



Pharming Group N.V.

Fourth quarter and full year
2024 financial results
and business update

March 13, 2025

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



Fabrice Chouraqui
Chief Executive Officer



Stephen Toor
Chief Commercial Officer



Anurag Relan, MD
Chief Medical Officer



Jeroen Wakkerman
Chief Financial Officer

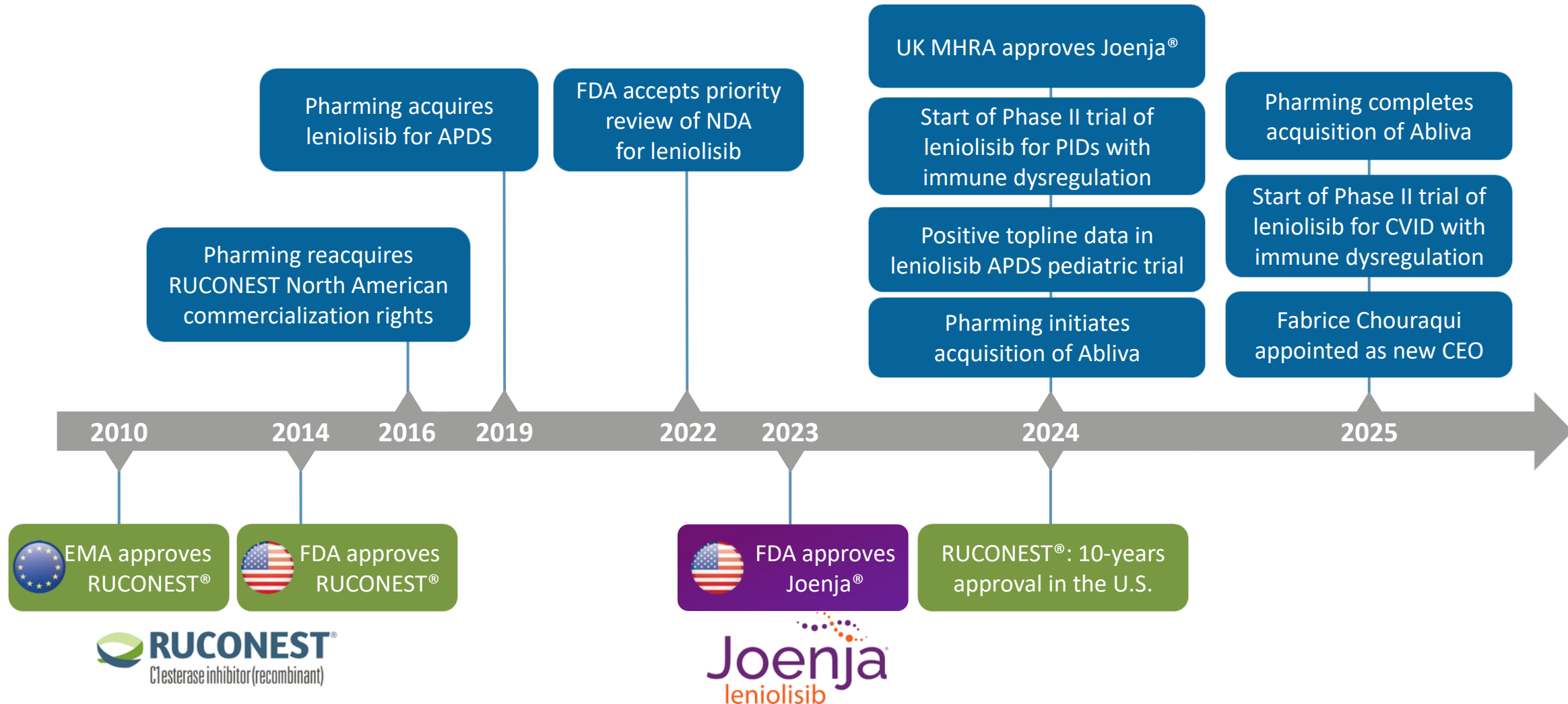
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Fabrice Chouraqui
Chief Executive Officer

Introduction

History of growth and innovation at Pharming



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 fourth quarter and full year 2024 performance reinforces growth foundation for the future



Revenues: FY24: US\$297 million (+21%)
4Q24: US\$93 million (+14%)

Operating profit and positive operating cash flow in 3Q-4Q 2024

EURONEXT AMS: PHARM
Nasdaq: PHAR



Growing commercial portfolio

RUCONEST® for acute HAE attacks

Joenja® (leniolisib) for APDS – U.S. and EU/Rest of World



Reaching more APDS patients with Joenja®

Additional patient finding

Targeted geographic expansion

Pediatrics label expansion



Pipeline addresses large opportunities

Leniolisib new indications (PIDs with immune dysregulation) – 2 Phase IIs

Abliva KL1333 (mtDNA mitochondrial disease) – pivotal study



Stephen Toor
Chief Commercial Officer

Commercial update

RUCONEST®

❖ Strong U.S. in-market demand

U.S. physician prescriber base +11% in FY24

New enrollments up 24% in FY24

❖ Revenue:

4Q24 US\$79.6M (+9%)

FY24 US\$252.2M (+11%)

❖ Well-positioned vs. new acute orals

Joenja®

❖ Increasing APDS patients on therapy

Found >240 in the U.S. and >880 globally

Paid therapy: 96 patients + 5 pending (U.S.)

