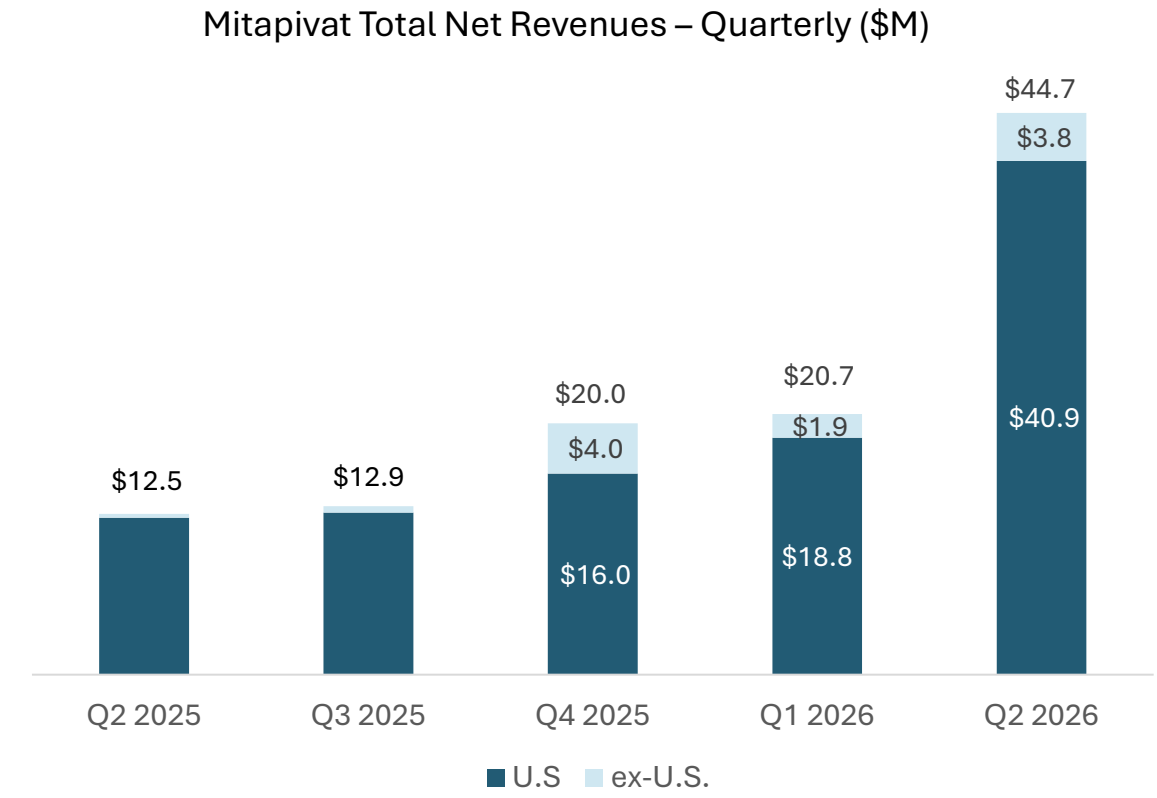
Statement of Operations ($M)	Q2 2026	Q2 2025
Mitapivat Net Revenue	$44.7	$12.5
U.S. Net Revenue	$40.9	$12.2
Ex-U.S. Net Revenue	$3.8	$0.3
Cost of Sales	$3.0	$1.7
Research & Development Expense	$100.8	$91.9
Selling, General & Administrative Expense	$51.5	$45.9
Net (Loss) Income	($100.7)	($112.0)

Balance Sheet	Q2 2026	Q4 2025
Cash, Cash Equivalents and Marketable Securities	$964.8M	$1.2B

Commercial Highlights

Tsveta Milanova, Chief Commercial Officer

Q2 2026 performance reflects strong launch execution and expanding AQVESME/PYRUKYND (mitapivat) demand



