



Pharming Group N.V.

First quarter 2026 financial
results

May 7, 2026

NASDAQ: **PHAR** | EURONEXT Amsterdam: **PHARM**



Fabrice Chouraqui
Chief Executive Officer

Introduction



Fabrice Chouraqui
Chief Executive Officer



Leverne Marsh
Chief Commercial Officer

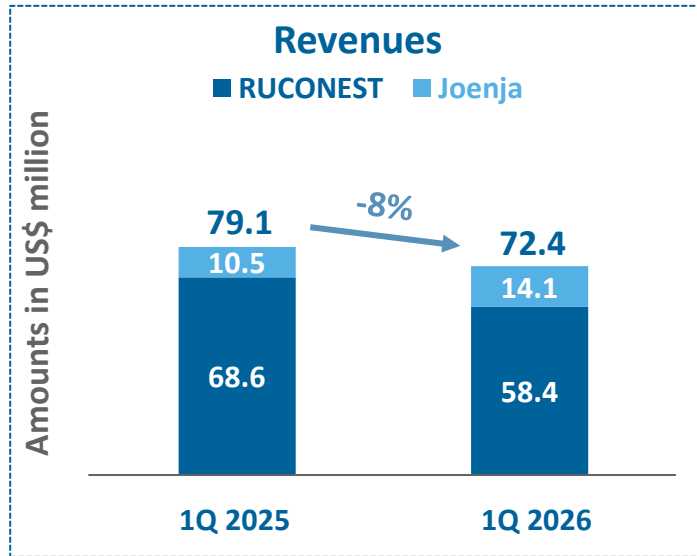


Anurag Relan, MD
Chief Medical Officer



Kenneth Lynard
Chief Financial Officer

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Joenja[®] momentum

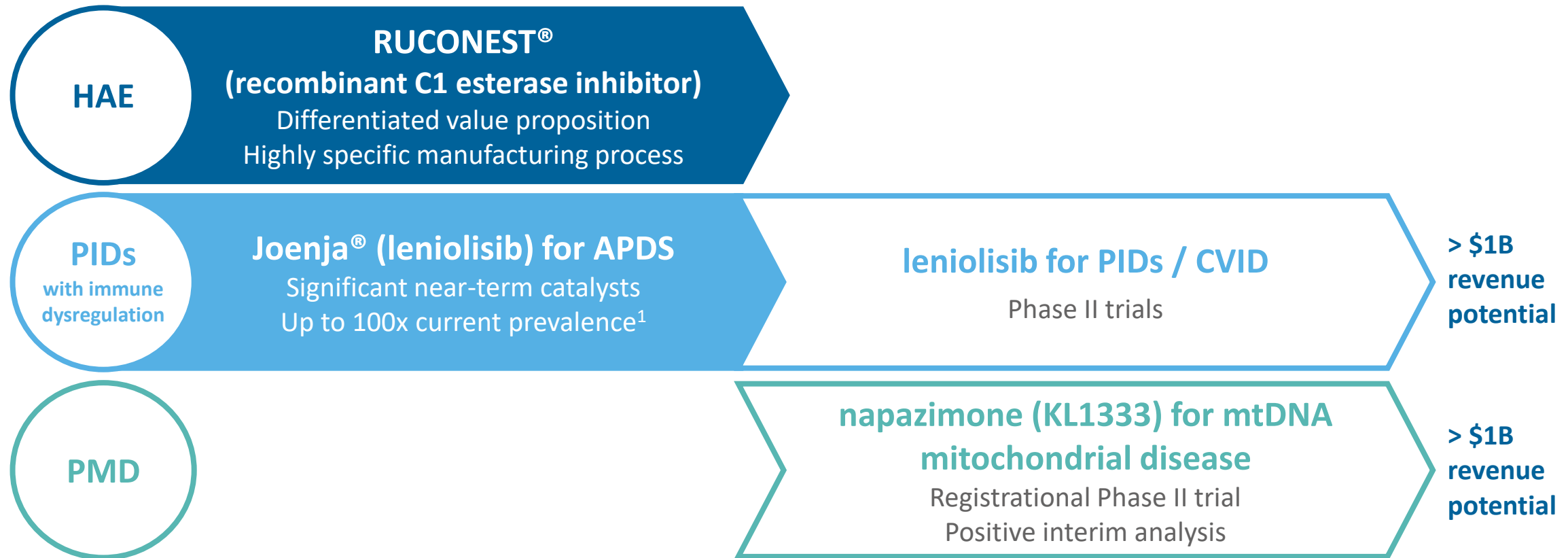
- ✓ 25% increase in U.S. patients
- ✓ Japan approval (4-11s)
- ✓ CHMP positive opinion (EU)
- ✓ Resubmitted PED sNDA (U.S.)