Additional 188 patients on therapy globally
(access programs and clinical studies)

❖ Revenue:

4Q24 US\$13.1M (+65%)

FY24 US\$45.0M (+147%)

RUCONEST® (rhC1INH):

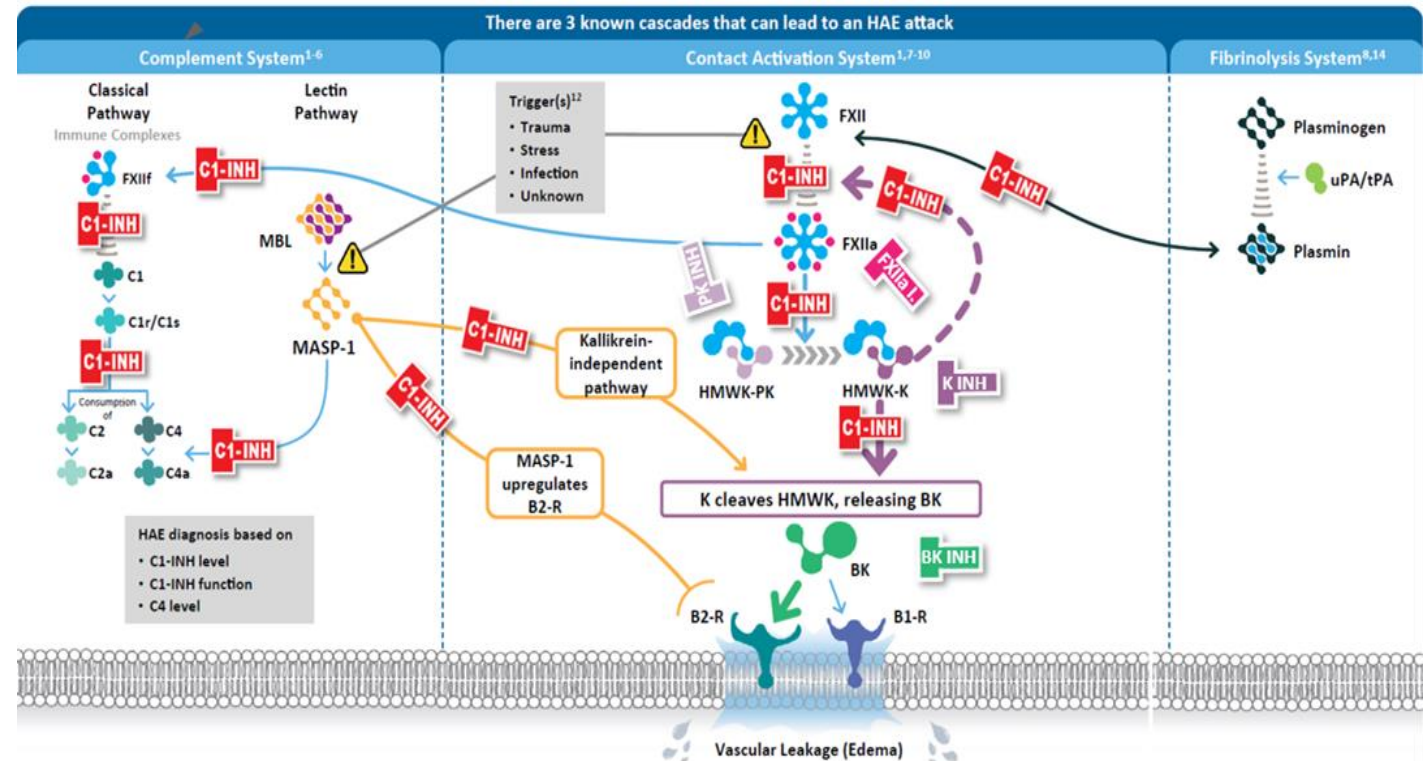
2nd most prescribed therapy for acute HAE attacks in the US