$44.7M Q2 2026 Total Net Revenue, up 3.6x vs prior year

Key Q2 2026 dynamics

- $40.9M U.S. Net Revenues
  - AQVESME launch execution in thalassemia, driven by increased demand
  - ~$5M one-time stocking and GtN benefit[1]
- $3.8M ex-U.S. Net Revenues
  - Driven by thalassemia anticipated demand in EU and continued early demand in the GCC
- Continued quarter-on-quarter variability due to ordering patterns, inventory dynamics and GtN

U.S. = united states; GtN = gross to net; EU = European Union; GCC = Gulf Cooperation Council. 1. In Q2 2026, approximately $5M in combined benefit from mitapivat stocking due to order timing and gross to net favorability.

Aqvesme™ (mitapivat) tablets 100mg – Q2 execution reinforces confidence in thalassemia launch trajectory

442 cumulative prescriptions[1]
as of June 30th

Patient mix reflects growing proportion of NTDT patients

Time to treatment initiation starting to approach expected **10-12 week average**

75%

Coverage policies established for **~75% of covered lives**

Broad and expanding prescriber base

As launch matures, plan to discontinue reporting prescriptions from REMS-certified HCPs for patients with completed start forms[1] after Q3 2026

1. Prescriptions from REMS-certified HCPs linked to completed patient start forms as of June 30, 2026 from date of FDA approval, December 23, 2025. NTDT = non-transfusion dependent thalassemia; REMS = risk evaluation and mitigation strategy; HCP = healthcare physician.

Aqvesme™ (mitapivat) tablets 100mg – thalassemia launch entering next phase in H2

Early launch wave
H1 2026

- Q1 benefitted from PDUFA delay, higher launch anticipation
- Highly motivated patients and prescribers
- Time to treatment initiation below anticipated average of 10-12 weeks

Broadening patient adoption
H2 2026

- Adoption expanding into broader NTDT population
- Time to treatment initiation aligns with expected 10–12 week average
- First cohort of patients reach ~6-month response evaluation window

Maturing launch
2027+

- Durable base of patients on therapy
- Real-world evidence continues to build
- Broad payer coverage supports patient access

H2 2026 dynamics reflect a broadening patient mix, evolving time to treatment initiation and the first response evaluation window for earliest AQVESME patient cohort

PDUFA = Prescription Drug User Fee Act; NTDT = non transfusion dependent thalassemia.

Focused launch planning supports potential first-in-class opportunity for mitapivat in sickle cell disease

Compelling initial launch target within a broader market opportunity

Potential to expand over time

~75,000 diagnosed sickle cell disease patients (≥16 years)	**~25,000 actively treated or in need of therapy**

Initial launch focused on majority of patients with hemolytic profile, defined by low or declining Hb

Key launch preparation ahead of potential U.S. approval

- **Target prescribers** – mapped and prioritizing
- **Commercial readiness** – enhancing infrastructure and field readiness
- **Market access** – advancing payer planning
- **Disease awareness** – strengthening education and community engagement

