



Pharming Group N.V.

First quarter 2025 financial
results and business update

May 8, 2025

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



Fabrice Chouraqui
Chief Executive Officer



Stephen Toor
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Anurag Relan, MD
Chief Medical Officer



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Chief Financial Officer

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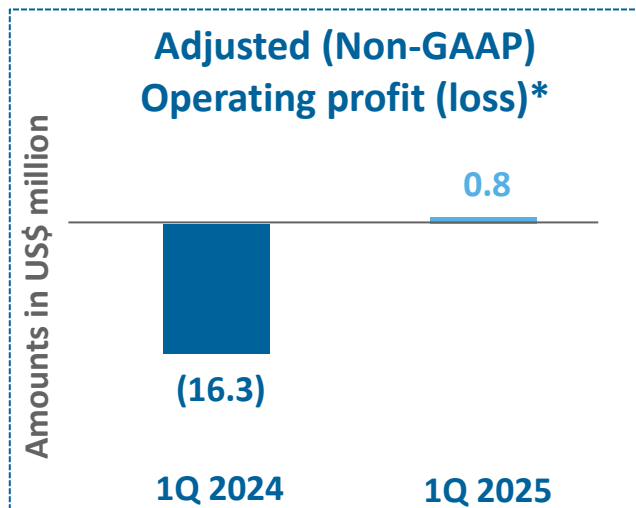
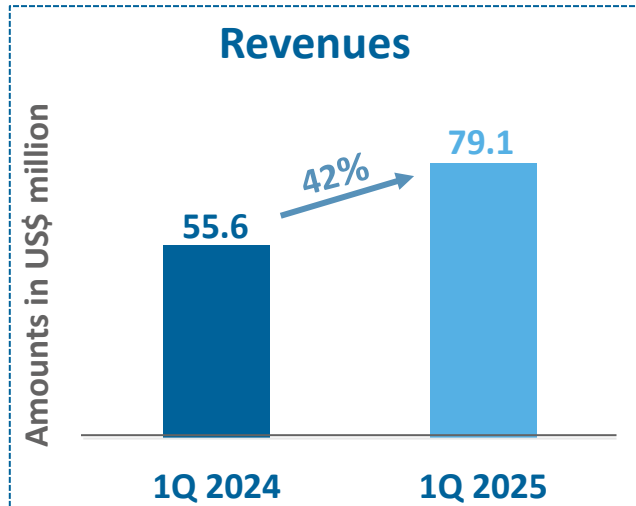


Fabrice Chouraqui
Chief Executive Officer

Introduction

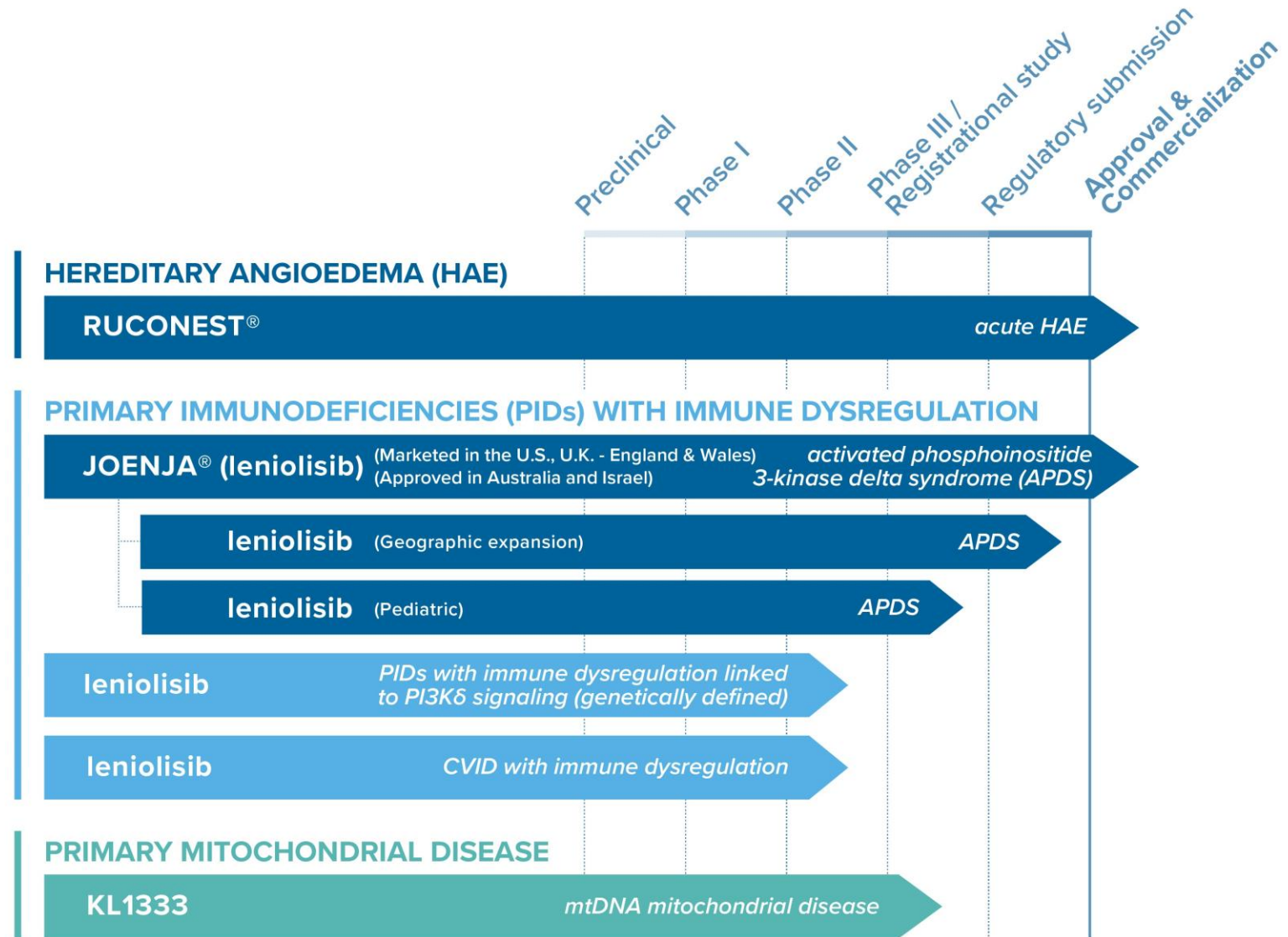
Develop a leading global rare disease company with a diverse portfolio and presence in large markets, leveraging proven and efficient clinical development, supply chain, and commercial infrastructure