- RUCONEST[®] revenue -15% largely due to anticipated inventory drawdowns and planned non-US markets exit
- Joenja[®] key growth driver – strong momentum in the U.S. and internationally, with 34% revenue growth. On track for U.S. pediatric label expansion and launches in Japan and Europe in 2026
- Disciplined cost management helped sustain positive cash flow from operations in the quarter (+US\$2 million)
- Reaffirmed 2026 total revenue guidance US\$405 - 425 million (8% to 13% growth)

Combination of commercial and pipeline assets poised to deliver strong value creation

Commercial

Pipeline



*These product candidates are under investigation, and their safety and efficacy have not been established. There is no guarantee that these products will receive health authority approval or become commercially available for the uses being investigated

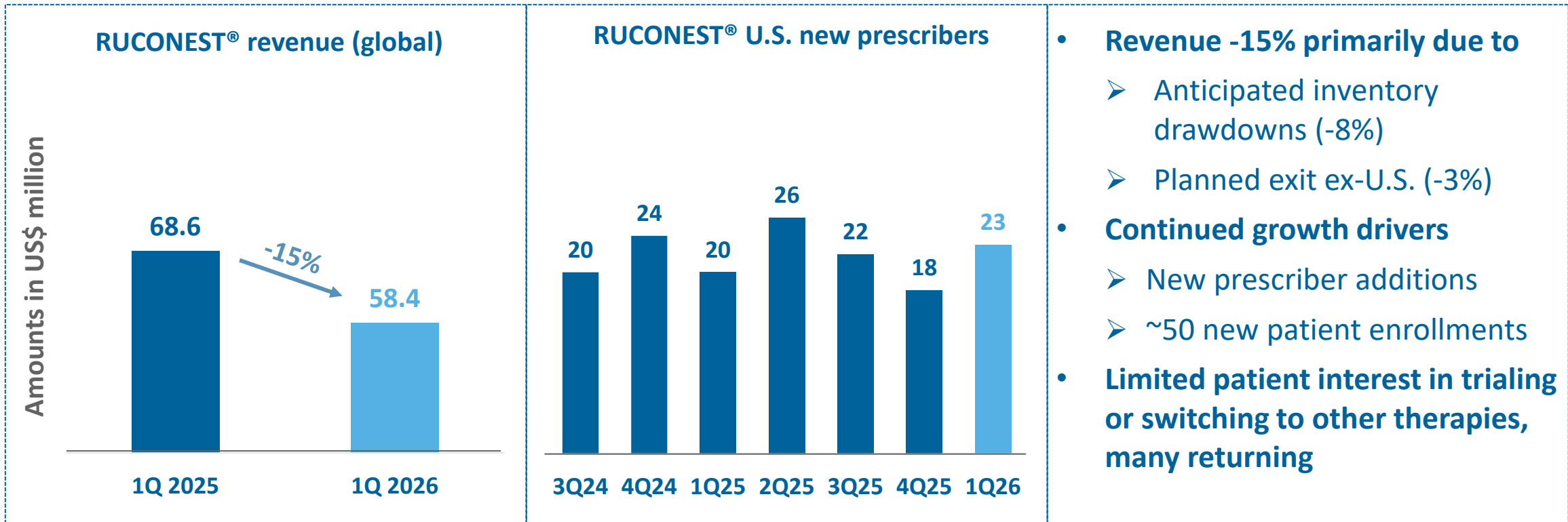
HAE: Hereditary Angioedema, **PIDs:** Primary Immunodeficiencies, **PMD:** Primary Mitochondrial Disease, **CVID:** Common Variable Immunodeficiency

1. Walsh et al., Scalable generation and functional classification of genetic variants in inborn errors of immunity to accelerate clinical diagnosis and treatment, Cell (2025), <https://doi.org/10.1016/j.cell.2025.05.037>



Leverne Marsh
Chief Commercial Officer

Commercial update



RUCONEST® - a cornerstone on-demand treatment for high burden HAE patients

High Burden of HAE

Unpredictable, Potentially Life-Threatening Swelling

Symptoms **progress to maximal severity** over several hours

Up to 44% of some HAE treatments **required more than one dose**

97%

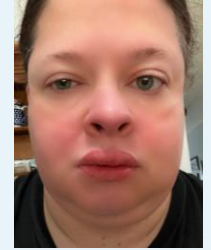
One and Done: 97% of acute attacks needed just one dose of RUCONEST®¹

93%

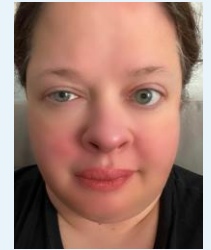
Relief That Lasts: Stopped 93% of attacks for at least 3 days²