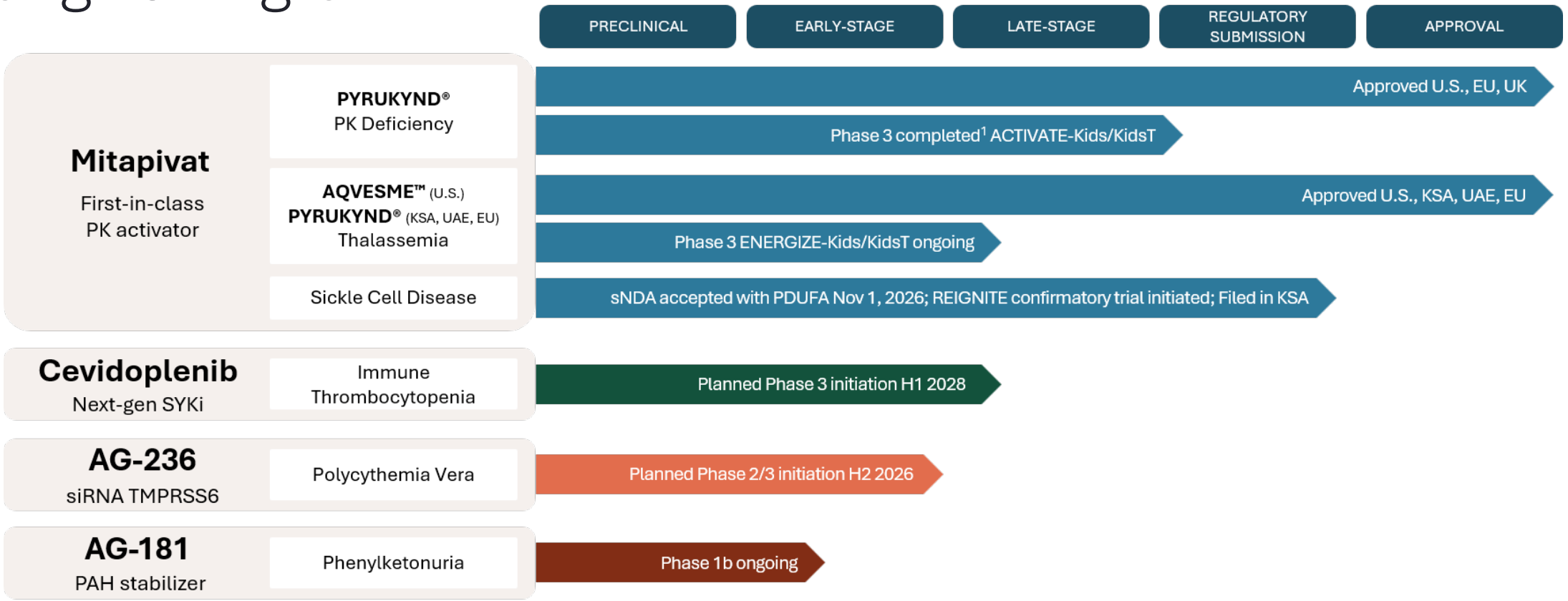
Mitapivat sNDA under priority review with November 1, 2026, PDUFA goal date

Hb = hemoglobin; U.S. = united states; sNDA = supplemental new drug application; PDUFA = Prescription Drug User Fee Act.

R&D Highlights

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

Focused pipeline advancing multiple opportunities for long-term growth



First patient dosed in REIGNITE Phase 3 confirmatory trial for mitapivat in sickle cell disease

1. Defined as completion of double-blind randomized portion of the trial. PK = pyruvate kinase; EU = European Union; UK = United Kingdom; KSA = Kingdom of Saudi Arabia; UAE = United Arab Emirates; sNDA = supplemental new drug application; PDUFA = Prescription Drug User Fee Act; PAH = Phenylalanine Hydroxylase; siRNA = small interfering RNA; SYKi = spleen tyrosine kinase inhibitor; TMPRSS6 = transmembrane protease serine 6; HV = healthy volunteer; sNDA = supplemental new drug application.

Cevidoplenib in-licensing adds next-generation SYK inhibitor designed for durable platelet control in ITP

	First-generation SYKi	Cevidoplenib
Efficacy	Inconsistent target inhibition associated with variable PLT response	Sustained target inhibition to support durable PLT control
Kinase selectivity	Limited selectivity → off-target effects	Optimized selectivity
PK/exposure	Variable exposure → inconsistent inhibition	BID dosing enables consistent exposure
Safety/tolerability	Limits durability and dosing	Designed to support efficacy with chronic use

Note: cross-trial comparisons limited by differing trials and endpoints

Cevidoplenib – next-generation, highly-selective SYK inhibitor

Dose-dependent activity supports target inhibition

Favorable safety profile with no DLTs observed through Phase 2

Designed to expand therapeutic window (selectivity + PK)

Cevidoplenib – designed to deliver sustained efficacy with tolerability suited for chronic use

SYK = spleen tyrosine kinase; SYKi = spleen tyrosine kinase inhibitor; ITP = immune thrombocytopenia; BID = twice-daily; DLT = dose-limiting toxicities; PK = pharmacokinetic; PLT = platelet.

