

Agios Expands into ITP with Global License for Cevidoplenib

Conference call for investors and analysts

01 June 2026



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Cevidoplenib in-licensing conference call agenda

- 1 Introduction** Morgan Sanford, VP Investor Relations
- 2 CEO Opening Remarks** Brian Goff, Chief Executive Officer
- 3 ITP Background and Treatment Landscape** Sarah Gheuens, MD, PhD, Chief Medical Officer, Head of R&D
- 4 Financial considerations** Cecilia Jones, Chief Financial Officer
- 5 CEO Closing Remarks** Brian Goff, Chief Executive Officer
- 6 Q&A Session**

Agios – path to sustainable leadership in rare hematology



Lead and innovate in hemolytic anemias

Mitapivat foundational PKa – driving near and mid-term value

Tebapivat next-gen PKa with potential for broader reach in sickle cell disease



Disciplined expansion in adjacent hematology

Diseases with clear biologic drivers, chronic treatment models, overlapping prescriber base

Cevidoplenib – next-gen SYKi – ITP
AG-236 – TMPRSS6i – PV

Building from hemolytic anemia leadership into adjacent rare hematology diseases

Leading rare hematology capabilities developed from foundation in hemolytic anemias

Proven foundation in hemolytic anemias with mitapivat

- ✓ Established proof of concept in ultra-rare disease – PK deficiency (PYRUKYND)
- ✓ AQVESME™ strong initial U.S. commercial launch progress in thalassemia
- ✓ Advancing sNDA towards U.S. accelerated approval in sickle cell disease

Leading capabilities to support rare hematology expansion

- 1 Efficient, rare hematology-focused clinical development
- 2 Proven regulatory innovation and execution
- 3 Specialist-focused commercialization in chronic rare hematology

Selective expansion into de-risked ITP opportunity



ITP significant, underserved patient population

- ~90,000 patients diagnosed in the U.S.¹
- ~24,000 patients refractory to ≥1 prior therapy



Shift to immune-modulating mechanism to target biology

- SYK inhibition clinically validated
- Potential to improve durability and tolerability



ITP is clear adjacency to Agios portfolio

- Overlapping prescriber base
- Chronic disease management model
- Meaningful unmet need

Disciplined deal structure – \$25M upfront payment, regulatory and development milestones gated to inflection points; Cevidoplenib in ITP represents up to \$1B PYS in U.S.

1. Deitz AC et al. 2006 Prevalence Estimate of Idiopathic Thrombocytopenic Purpura (ITP) in the United States Blood (ASH Annual Meeting Abstract), 2006; 108(11):3955. ITP = Immune Thrombocytopenia; SYK = spleen tyrosine kinase; PYS = peak year sales.

Substantial unmet need remains for patients with ITP

ITP is a serious blood disorder marked by immune-driven platelet loss and bleeding risk



Autoantibodies attack blood platelets, essential to clotting

Diagnosis of exclusion requiring platelet count $<100,000/\mu\text{L}$



Antibodies mark platelets for destruction by macrophages

Symptoms include bruising, bleeding episodes and fatigue

Estimated ~200,000 patients diagnosed globally with ~90,000¹ diagnosed with ITP in the U.S.

Current ITP therapies leave a clear need for durable, well-tolerated immune-targeted options

1L	Broad immunosuppression (corticosteroids)	Rapid, but not durable; toxicity limits chronic use
2L+	Platelet production (TPO-RAs)	Does not address immune-mediated destruction
3L+	Kinase inhibition (BTKi; first-gen SYKi) / B-cell depletion (anti-CD20)	Inconsistent durability; tolerability constraints

SYK inhibition – optimal mechanism for the treatment of ITP

- Targets Fc-mediated platelet destruction
- Drives durable platelet responses
- Enables chronic use with combinable profile

Next-gen SYK inhibition designed to expand therapeutic index—maintaining efficacy while enhancing tolerability

~24K U.S. patients refractory to ≥ 1 prior therapy – opportunity for durable, mechanism-targeted therapies

Cevidoplenib – optimizing SYK inhibition for sustained efficacy in ITP

	First-generation SYKi	Cevidoplenib
Efficacy	Inconsistent target inhibition associated with variable PLT response	Sustained target inhibition to supports 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 SYKi

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

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

		Cevidoplenib (400mg BID) N = 22	Placebo N = 12	2-sided P-value
Primary endpoint	PLT $\geq 30,000/\mu\text{L}$ AND AVG PLT $\geq 2\times$ baseline at any visit during 12-week treatment period ¹	63.6%	33.3%	0.151
Key secondary endpoint	≥ 2 consecutive PLT $\geq 30,000/\mu\text{L}$	50.0%	8.3%	0.015 ²
Other secondary endpoints	PLT $\geq 50,000/\mu\text{L}$ in ≥ 4 of the last 6 visits	27.3%	0%	-
	PLT $\geq 50,000/\mu\text{L}$ 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 $\geq 30,000/\mu\text{L}$ 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.