Strong first quarter 2025 performance



- Continued strong RUCONEST[®] growth
- Acceleration in Joenja[®] patient uptake
- Raising 2025 revenue guidance to US\$325-340 million
- Achieved operating profitability (adjusted – non-GAAP)

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

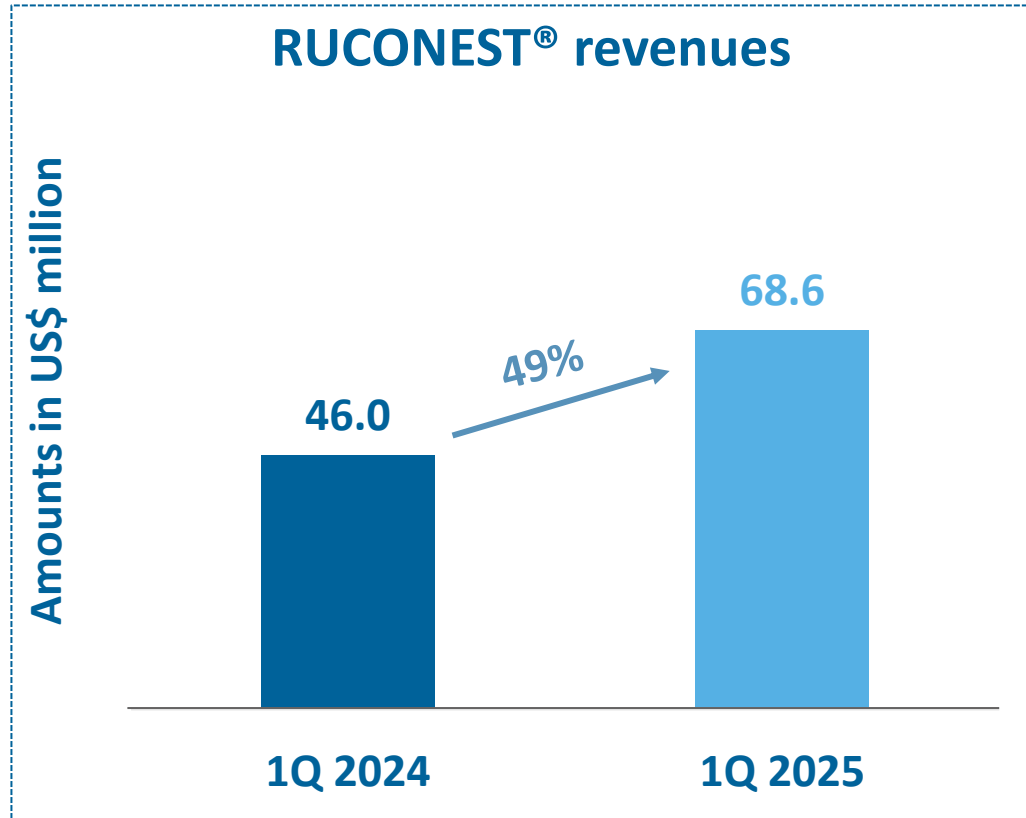




Stephen Toor

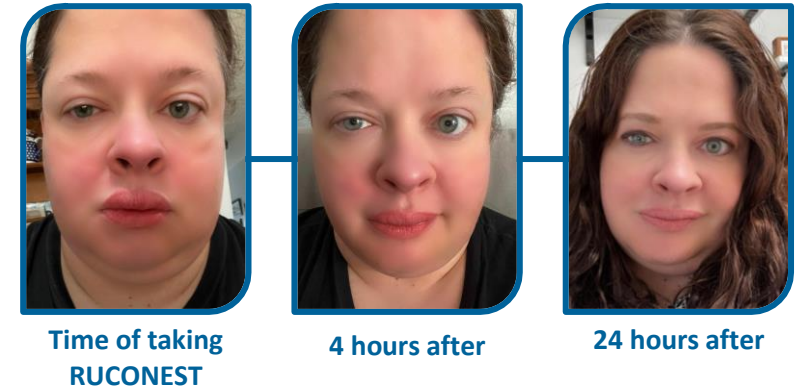
Chief Commercial Officer

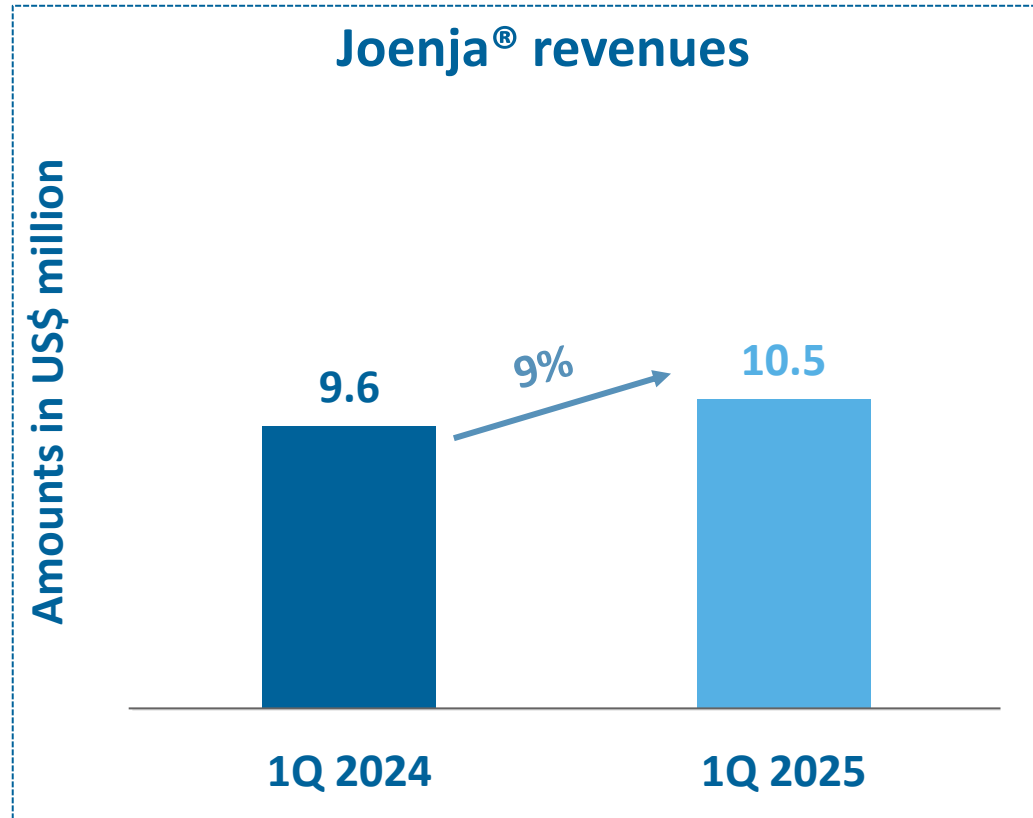
Commercial update



- **Strong U.S. in-market demand**
 - Continuing to add prescribers and patients
 - New patient enrollments remain high (>90)
- **Continued robust U.S. volume growth**
 - Quarterly growth +37%
 - 1Q25 boosted by lower inventory at the SPs in 4Q24 & faster prior authorizations

- ◆ **Type 1, Type 2, and Normal C1-INH HAE patients rely on RUCONEST**