85%

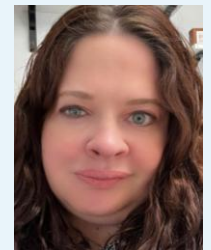
Confident Self-Administration: 85% of patients were confident self-administering³



Time of taking RUCONEST®

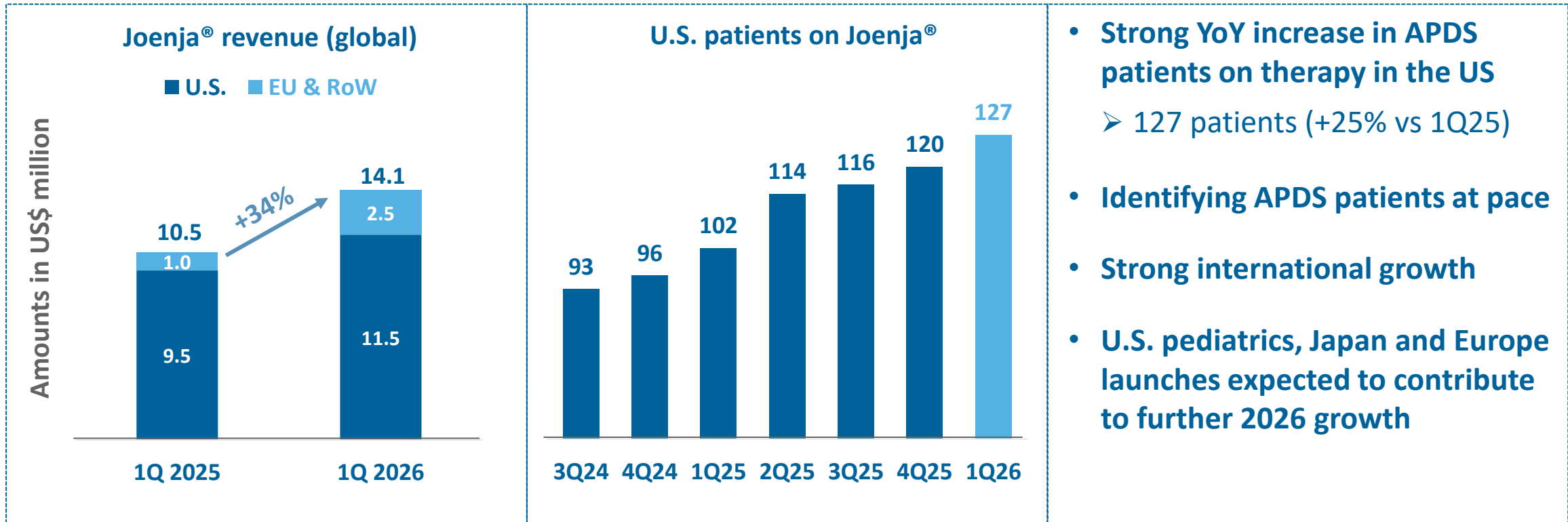


4 hrs after

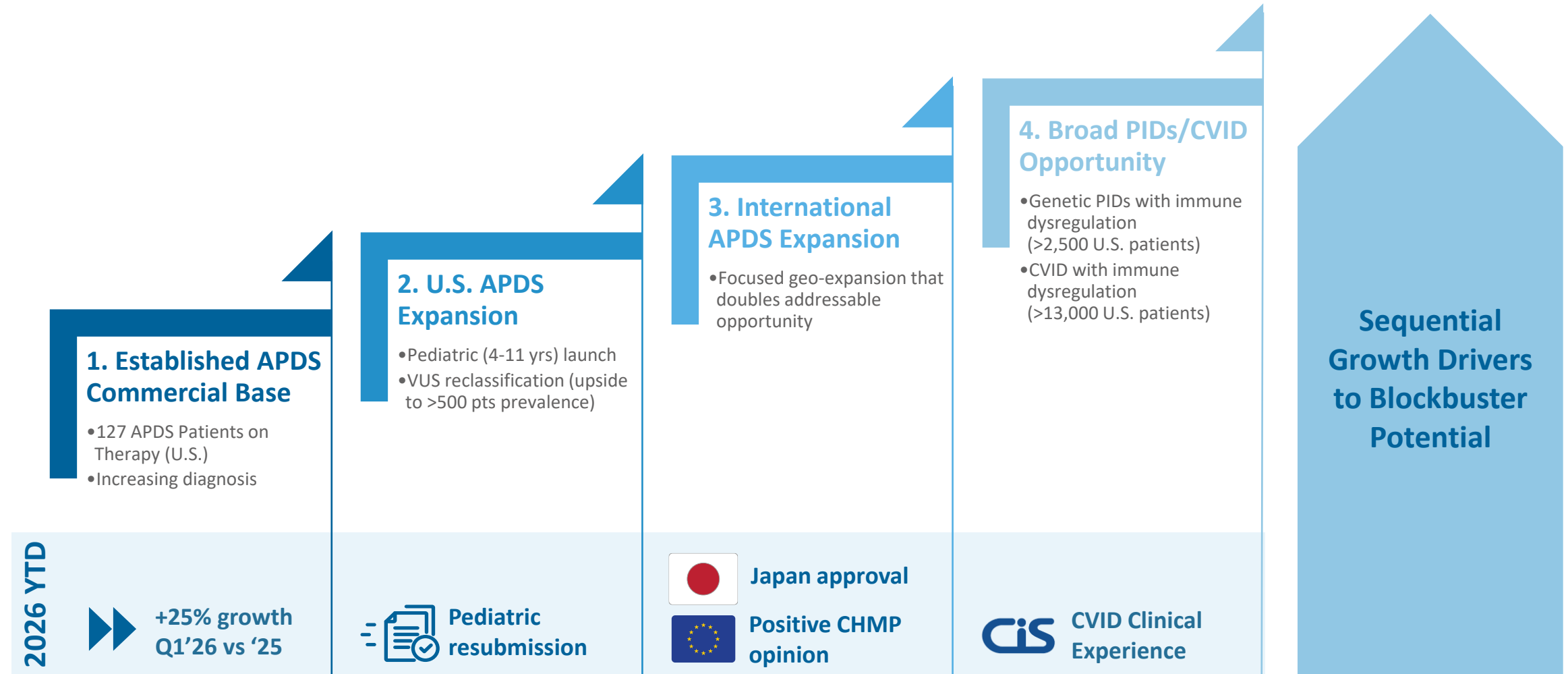


24 hrs after

Overwhelming majority of RUCONEST® patients remain on therapy nine months into oral competitor launch with continued acquisition of new patients



Joenja[®] expansion from APDS foundation into broad blockbuster immunology opportunity



*Based on APDS prevalence est. ~1.5 patients / million in U.S. and Core 8 ex-US markets. Patients identified: 282 patients in the U.S. (187 eligible 12+, 57 eligible 4-11), 1016 globally (as of March 31, 2026).



Anurag Relan, MD
Chief Medical Officer

R&D update

U.S.

Pediatric label expansion – sNDA for patients aged 4-11

CRL received January 30, 2026, with two issues raised by FDA

- Clinical pharmacology
- Analytical batch testing methodology

- Type A meeting held March 26, 2026, including 2 APDS expert physicians
- Alignment on path, as confirmed by meeting minutes
- Resubmitted sNDA for highest doses (decision within 6 months or sooner)