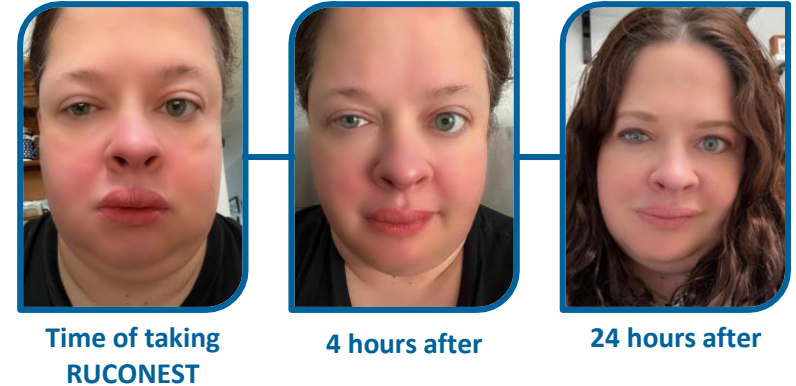
Only recombinant treatment that targets the root cause of HAE by replacing C1-INH

Only recombinant treatment that acts at multiple points in the cascades leading to HAE attacks

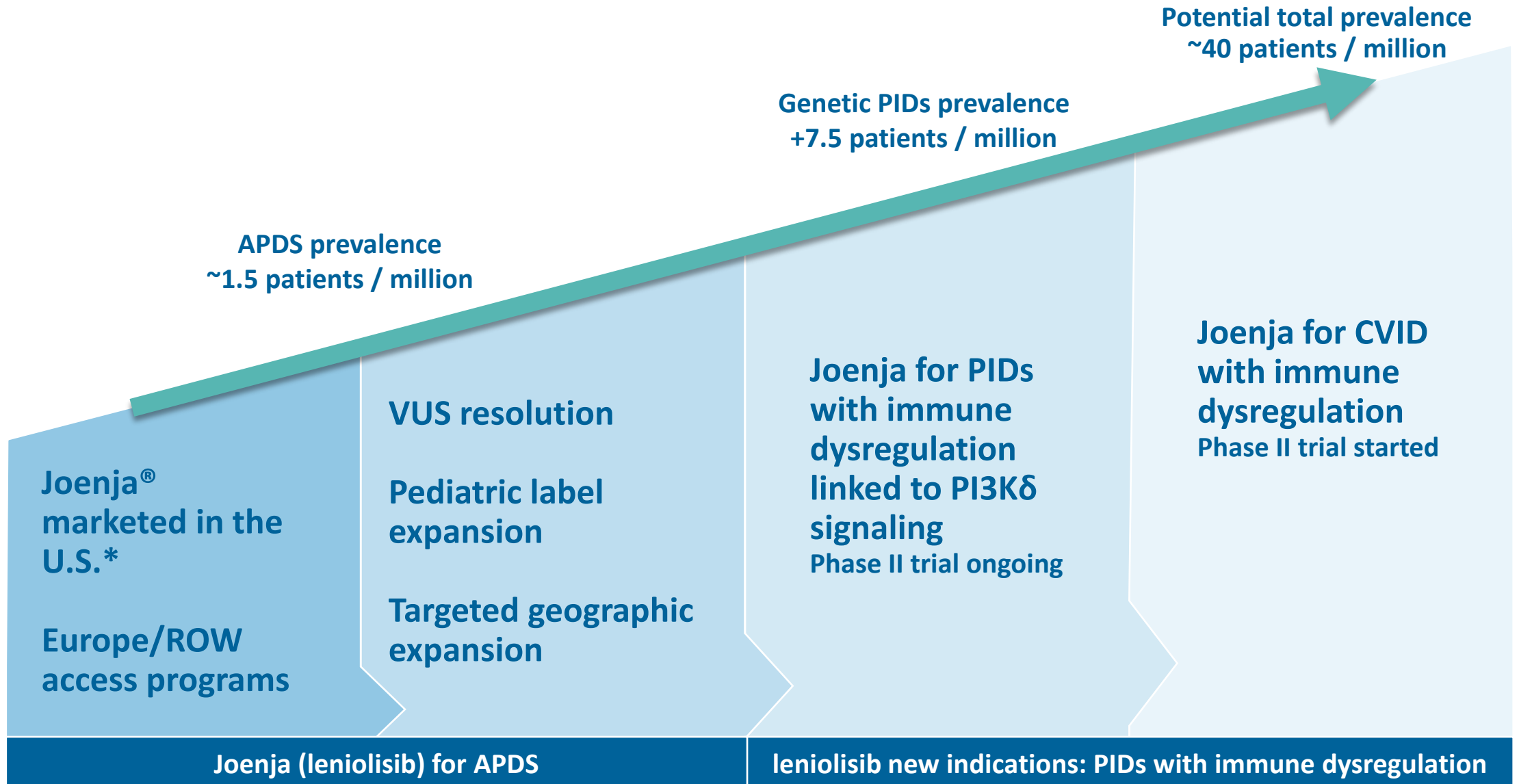
97% patients needed just 1 dose¹
93% acute attacks stopped for at least 3 days²



- ❖ Type 1, Type 2, and Normal C1-INH HAE patients rely on RUCONEST
- ❖ RUCONEST patients experience moderate to severe attacks, and attack more frequently
- ❖ RUCONEST patients switching from other acute treatments
 - Fail on icatibant and other acute therapies
 - Need to re-dose to resolve attacks