- ◆ **97% patients needed just 1 dose¹**
- ◆ **93% acute attacks stopped for at least 3 days²**
- ◆ **RUCONEST® mostly used by patients experiencing moderate to severe attacks, who attack more frequently**
 - Fail on icatibant and other acute therapies
 - Need to re-dose with other treatments to resolve attacks



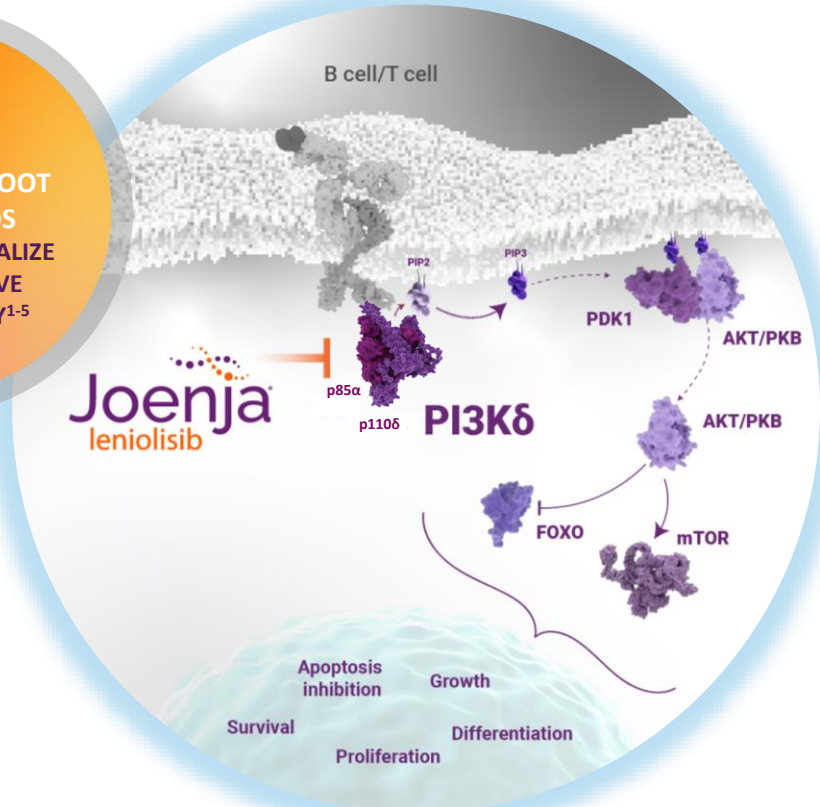


- **Increasing APDS patients on therapy**
 - 102 U.S. patients (+23% vs 1Q24)
 - Acceleration in patient uptake (+6 in 1Q25, most since 2Q24)
- **18% volume growth**
- **Launched in U.K. (England and Wales) in April**
- **Additional 187 APDS patients globally (access programs and clinical studies)**

Joenja[®]: immune modulator that targets the root cause of APDS Helps address immune deficiency and immune dysregulation