- New sNDA for lowest doses (filing this summer)

CIS presentations highlight leniolisib compassionate use experience in CVID and additional insights in APDS

CIS 2026 Annual Meeting | May 6-9, 2026



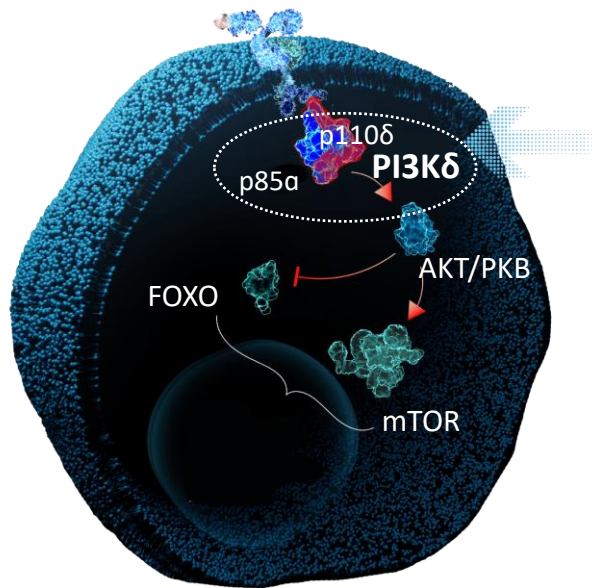
APDS

- *Interim Safety and Efficacy Outcomes of an Open-Label Long-Term Extension Study of Treatment With PI3K δ Inhibitor Leniolisib in Pediatric Patients Aged 4-11 Years With Activated PI3K δ Syndrome (APDS)*
- *Pediatric and Adolescent Activated Phosphoinositide 3-kinase Delta Syndrome (APDS): Demographic and Clinical Findings from the APDS-Characterization and Clinical Outcomes Immunologic Registry (APDS-CHOIR)*
- *Caregiver- and Clinician-Reported Symptoms in Pediatric Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS) Receiving Leniolisib*
- *Symptom Changes in Pediatric Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS) Receiving Leniolisib*
- *Use of Sirolimus and Immunoglobulin Replacement Therapy (IRT) In Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS): A Systematic Literature Review (SLR)*
- *Caregivers of Children with APDS Balance Complex Medical Care, Family, and Work Responsibilities*