Disciplined deal structure with significant PYS opportunity

\$25M upfront payment

Limited near-term impact to OpEx

Up to \$140M milestones¹

Development and regulatory milestones tied to value inflection points

Tiered Royalties

High single-digit to mid-teen upon commercialization preserves upside

Capital deployed over time, aligned to key value inflection points

Still anticipate FY 2026 OpEx will be approximately flat vs FY 2025, excluding \$25M upfront payment

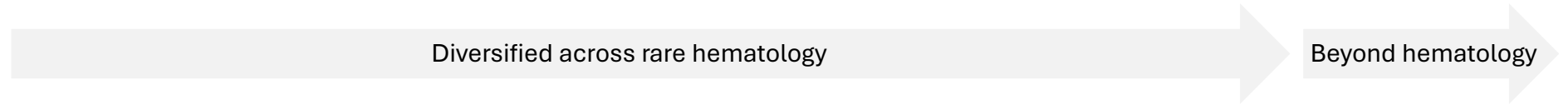
1. \$140M in development and regulatory milestones for up to three indications in U.S. and Europe. Does not include additional commercial milestones. OpEx = operating expenses.

Disciplined expansion into high-value ITP with differentiated next-generation SYKi Cevidoplenib

- ▶▶ **Disciplined expansion into high-value, strategically aligned ITP market**
 - Clear adjacency with overlapping prescribers and chronic treatment model
- ▶▶ **Next-gen SYK inhibitor, potential to improve tolerability and durability**
 - Optimized SYK selectivity; established safety through Phase 2
- ▶▶ **Phase 2 data show clinically meaningful, durable platelet responses**
 - Clinical activity on endpoints aligned with ITP registrational trials

Phase 3 planned H1 2028, enabling continued focus on delivering 2026 priorities

Cevidoplenib strengthens Agios focused rare hematology portfolio



	PK deficiency	Thalassemia	Sickle Cell Disease	ITP	Polycythemia Vera	PKU
	PYRUKYND	AQVESME	Mitapivat/ Tebapivat	Cevidoplenib	AG-236	AG-181
Mechanism of action	PK activator	PK activator	PK activator	SYK inhibitor	TMPRSS6 siRNA	PAH inhibitor
Development stage	Approved	Approved	Registration/ Phase 2	Completed Phase 2	Phase 1 HV	Phase 1b in PKU patients
Global indication market value in 2030 ¹	Ultra-rare	\$1B+	\$3B+	\$3B+	\$4.5B+	\$1B+

Agios' expanding pipeline focused on rare disease markets valued at >\$10B in 2030¹

1. Evaluate Pharma estimated global market size in 2030.

Q&A Session – Cevidoplenib in-licensing conference call



Brian Goff
Chief Executive Officer



Sarah Gheuens, MD, PhD
Chief Medical Officer, Head of
Research & Development



Cecilia Jones
Chief Financial Officer



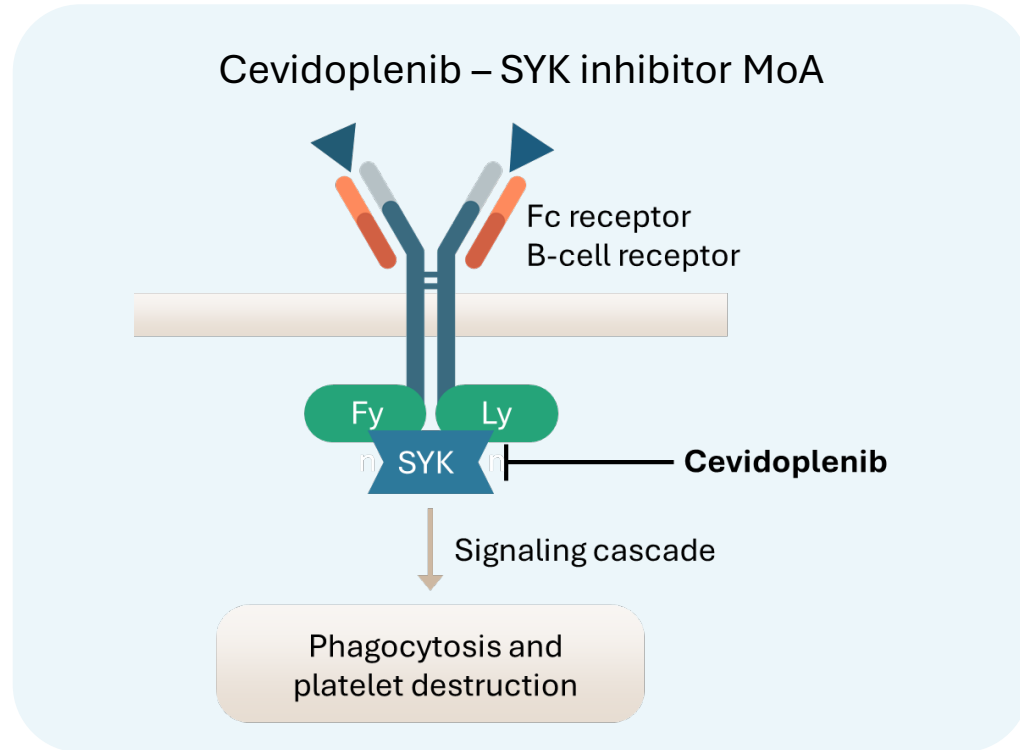
Tsveta Milanova
Chief Commercial Officer