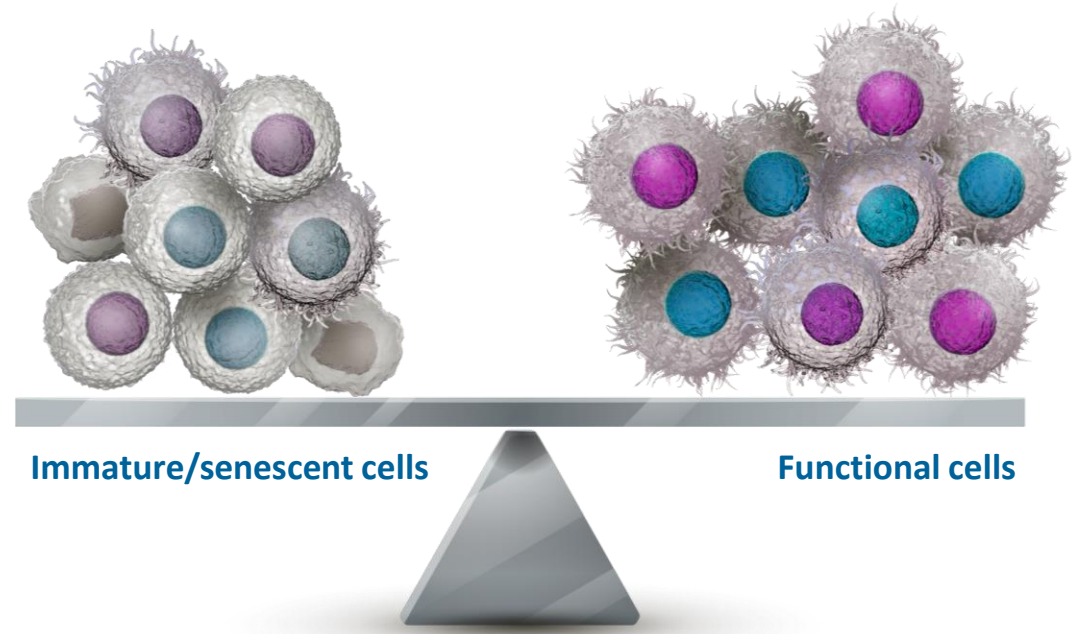


JOENJA WAS DESIGNED TO TARGET THE ROOT CAUSE OF APDS TO HELP NORMALIZE THE HYPERACTIVE PI3Kδ PATHWAY¹⁻⁵



Note: Illustration does not include all steps in the signaling pathway.

Joenja[®] facilitates a balanced PI3Kδ pathway to support proper immune function⁶



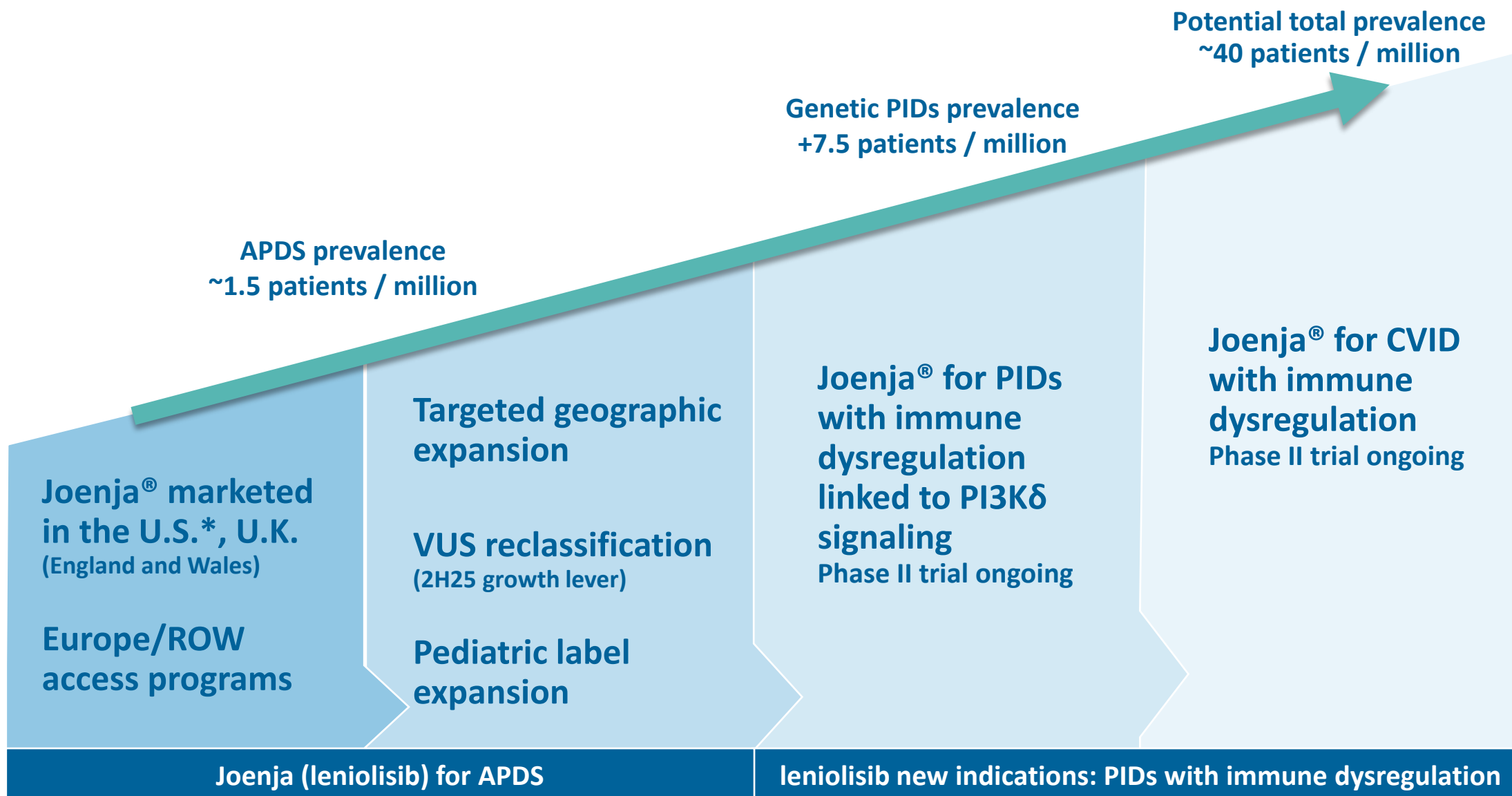
This is a graphical representation of a complex biological process.

AKT/PKB, protein kinase B; FOXO, forkhead box O; mTOR, mammalian target of rapamycin; p85α, the regulatory subunit of the PI3Kδ enzyme; p110δ, the catalytic subunit of the PI3Kδ enzyme.
 1. Fruman DA, et al. *Cell*. 2017;170(4):605-635. 2. Okkenhaug K, Vanhaesebroeck B. *Nat Rev Immunol*. 2003;3(4):317-330. 3. Hoegenauer K, et al. *ACS Med Chem Lett*. 2017;8(9):975-980. 4. Rao VK, et al. *Blood*. 2017;130(21):2307-2316. 5. Rao VK, et al. *Blood*. 2023;141(9):971-983. 6. Nunes-Santos CJ, et al. *J Allergy Clin Immunol*. 2019;143(5):1676-1687.