CVID/PID

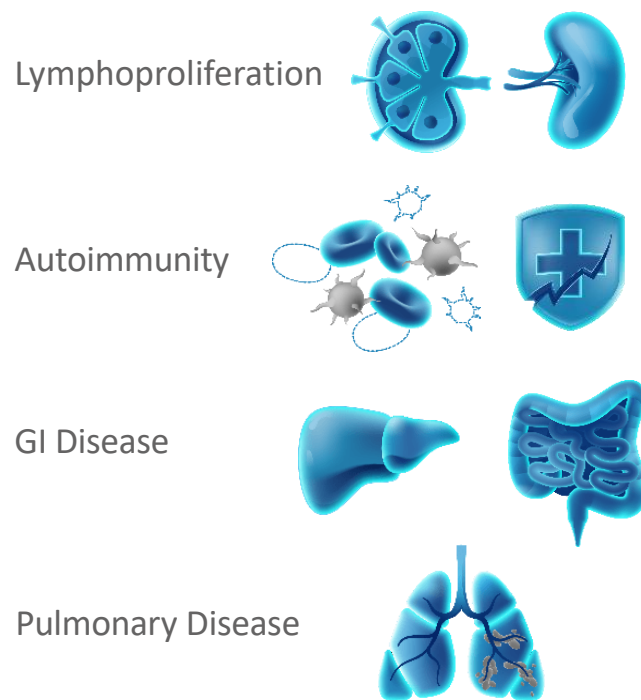
- *Clinical Experience With Use of the PI3K δ Inhibitor Leniolisib to Treat Immune Dysregulation in Patients With CVID and CVID-Like Disorders*
- *A Phase 2 Clinical Study of Leniolisib in Primary Immunodeficiencies With Enhanced PI3K Pathway Signaling: Study Design & Subject Baseline Characteristics*

PI3K δ is a master regulator of the immune system



- ↑ Cell trafficking
- ↑ Cell Growth
- ↑ Cell proliferation
- ↑ Cell Differentiation
- ↑ Apoptosis inhibition/survival

Immune dysregulation pathology



Shared pathology under the influence of PI3K δ

APDS
>500 U.S. patients

Genetic PIDs with immune dysregulation linked to PI3K δ
>2,500 U.S. patients

CVID with immune dysregulation
>13,000 U.S. patients

Leniolisib in PIDs/CVID

Fully enrolled Phase II studies, readouts 2H 2026

Two Phase II, dose range-finding studies designed in similar manner:

- **CVID with immune dysregulation:** N=20
Single arm, open-label, multi-center study
- **Genetic PIDs with immune dysregulation:**
N=12, Single arm, open-label, single center study at NIH

10mg

30mg

70mg

Patients from both studies able to rollover into single 3 year open-label extension (OLE) study

Objectives

Assess safety and tolerability of leniolisib; confirm dosing strategy

Endpoints: AEs; PK/PD

Estimate clinical efficacy of leniolisib for immune dysregulation (lymphoproliferation and autoimmunity)