Strong presence at the 2026 EHA Congress with data across thalassemia and sickle cell disease

10 abstracts accepted — RISE UP Phase 3 selected for EHA plenary session

RISE UP Phase 3 trial in sickle cell disease[1] – new PRO and transfusion burden data

41% fewer patients requiring transfusion

56% reduction in RBC units transfused

Hb responders reported improvements on pain and physical function

SATISFY Phase 2 trial – 56-week follow-up results[2]

48% achieved Hb response at 56 weeks

+1.1 g/dL mean Hb improvement

↓ iron burden: lower ferritin and liver iron

ENERGIZE Phase 3 Open-label Extension in NTDT

~60% continuing on mitapivat achieved Hb response

~50% of placebo-switch patients achieved Hb response

43.6 weeks mean response duration, up from 17.9

ENERGIZE Phase 3 sub-group – higher baseline Hb ≥9.5g/dL in NTDT

38.9% Hb response vs 0% placebo (Hb≥9.5g/dL)

1.5 g/dL mean Hb rise in responders

5.1-pt greater FACIT-Fatigue improvement vs +0.8 placebo

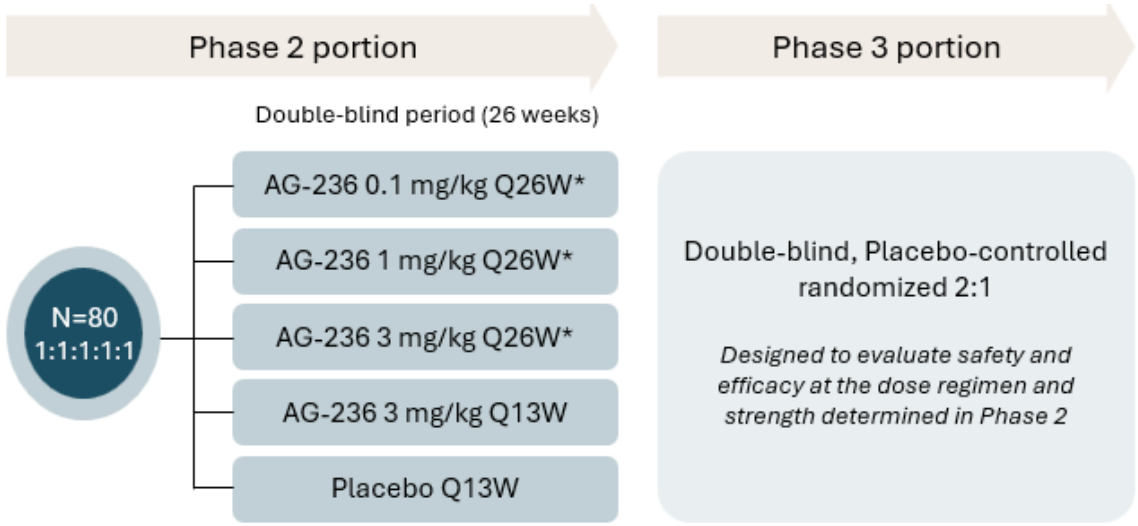
1. RISE UP Phase 3 trial results: mitapivat achieved Hb response (≥1.0 g/dL increase from baseline in avg Hb, wks 24–52) in 40.6% vs. 2.9% placebo (p<0.0001); annualized SCPC rate 2.62 vs. 3.05 placebo (p=0.1213, ns).
2. Effects of mitapivat on iron burden and spleen size in erythrocyte membranopathies and congenital dyserythropoietic anemia type II. EHA = European Haematology Association; PRO = patient reported outcomes; RBC = red blood cell; NTDT = non-transfusion dependent thalassemia; Hb = hemoglobin; FACIT = Functional Assessment of Chronic Illness Therapy.