24-year-old male with APDS whose progress was followed in the Joenja[®] open-label extension study for 6 years

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	<ul style="list-style-type: none">• Experienced fatigue from IRT infusions, anxiety, and difficulty coping with treatment burden• Hospitalized yearly for infections• Frequently prescribed antibiotics	<ul style="list-style-type: none">• Stopped IRT infusions and fatigue got better• No hospitalizations• He had 7 infections, none of which returned• Only doctor he visits regularly is his immunologist
Clinical manifestations	<ul style="list-style-type: none">• Low blood platelet counts• Damaged lung airways• Gastrointestinal issues and migraines	<ul style="list-style-type: none">• Blood platelet count increased• Damaged lung airways did not get worse

Joenja[®] (leniolisib) Expanding the addressable patient population



* 102 patients on paid therapy. U.S. Pricing: 30-day supply \$49,500, Annual cost (WAC) \$594,000



Anurag Relan, MD

Chief Medical Officer

R&D Update



Variants of Uncertain Significance

- ◆ >1300 patients in the US with VUS test result
- ◆ VUSs: insufficient data to determine if variant is disease causing
- ◆ With additional data, up to 20% of VUS results could be reclassified as disease causing for APDS*



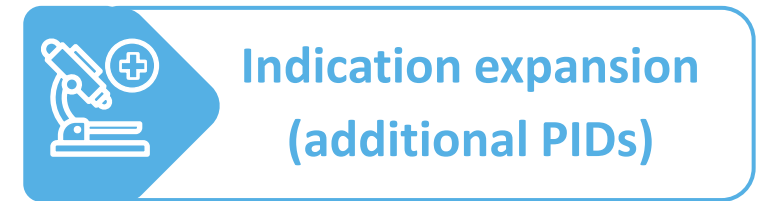
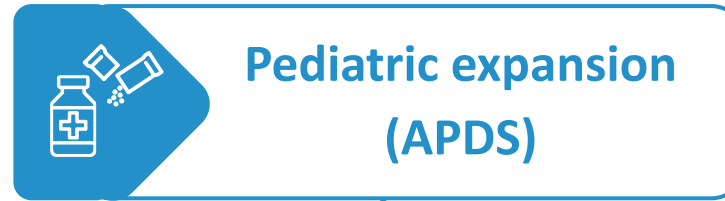
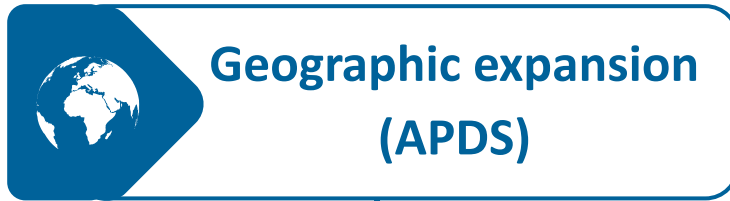
VUS Resolution Steps

- ◆ High throughput screening (MAVE) study completed, identifying many new variants causing PI3K δ hyperactivity
- ◆ Data to be published shortly (submitted and under review)
- ◆ Genetics testing labs will review study data, reclassify variants, and update test reports.
- ◆ Expected positive impact from reclassifications later this year

* As results become available, patients with validated variants could be diagnosed with APDS and be eligible for Joenja[®] treatment.

Joenja[®] development status

Expanding the addressable patient population



* Anticipate regulatory action in 2026 for Canada

Patient Population

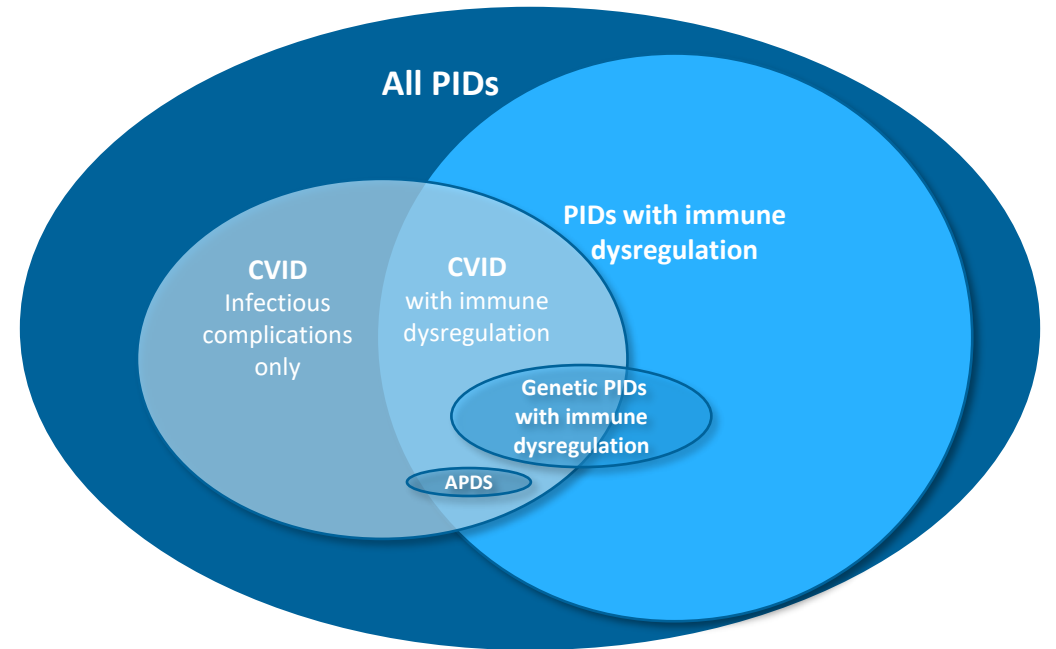
- PID patients with clinical manifestations similar to APDS, including early mortality
- Significant unmet clinical need, no approved therapies
- Large group: Prevalence 5-26x APDS

Rationale

- Critical role of PI3K δ in lymphocyte regulation, driving lymphoproliferation and autoimmunity
- Same therapeutic strategy as in APDS: modulate PI3K δ to address immune dysregulation
- Positive experience in compassionate use patients

Two Phase II studies underway

- Genetically defined PIDs with immune dysregulation¹
- Common variable immunodeficiency (CVID) with immune dysregulation²
- Topline results mid-2026



Not to scale with population sizes

1. PIDs include ALPS-FAS, CTLA4 haploinsufficiency, NFKB1 haploinsufficiency and PTEN deficiency, amongst others. Prevalence 7.5 patients / million