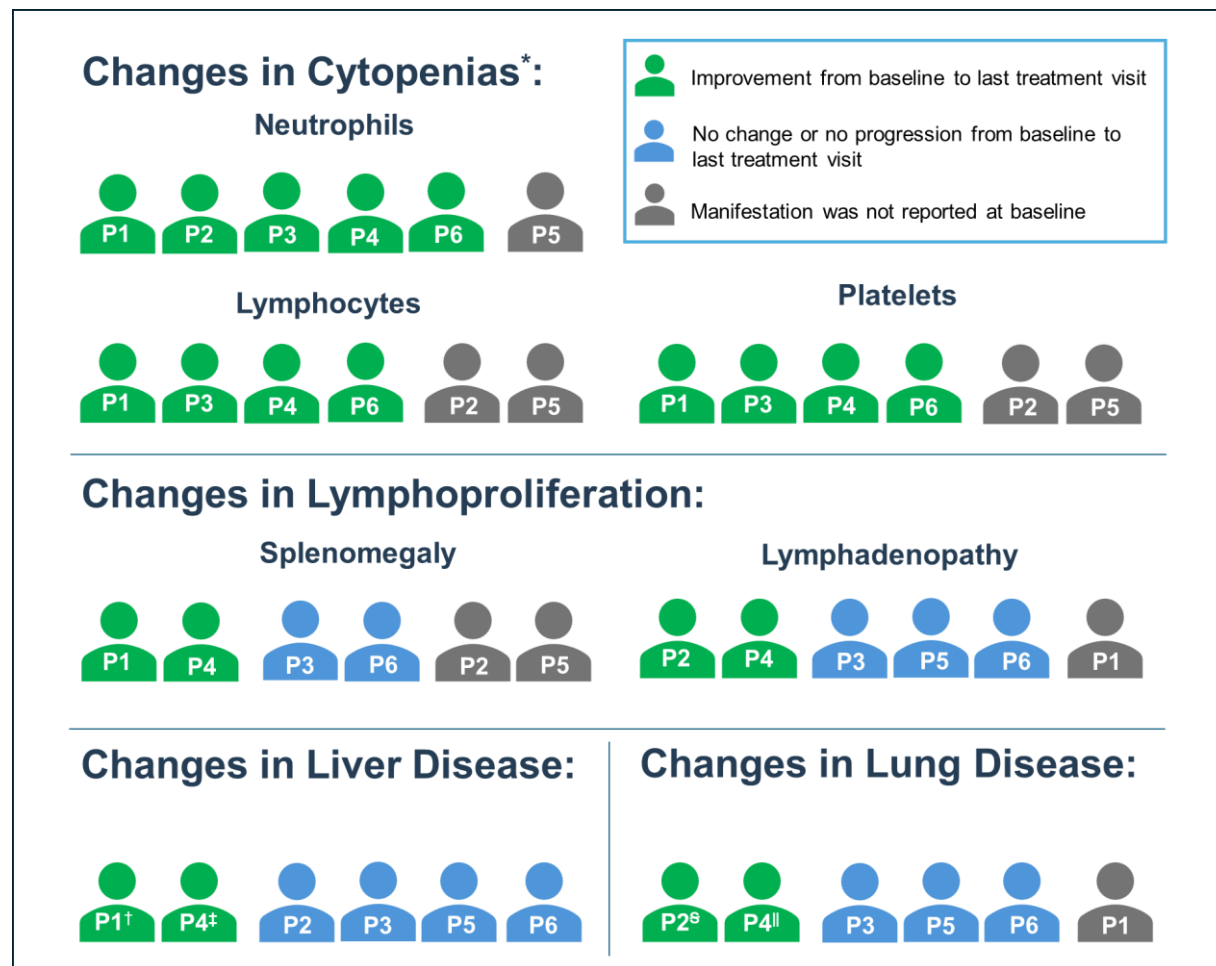
Endpoints:

- Lymph node and spleen size
- Blood cell counts
- End-organ disease measures (lung, liver)
- Patient/clinician reported outcomes*
- Biomarker assessments (CXCL13, B-cell subsets, IFN γ)

Leniolisib experience in CVID patients with immune dysregulation

Presented at CIS 2026 Annual Meeting

- Six CVID/CVID-like patients with immune dysregulation received leniolisib via expanded access for a median 1.4 yrs (0.5–2.5 yrs)
- Clinicians reported improvement across all manifestations shown, with no progression; immune profiles also improved (reduced transitional & CD21lo B cells)
- Adverse events were generally manageable and consistent with the disease severity



Information summarized here is clinician-reported on an individual patient basis in the absence of any prospectively defined data collection strategy