Joenja[®] (leniolisib) – Reaching more APDS patients and expanding the addressable patient population



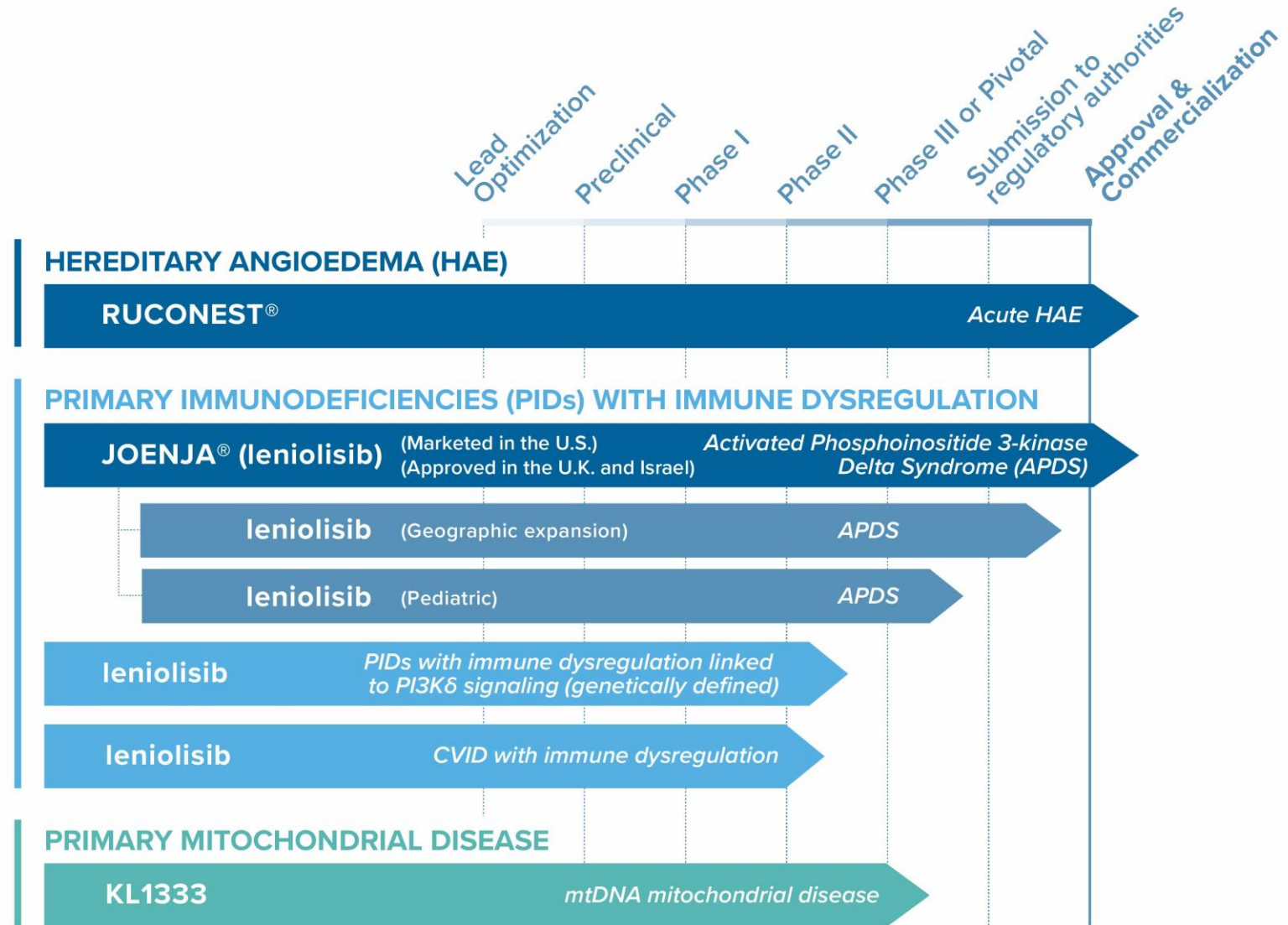
* 96 patients on paid therapy + 5 pending. U.S. Pricing: 30-day supply \$49,500, Annual cost (WAC) \$594,000



Anurag Relan, MD

Chief Medical Officer

R&D Update





Variants of Uncertain Significance

- ❖ VUSs: insufficient data to determine if variant is disease causing
- ❖ >1200 patients in the U.S.
- ❖ VUSs may be reclassified as APDS with additional evidence*



VUS study results

- ❖ High throughput screening (MAVE) study, completed in December, identified novel variants leading to PI3K δ hyperactivity
- ❖ Genetics testing labs to review study data, reclassify variants and update test reports
- ❖ Additional APDS patients to be identified over the course of 2025