Advancing AG-236 into operationally seamless Phase 2/3 program in polycythemia vera on positive Phase 1 HV data

Addressing broad target population
Includes patients requiring frequent phlebotomy and those on stable cytoreductive therapy

Efficient registrational strategy
Operationally seamless Phase 2/3 design

AG-236 – potential chronic disease differentiation
Sustained hepcidin and iron regulation | Potential for Q6M dosing | No titration required



Key eligibility – Adults with polycythemia vera requiring frequent phlebotomy (≥3 in 6 months or ≥5 in 12 months, including ≥1 within 3 months of randomization)

*All patients receive AG-236 or placebo every 13 weeks throughout the randomized double-blind period to maintain blinding

Advancing AG-236 through operationally seamless Phase 2/3 strategy to optimize therapeutic window and support rapid progression toward registration

HV = healthy volunteer; Q6M = every 6-months; Q26W = every 26-weeks; Q13W = every 13-weeks.

CEO Closing Remarks

Brian Goff, Chief Executive Officer

Continued execution positions Agios for multiple near- and long-term value drivers

Execute high-impact U.S. launch for AQVESME™ (mitapivat) in thalassemia	**442 cumulative prescriptions**[1] for AQVESME +200 in Q2 2026
Potential to expand PK activation into sickle cell disease	Mitapivat sNDA accepted with Priority Review and **PDUFA goal date November 1, 2026**
Unlock future value in hematology and other rare disease by advancing early-stage pipeline	**Advancing AG-236** into Phase 2/3 program; In-licensed **next-gen SYKi, cevidoplenib** in ITP
Ensure long-term sustainability through disciplined capital allocation and operational efficiency	Excluding $25M upfront for in-licensing of next-gen SYKi, expect **FY 2026 OpEx to be approximately flat vs FY 2025**

1. Prescriptions from REMS-certified HCPs linked to completed patient start forms as of June 30, 2026 from date of FDA approval, December 23, 2025. PK = pyruvate kinase; sNDA = supplemental new drug application; PDUFA = Prescription Drug User Fee Act; SYKi = spleen tyrosine kinase inhibitor; ITP = immune thrombocytopenia.

2026 catalyst progress sharpens focus on highest-value opportunities

H1 2026

Sickle Cell Disease
Mitapivat
Pre-sNDA meeting Q1

Lower-Risk MDS
Tebapivat
Phase 2b topline data

Clinical activity observed did not meet Agios' threshold for advancement

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

Support advancement to Phase 2/3 operationally seamless program

H2 2026

Sickle Cell Disease
Tebapivat
Phase 2 topline data

Not meaningfully differentiated to advance, validates PKa mechanism

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

Sickle Cell Disease
Mitapivat
PDUFA November 1, 2026

New catalyst – following sNDA acceptance with Priority Review

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; PDUFA = Prescription Drug User Fee Act; U.S. = united states.

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

Diversified across rare hematology → Beyond hematology

	PK Deficiency	Thalassemia	Sickle Cell Disease	ITP	Polycythemia Vera	PKU
	PYRUKYND	**AQVESME**	**Mitapivat**	**Cevidoplenib**	**AG-236**	**AG-181**
Mechanism of action	PK activator	PK activator	PK activator	SYK inhibitor	siRNA TMPRSS6i	PAH stabilizer
Development stage	Approved	Approved	Registration	Completed Phase 2	Phase 2/3	Phase 1b in PKU patients
Global indication market value in 2030[1]	Ultra-rare	$1B+	$3B+	$3B+	$1B+	$1B+