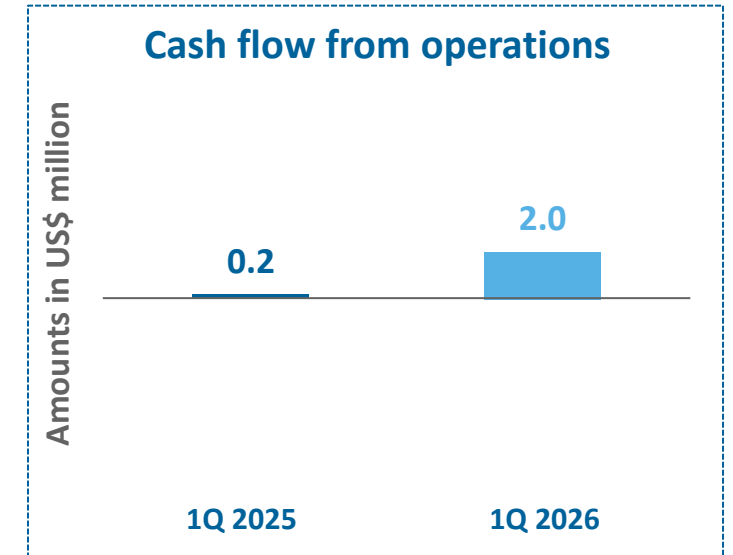
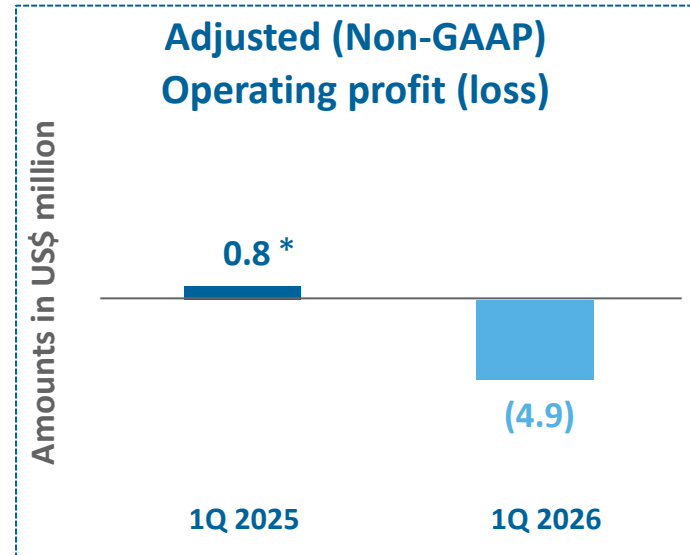
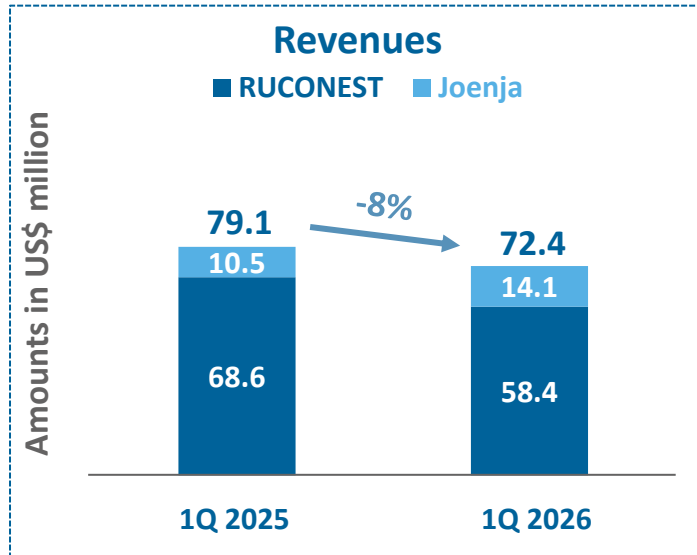
* Increase in leukocytes was reported in P4.
 † Mild improvement observed via magnetic resonance elastography.
 ‡ Improvement based on stabilized transaminitis.
 § Pulmonary nodules resolved and dyspnea improved.
 || Improvement based on findings from imaging, lung volume & diffusion capacity.



Kenneth Lynard
Chief Financial Officer

**Financial results
and outlook**

Financial highlights: 1Q 2026 vs 1Q 2025



- ◆ Strong Joenja[®] growth, total revenues of US\$72.4M, -8% on RUCONEST[®] inventory drawdowns and non-U.S. exit.
- ◆ Positive operating cash flow of US\$2.0M, reflecting continued strong cost management and financial discipline.
- ◆ Cash and marketable securities US\$171.8M, down US\$9.3M vs FY25 due to -US\$12.3M for DSP lease termination.

* Adjusted operating profit for 1Q 2025 excludes US\$7.8 million of non-recurring Abliva acquisition-related expenses.

◆ Revenue and operating expenses (in constant currency):

	FY 2026 Guidance	Notes
Total Revenues	US\$405 - 425 million	<ul style="list-style-type: none">• 8 - 13% growth, with quarterly fluctuations
Operating Expenses	US\$330 - 335 million	<ul style="list-style-type: none">• +US\$60M R&D to advance pipeline• -US\$9M G&A structural cost reductions <i>(as announced in October 2025)</i>

- ◆ Significant and accelerating Joenja[®] growth, and continued RUCONEST[®] growth.
- ◆ Strong financial discipline, and prioritized investments to drive near- and long-term value creation.
- ◆ Available cash and future cash flows expected to cover current pipeline and pre-launch costs.