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

Pediatric

Phase III trial for children 4-11 years old with APDS

Positive topline data announced December 2024

- ◆ 21 patients enrolled in U.S., Europe, and Japan
- ◆ Both co-primary endpoints show improvement consistent with the RCT in adolescents and adults
- ◆ Benefits seen across the four tested dose levels
- ◆ No deaths/discontinuations due to AEs. No new safety findings
- ◆ Data to be presented at CIS conference in May
- ◆ Regulatory filings beginning with the U.S. in second half 2025

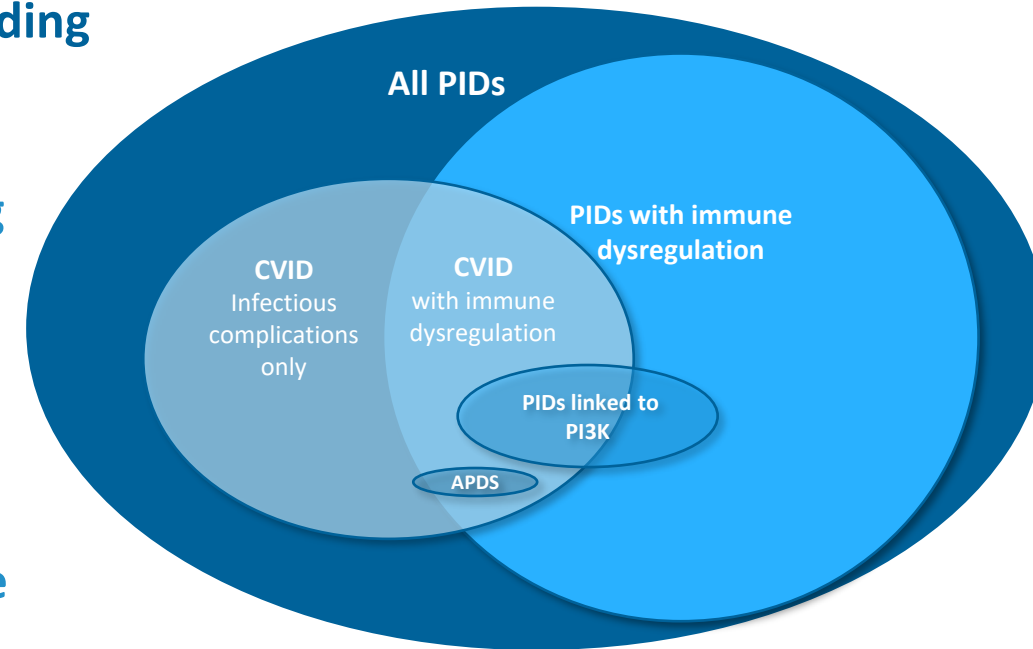
PI3K δ activity drives immune dysregulation in PIDs, providing strong rationale for development of leniolisib

PIDs with immune dysregulation linked to PI3K δ signaling

- Multiple genetically identifiable PIDs¹
- Clinical manifestations, disease onset, and severity similar to APDS
- No approved therapies
- Phase II study started October 2024²
- FDA Fast Track designation

Common variable immunodeficiency (CVID) with immune dysregulation

- Similar clinical phenotype to APDS
- Identified independently of genetics
- Positive regulatory feedback on large unmet medical need and rationale
- Phase II study started; first patient expected to be dosed late-March



Not to scale with population sizes


Prevalence estimates

APDS	1.5/million
PIDs linked to PI3K	7.5/million
CVID with immune dysregulation	39/million ³

1. PIDs include ALPS-FAS, CTLA4 haploinsufficiency, NFKB1 haploinsufficiency and PTEN deficiency, amongst others
2. Single arm, open-label, dose range-finding study
3. CVID prevalence excludes APDS but includes most of the PIDs linked to PI3K indication patient population

Joenja[®] development status

Expanding the addressable patient population



Geographic expansion (APDS)

Europe – CHMP review extended to January 2026
Single outstanding CMC request
Positive clinical benefit and safety concluded

U.K. – Marketing authorization received in 2024
U.K. under evaluation by NICE

CAN, AUS submissions under regulatory review
Canada decision in 2026*
Australia approval in 2025*

Japan clinical study: Patient enrollment complete, positive interim analysis
PMDA filing mid-2025


**Other country regulatory approvals/filings
Access Programs**



Pediatric expansion (APDS)

4 to 11 years – Positive topline data
Filing to begin 2H 2025

1 to 6 years – Enrollment continuing



Indication expansion (additional PIDs)

PIDs with immune dysregulation linked to PI3K δ signaling
Phase II trial ongoing

CVID with immune dysregulation
Started Phase II trial

* Anticipate regulatory action in 2025 for Australia and in 2026 for Canada



Primary mitochondrial diseases – rare disorders impairing mitochondrial energy production

- Severe fatigue, myopathy (muscle weakness), and reduced life expectancy
- Poor quality of life (e.g., loss of job, social isolation, depression)



KL1333 positioned to become first standard of care in mitochondrial DNA (mtDNA) disease