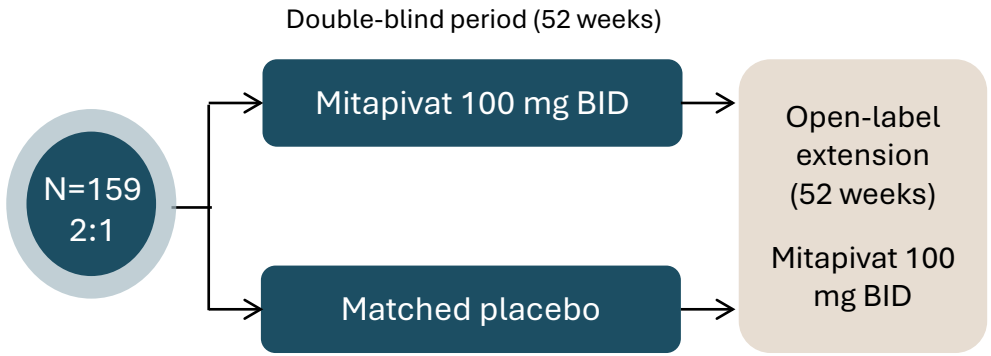
Agios' expanding pipeline focused on rare disease markets valued at >$10B by 2030

1. Evaluate Pharma estimated global market value of indication in 2030. PK = pyruvate kinase; SYK = spleen tyrosine kinase; ITP = immune thrombocytopenia; siRNA = small interfering RNA; TMPRSS6i = transmembrane protease serine 6 inhibitor. HV = healthy volunteer; PAH = phenylalanine hydroxylase; PKU = phenylketonuria.

Q&A session

Appendix

Appendix – REIGNITE mitapivat confirmatory trial in sickle cell disease



Primary endpoint: Transfusion-free from week 4 to 52

Key secondary endpoints: Number of RBC units transfused from week 4 through week 52, average change from baseline in Hb, indirect bilirubin and LDH from week 24 to 52

Exploratory endpoint: annualized rate of SCPC

Confirmatory trial design based on RISE UP Phase 3 trial

- 98% power for primary endpoint and ≥90% power for key secondary endpoints
- Assumed effect size based on the RISE UP Phase 3 trial

Enriched population with measurable clinical burden

- Recent transfusion history (≥1 in prior year)
- Signs/symptoms of hemolysis
- Average Hb 5.5-10.5 g/dL during screening
- Excludes chronic transfusion

Well-designed confirmatory trial with assumptions anchored in prior randomized Phase 3 data

BID = twice daily; RBC = red blood cell; LDH = lactate dehydrogenase; SCPC = sickle cell pain crisis; Hb = hemoglobin.

Appendix – Cevidoplenib Phase 2 secondary endpoints show clinically meaningful platelet responses aligned with registrational standards

		Cevidoplenib (400 mg BID) N=22	Placebo N=12	2-sided p-value
Primary endpoint	PLT ≥30,000/μL and AVG PLT ≥2x baseline at any visit during 12-week treatment period[1]	63.6%	33.3%	0.151
Key secondary endpoint	≥2 consecutive PLT ≥30,000/μL	50.0%	8.3%	0.015[2]
Other secondary endpoints	PLT ≥50,000/μL in ≥4 of the last 6 visits	27.3%	0%	-
	PLT ≥50,000/μL in ≥4 of the last 8 visits	36.4%	0%	-

Primary endpoint limited assessment of benefit
- Does not measure durability of PLT response
- Response dependent on baseline PLT level
- Not aligned with ITP regulatory precedent

Secondary endpoints evaluated durability
- Repeated PLT responses over time
- Endpoints aligned with ITP regulatory precedent

Phase 2 results show clinically meaningful, durable platelet responses – planned Phase 3 initiation in H1 2028, following completion of CMC development work

1. Primary endpoint – patient platelet response defined as platelet count ≥30,000/μL and doubling the baseline (average of two previous counts) at any visit during treatment and without use of rescue medication.
2. Nominal p-value. AVG = Average; PLT = platelet; ITP = immune thrombocytopenia; CMC = chemistry manufacturing and controls.