Fabrice Chouraqui
Chief Executive Officer

Conclusion

Q1 Financial Performance

- ◆ Joenja[®] commercial and regulatory momentum
- ◆ RUCONEST[®] revenue variability
- ◆ Positive net cash flow from operations
- ◆ Reaffirmed \$405-425M (+8-13%) 2026 revenue guidance

Strategic growth priorities

- ◆ Unlock Joenja[®] APDS growth catalysts
- ◆ Reinforce Ruconest positioning for hard-to-treat patients
- ◆ Enhanced capital allocation to optimize growth

High value pipeline

- ◆ Joenja[®] (leniolisib) for PIDs/CVID with immune dysregulation
 - Phase II readouts (2026)
- ◆ Napazimone KL1333 for mtDNA mitochondrial disease
 - Pivotal study readout (2027)

Building a leading rare disease company

- ◆ Proven commercial and development capabilities
- ◆ Growth-oriented leadership team
- ◆ Scalable organization



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Appendix

Statement of profit and loss

Amounts in US\$ '000	1Q 2026	1Q 2025
Revenues	72,447	79,094
Costs of sales	(6,644)	(8,323)
Gross profit	65,803	70,771
Other income	377	383
Research and development	(25,557)	(21,142)
General and administrative	(15,217)	(22,486)
Marketing and sales	(30,317)	(34,570)
Other Operating Costs	(71,091)	(78,198)
Operating profit (loss)	(4,911)	(7,044)
Other finance income	3,237	604
Other finance expenses	(2,695)	(5,098)
Finance result, net	543	(4,494)
Share of net profits (loss) in associates using the equity method	(385)	(250)
Profit (loss) before tax	(4,753)	(11,788)
Income tax credit (expense)	(459)	(3,100)
Profit (loss) for the period	(5,213)	(14,888)
Attributable to:		
Equity holders of the parent	(5,213)	(14,719)
Non-controlling interests	—	(169)
Earnings per share		
Basic, attributable to equity holders of the parent (US\$)	(0.007)	(0.022)
Diluted, attributable to equity holders of the parent (US\$)	(0.007)	(0.022)

Balance sheet – assets

Amounts in US\$ '000	March 31, 2026	December 31, 2025
Non-current assets		
Intangible assets	130,988	135,538
Property, plant and equipment	6,880	7,233
Right-of-use assets	16,218	16,738
Long-term prepayments	93	94
Deferred tax assets	30,668	31,017
Investment accounted for using the equity method	1,533	1,944
Investment in debt instruments designated as at FVTPL	6,580	6,703
Restricted cash	895	1,227
Total non-current assets	193,854	200,495
Current assets		
Inventories	64,577	64,902
Trade and other receivables	60,034	54,704
Restricted cash	725	761
Marketable securities	117,796	33,796
Cash and cash equivalents	52,379	145,305
Total current assets	295,511	299,469
Total assets	489,365	499,963

Equity		
Share capital	8,065	8,009
Share premium	518,601	513,257
Other reserves	23,749	28,819
Accumulated deficit	(281,455)	(272,983)
Shareholders' equity	268,960	277,102
Non-controlling interests	—	—
Total equity	268,960	277,102
Non-current liabilities		
Convertible bonds	93,390	92,719
Lease liabilities	14,670	14,351
Total non-current liabilities	108,060	107,070
Current liabilities		
Convertible bonds	5,375	5,336
Provisions	675	1,187
Trade and other payables	103,526	105,899
Lease liabilities	2,770	3,369
Total current liabilities	112,346	115,791
Total equity and liabilities	489,365	499,963

Amounts in \$'000	1Q 2026	1Q 2025
Profit (loss) before tax	(4,753)	(11,788)
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>		
Depreciation, amortization, impairment of non-current assets	3,116	2,582
Equity settled share based payments	3,225	2,576
Loss (gain) on disposal of leases	6	4
Other finance income	(812)	(604)
Other finance expenses	244	5,028
Share of net losses (profits) in associates using the equity method	385	232
Operating cash flows before changes in working capital	1,411	(1,970)
<i>Changes in working capital:</i>		
Inventories	(264)	(1,083)
Trade and other receivables	(4,395)	5,385
Payables and other current liabilities	5,285	(2,857)
Provisions	(512)	—
Restricted cash	331	(26)
Total changes in working capital	445	1,419
Interest received	379	737
Income taxes received (paid)	(284)	46
Net cash flows generated from (used in) operating activities	1,952	232

Capital expenditure for property, plant and equipment	(238)	(282)
Investment intangible assets	—	(6)
Investment in associates using the equity method	—	(411)
Purchases of marketable securities	(102,646)	—
Proceeds from sale of marketable securities	18,124	67,866
Acquisition of a subsidiary, net of cash acquired	—	(57,476)
Net cash flows generated from (used in) investing activities	(84,760)	9,691
Payment of lease liabilities	(12,975)	(715)
Interests on lease liabilities	(125)	(275)
Settlement of share based compensation awards	3,878	241
Acquisition of non-controlling interests	—	(5,970)
Net cash flows generated from (used in) financing activities	(9,222)	(6,719)
Increase (decrease) of cash	(92,031)	3,204
Exchange rate effects	(895)	1,945
Cash and cash equivalents at January 1	145,305	60,093
Total cash and cash equivalents at March 31	52,379	65,242