**Krishnan Viswanadhan,
Pharm D.**
Chief Strategy & Operations Officer

Appendix

Cevidoplenib – next-generation SYK inhibitor in ITP

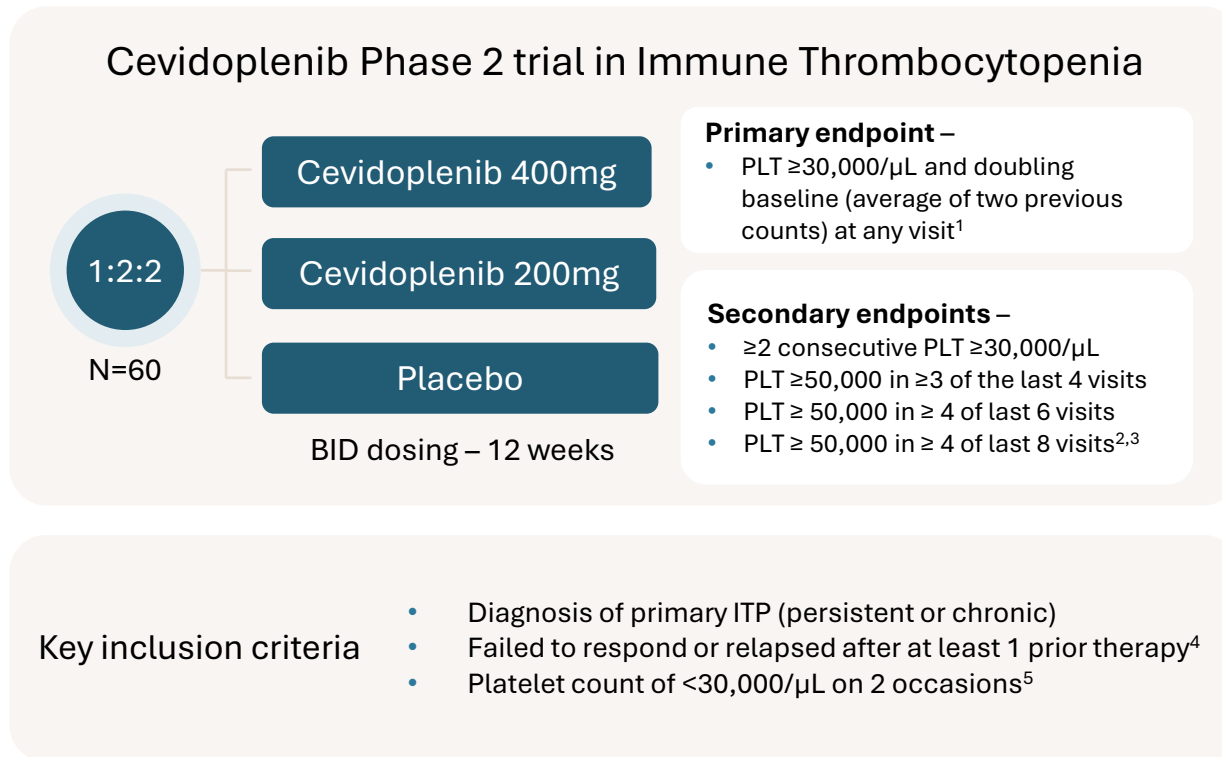


Cevidoplenib is a highly selective SYK inhibitor

In ITP, autoantibody-coated platelets engage Fc γ receptors on macrophages, activating SYK—the key intracellular switch that drives phagocytosis and platelet destruction

SYK inhibition directly blocks Fc-mediated platelet destruction – the central driver of ITP

Cevidoplenib – Phase 2 data showed promising efficacy and supports progression to Phase 3 trial



- Cevidoplenib did not achieve statistical significance for the primary endpoint (2-sided p-value was > 0.05)
- Observed responses for Cevidoplenib 400mg BID on key secondary endpoint, nominal p-value < 0.05
 - **50.0%** of patients had ≥ 2 consecutive PLT $\geq 30,000\mu\text{L}$ vs. 8.3% placebo (p = 0.015)
- Overall proportion of patients who achieved other secondary endpoints encouraging (400mg BID)
 - **27.3%** PLT $\geq 50,000\mu\text{L}$ in ≥ 4 of last 6 visits vs 0% placebo
 - **36.4%** PLT $\geq 50,000\mu\text{L}$ in ≥ 4 of last 8 visits vs 0% placebo

1. Primary endpoint – patient platelet response defined as platelet count $\geq 30,000/\mu\text{L}$ and doubling the baseline (average of two previous counts) at any visit during treatment and without use of rescue medication. 2. of the 12 week double-blind period. 3. Other secondary endpoints include ITP bleeding score, SF-36 QoL score and safety measures. 4. 31.7% failed on 1-2 LoT; 68.3% failed on ≥ 3 LoT 5. at least 7 days apart with confirmatory count taken during screening. ITP = Immune Thrombocytopenia; PLT = platelet; BID = twice daily.