- Novel mechanism of action addresses the underlying disorder
- >30,000 diagnosed patients*



Pivotal study ongoing with positive interim analysis confirming FDA-agreed primary endpoints

- Patient recruitment for second wave of pivotal FALCON clinical trial to start as soon as possible
- Read-out anticipated in 2027 with potential FDA approval by end of 2028



Significant unmet medical need and no approved therapies

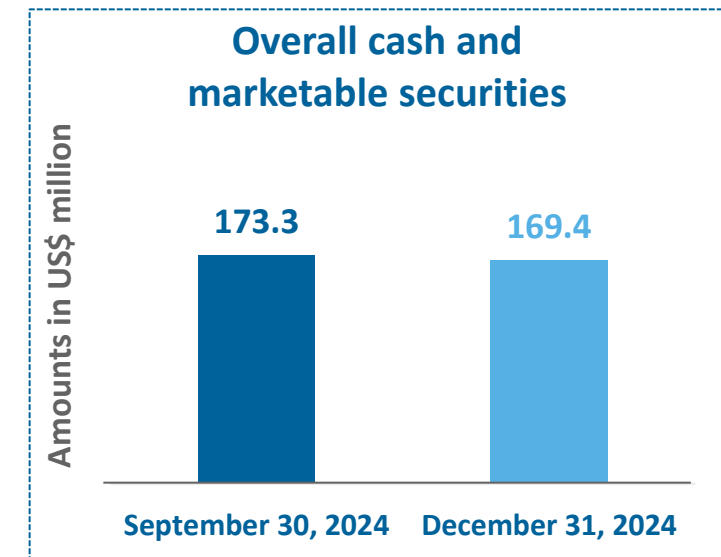
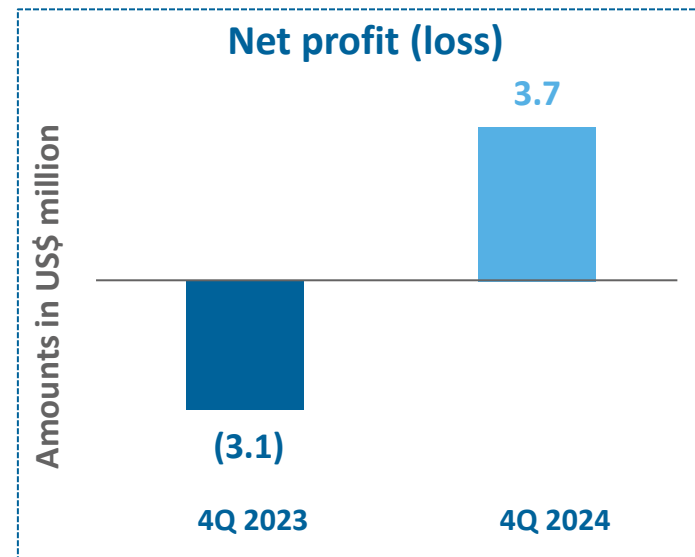
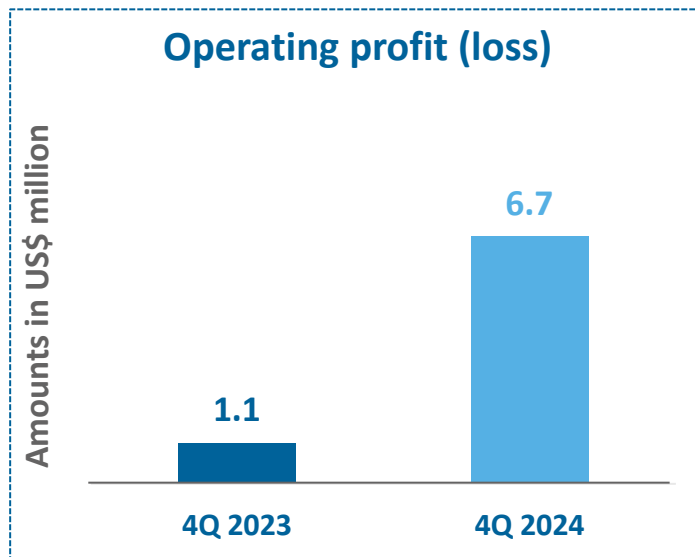
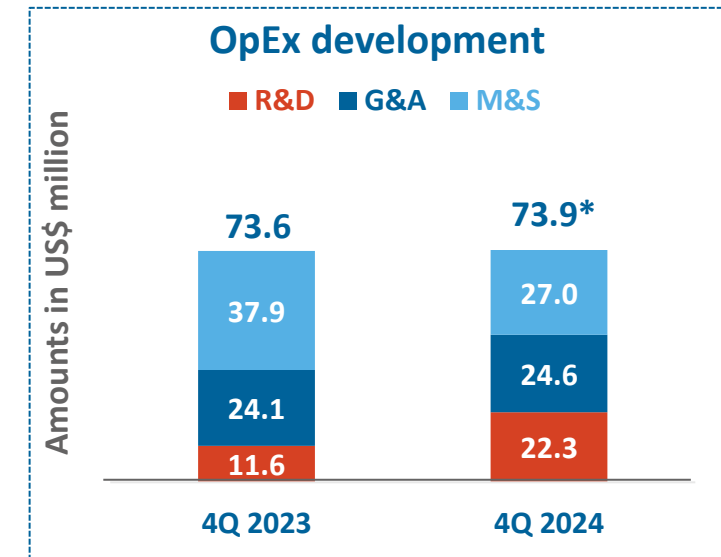
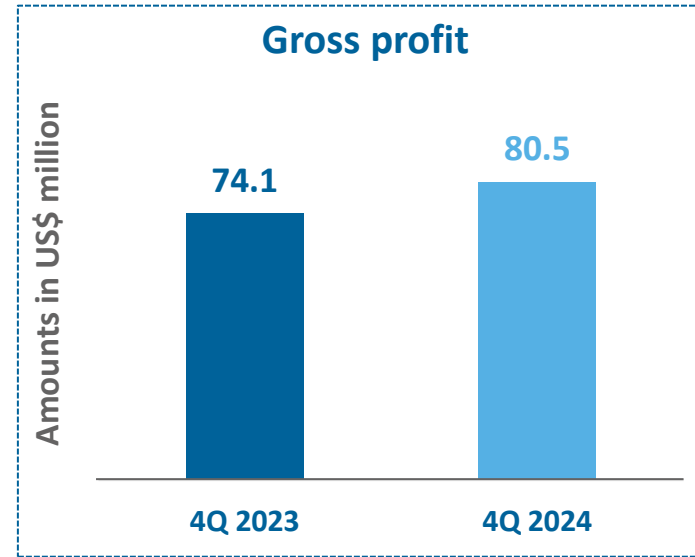
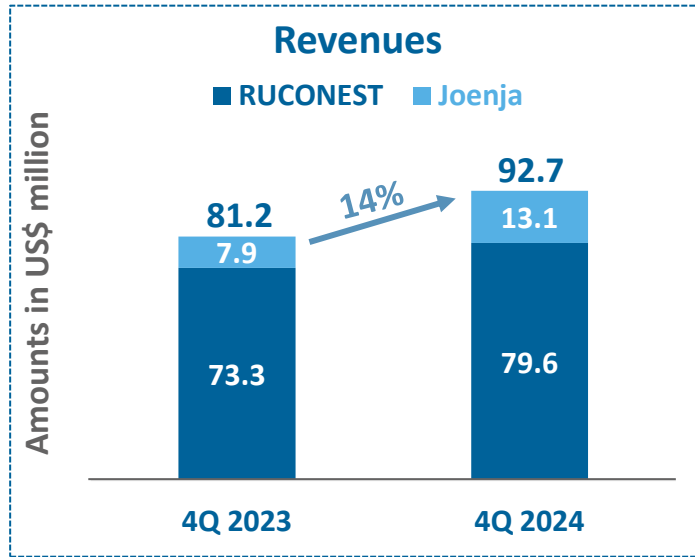
- Builds on Pharming's existing rare disease expertise and infrastructure
- Concentrated centers of excellence and strong advocacy groups