2. Prevalence 39 patients/million

KL1333 for primary mitochondrial disease: Potential first standard of care

KL1333 targets underlying pathology of low NAD⁺ / NADH

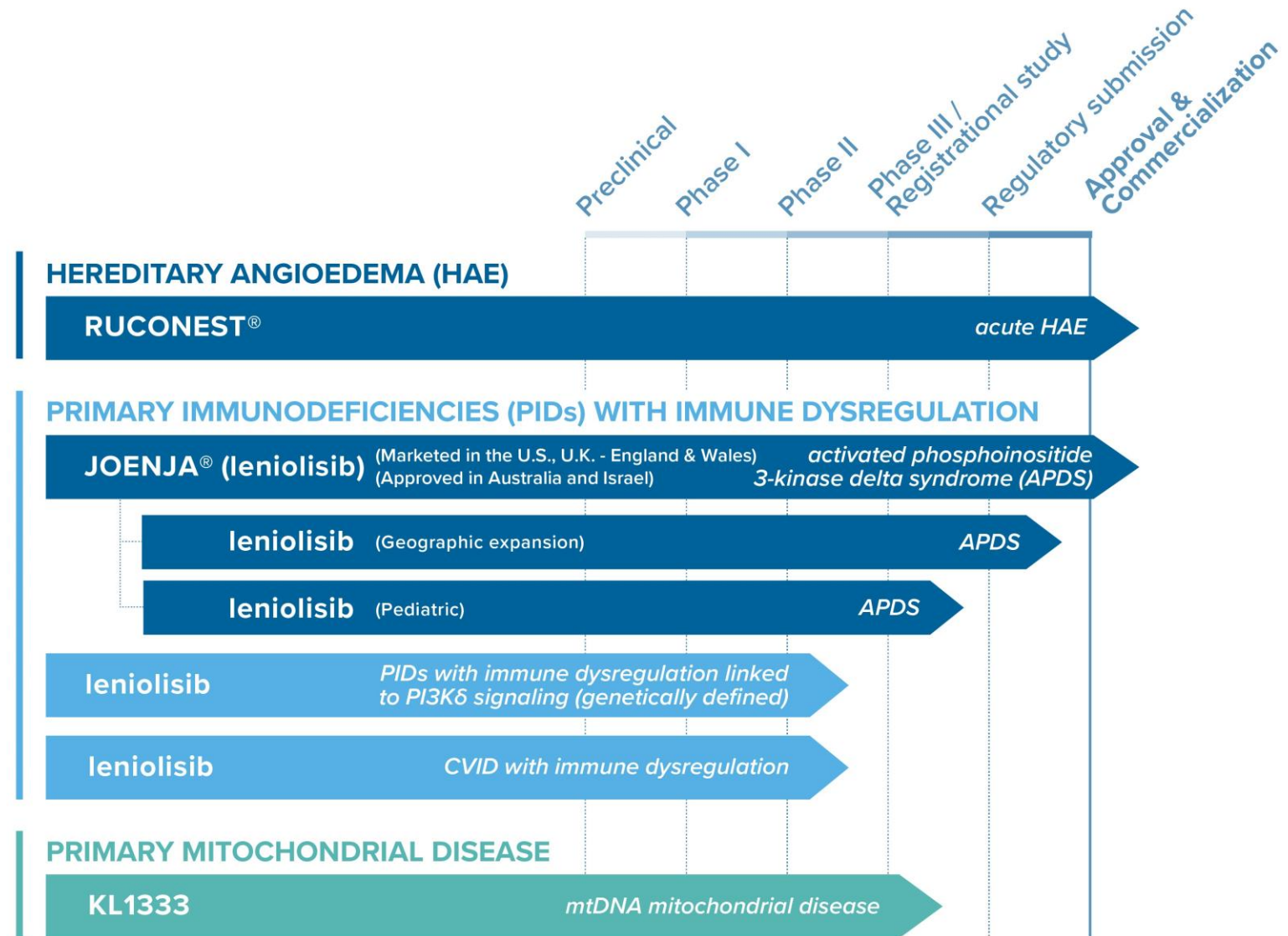
- Normalizes NAD⁺/NADH ratio and mitochondrial function, with evidence from in vitro data, animal models, and in patients treated with KL1333
- >30,000 diagnosed patients with mitochondrial DNA disease potentially addressable by KL1333¹

Registrational clinical study underway

- Clinically-relevant endpoints, supported by FDA
- Positive interim analysis in pivotal study
- Patient recruitment for second wave started April 2025
- Expect readout in 2027 and FDA approval end of 2028

“The fatigue is almost impossible to describe because it seems other-worldly. It feels as though someone has taped cinder blocks to my eyelids some mornings and there is no way to keep them open.”²

1. In US, EU4 and UK. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome.
2. UNITED MITOCHONDRIAL DISEASE FOUNDATION, Voice of the Patient Report, 2019.

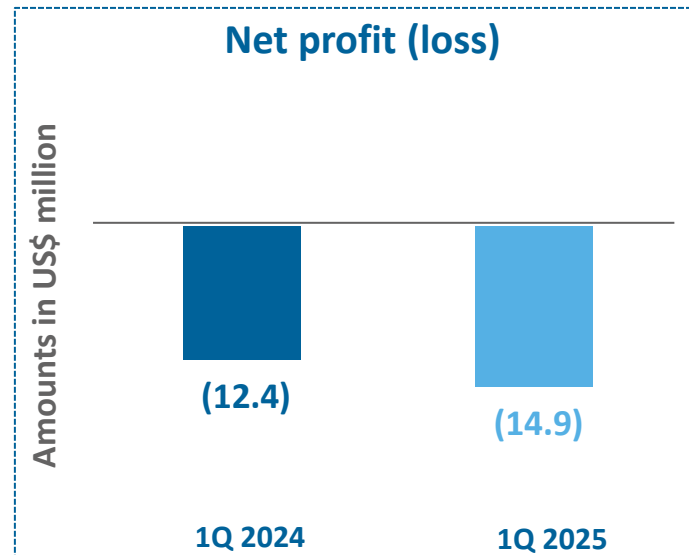
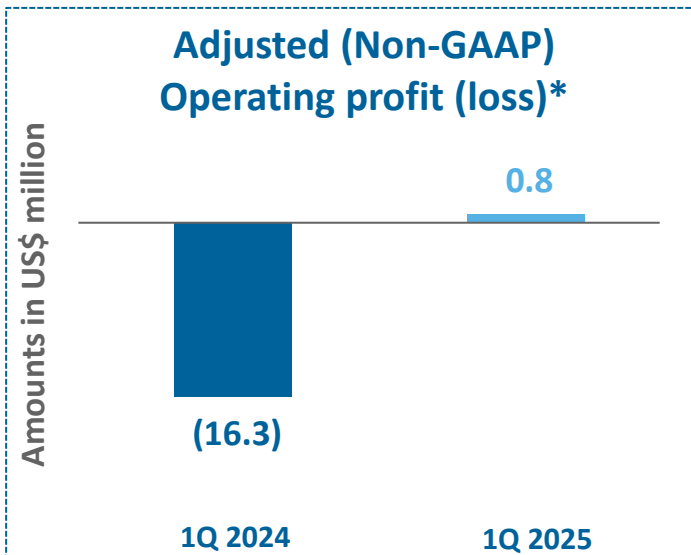
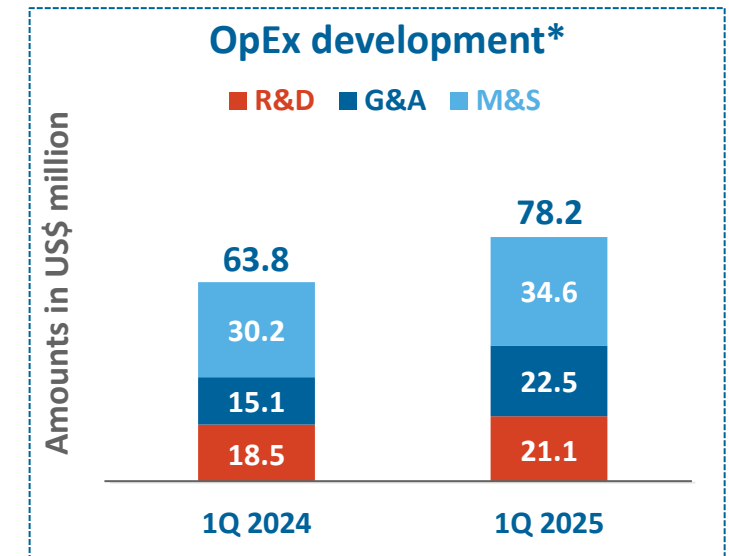
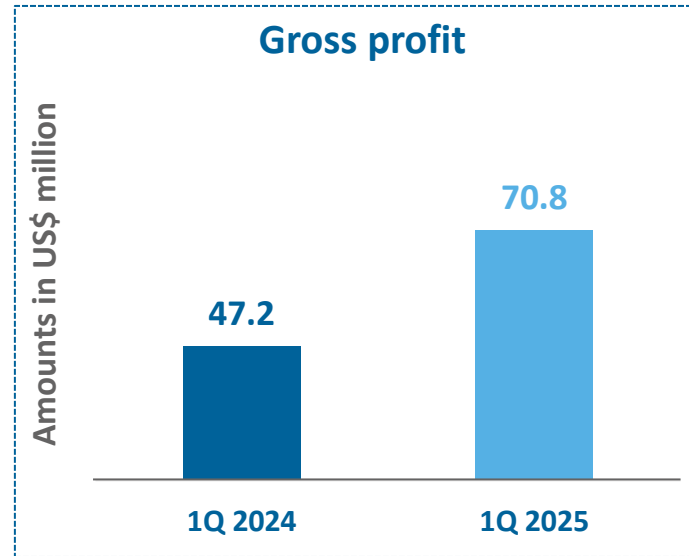
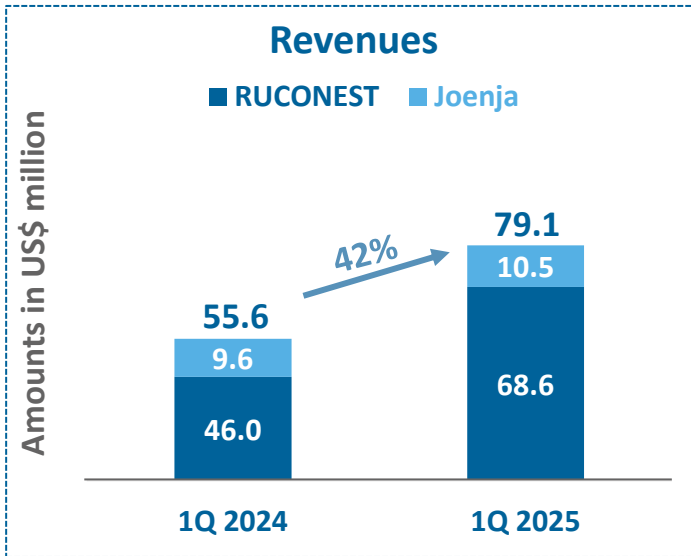




Jeroen Wakkerman
Chief Financial Officer

Financials

Financial highlights: 1Q 2025 vs 1Q 2024



* Adjusted operating profit for 1Q 2025 excludes US\$7.8 million of non-recurring Abliva acquisition-related expenses (US\$5.7 million in G&A, \$2.1 million in R&D).

** Decrease in cash primarily driven by purchases of Abliva shares totaling US\$66.1 million.

Income statement

Amounts in US\$m	1Q 2025	1Q 2024
Revenues	79.1	55.6
Cost of Sales	(8.3)	(8.4)
Gross profit	70.8	47.2
Other income	0.4	0.3
Research and development*	(21.1)	(18.5)
General and administrative*	(22.5)	(15.1)
Marketing and sales	(34.6)	(30.2)
GAAP operating profit (loss)	(7.0)	(16.3)
+ non-recurring Abliva acquisition-related expenses*	7.8	-
Adjusted (Non-GAAP) operating profit (loss)	0.8	(16.3)

Balance sheet

- Cash purchases of Abliva shares totaling US\$66.1 million
- Recognized intangible asset related to KL1333 (US\$63.1 million), goodwill (US\$13.4 million) and deferred tax liabilities (US\$12.8 million)
- Other net identifiable assets were not significant and were recognized at fair value

* US\$7.8 million of non-recurring Abliva acquisition-related expenses in 1Q 2025 (US\$5.7 million in General and administrative and US\$2.1 million in Research and development expenses).

◆ Revenue and operating expenses:

	FY 2025 Guidance	Notes
Total Revenues	US\$325 - 340 million	9 - 14% growth
Operating Expenses (pre-Abliva impact)	Flat vs. FY 2024	
Operating Expenses (Abliva-related)	~US\$30 million	Includes R&D and non-recurring transaction and integration costs

◆ Available cash and future cash flows expected to cover current pipeline investments and pre-launch costs

Strong start to 2025

- 1Q25 revenues up 42%
- Strong RUCONEST[®] growth and increased Joenja[®] patient uptake
- Achieved operating profit (adjusted non-GAAP)
- Raising 2025 revenue guidance to US\$325-340M
- Optimize capital allocation through \$10M annual G&A reduction

High value pipeline

- Joenja[®] (leniolisib) for PIDs with immune dysregulation
 - Genetic PIDs
 - CVID
- KL1333 for mtDNA mitochondrial disease
 - Registrational trial ongoing

Significant catalysts

- Joenja[®] for APDS – VUSs, pediatric label, geo expansion (2025-27)
- Leniolisib for PIDs PhII readouts (2026)
- KL1333 pivotal study readout (2027)



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Appendix

Statement of profit and loss

Amounts in US\$ '000	1Q 2025	1Q 2024
Revenues	79,094	55,586
Costs of sales	(8,323)	(8,386)
Gross profit	70,771	47,200
Other income	383	345
Research and development	(21,142)	(18,521)
General and administrative	(22,486)	(15,087)
Marketing and sales	(34,570)	(30,249)
Other Operating Costs	(78,198)	(63,857)
Operating profit (loss)	(7,044)	(16,312)
Other finance income	604	1,779
Other finance expenses	(5,098)	(1,556)
Finance result, net	(4,494)	223
Share of net profits (loss) in associates using the equity method	(250)	(535)
Profit (loss) before tax	(11,788)	(16,624)
Income tax credit (expense)	(3,100)	4,176
Profit (loss) for the period	(14,888)	(12,448)
Attributable to:		
Equity holders of the parent	(14,719)	(12,448)
Non-controlling interests	(169)	—
Earnings per share		
Basic, attributable to equity holders of the parent (US\$)	(0.022)	(0.019)
Diluted, attributable to equity holders of the parent (US\$)	(0.022)	(0.019)

Amounts in US\$ '000	March 31, 2025	December 31, 2024
Non-current assets		
Intangible assets	138,863	61,039
Property, plant and equipment	7,770	7,752
Right-of-use assets	16,457	16,382
Long-term prepayments	94	90
Deferred tax assets	18,390	30,544
Investment accounted for using the equity method	672	466
Investments in equity instruments designated as at FVTOCI	1,311	—
Investment in debt instruments designated as at FVTPL	3,939	3,767
Restricted cash	1,579	1,505
Total non-current assets	189,075	121,545
Current assets		
Inventories	59,346	55,724
Trade and other receivables	47,487	54,823
Marketable securities	47,180	112,949
Cash and cash equivalents	60,093	54,944
Total current assets	214,106	278,440
Total assets	403,181	399,985

Equity		
Share capital	7,806	7,769
Share premium	490,301	488,990
Other reserves	8,692	(209)
Accumulated deficit	(292,801)	(275,489)
Shareholders' equity	213,998	221,061
Non-controlling interests	1,292	—
Total equity	215,290	221,061
Non-current liabilities		
Convertible bonds	83,849	78,154
Lease liabilities	26,506	26,968
Total non-current liabilities	110,355	105,122
Current liabilities		
Convertible bonds	4,555	4,245
Trade and other payables	68,748	66,611
Lease liabilities	4,233	2,946
Total current liabilities	77,536	73,802
Total equity and liabilities	403,181	399,985

Amounts in \$'000	1Q 2025	1Q 2024
Profit (loss) before tax	(11,788)	(16,624)
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>		
Depreciation, amortization, impairment of non-current assets	2,582	5,921
Equity settled share based payments	2,576	2,427
Loss (gain) on disposal of leases	4	—
Other finance income	(604)	(1,779)
Other finance expenses	5,028	1,556
Share of net losses (profits) in associates using the equity method	232	535
Other	—	783
Operating cash flows before changes in working capital	(1,970)	(7,181)
<i>Changes in working capital:</i>		
Inventories	(1,083)	877
Trade and other receivables	5,385	7,461
Payables and other current liabilities	(2,857)	(9,414)
Restricted cash	(26)	28
Total changes in working capital	1,419	(1,048)
Interest received	737	582
Income taxes received (paid)	46	—
Net cash flows generated from (used in) operating activities	232	(7,647)

Capital expenditure for property, plant and equipment	(282)	(80)
Investment intangible assets	(6)	—
Disposal of investment designated as at FVOCI	—	1,971
Investment in associates using the equity method	(411)	—
Purchases of marketable securities	—	(94,778)
Proceeds from sale of marketable securities	67,866	93,551
Acquisition of a subsidiary, net of cash acquired	(57,476)	—
Net cash flows generated from (used in) investing activities	9,691	664
Payment of lease liabilities	(715)	(1,034)
Interests on lease liabilities	(275)	(290)
Interests on convertible bonds	—	(2,031)
Settlement of share based compensation awards	241	884
Acquisition of non-controlling interests	(5,970)	—
Net cash flows generated from (used in) financing activities	(6,719)	(2,471)
Increase (decrease) of cash	3,204	(9,454)
Exchange rate effects	1,945	(395)
Cash and cash equivalents at January 1	54,944	61,741
Total cash and cash equivalents at March 31	60,093	51,892