Jeroen Wakkerman
Chief Financial Officer

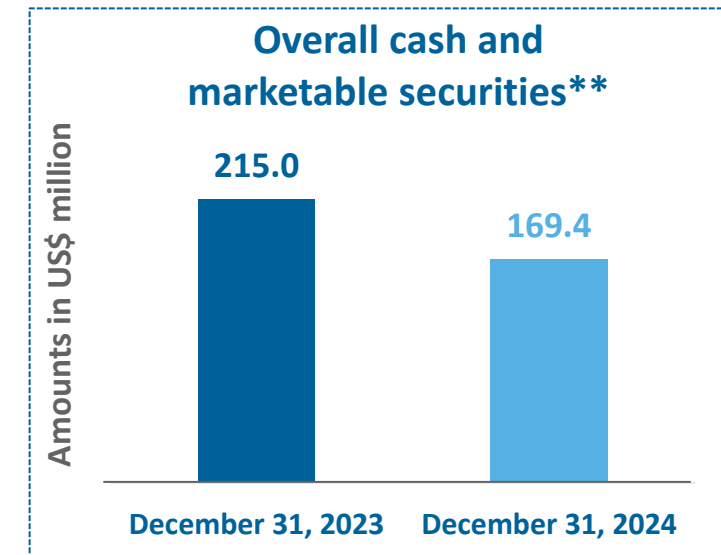
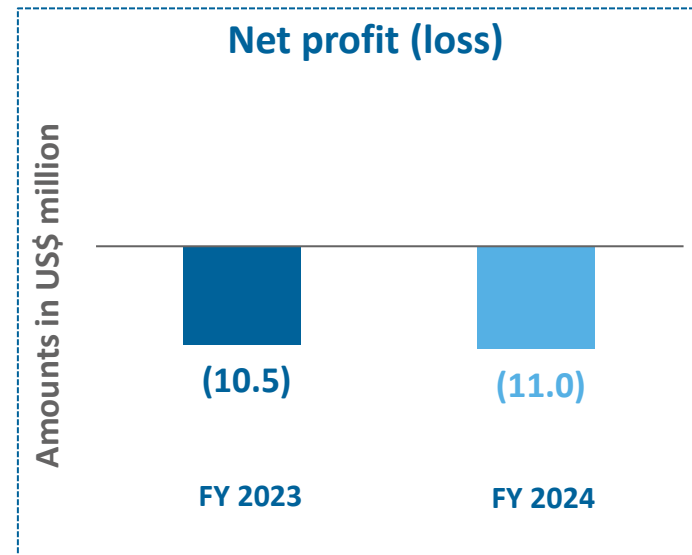
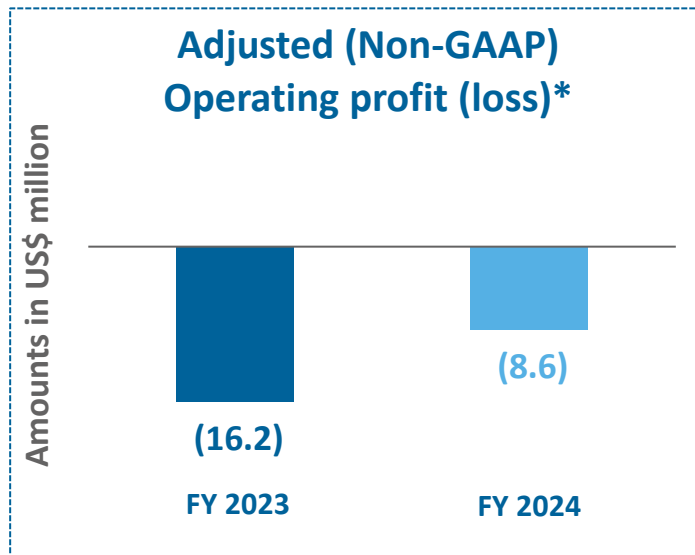
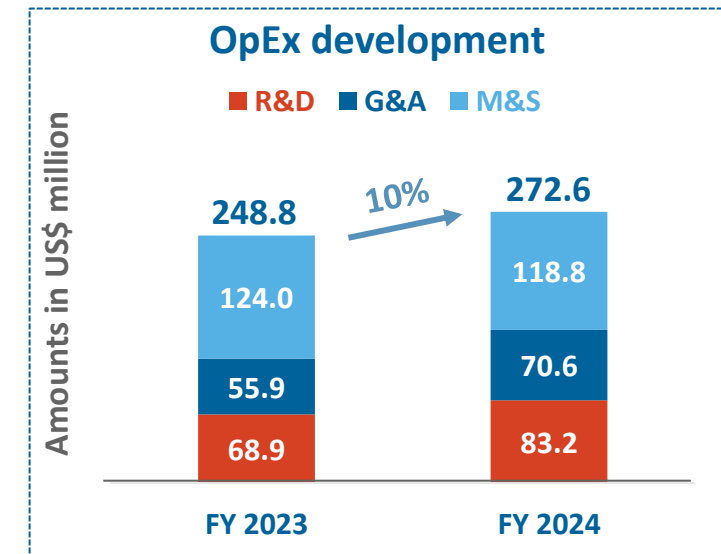
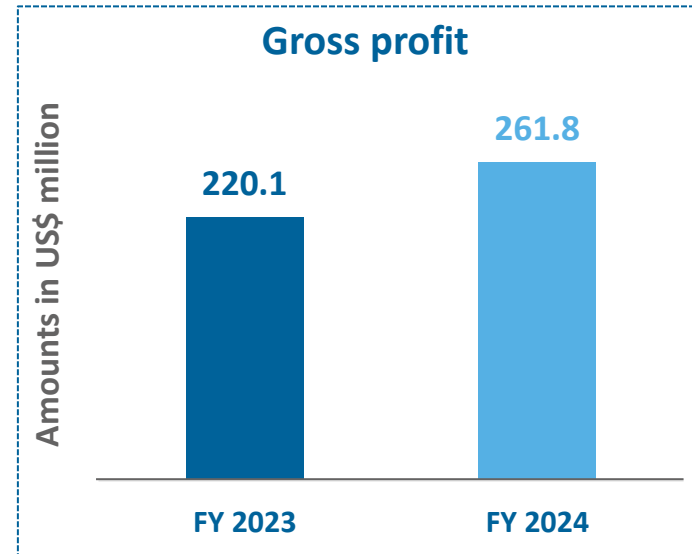
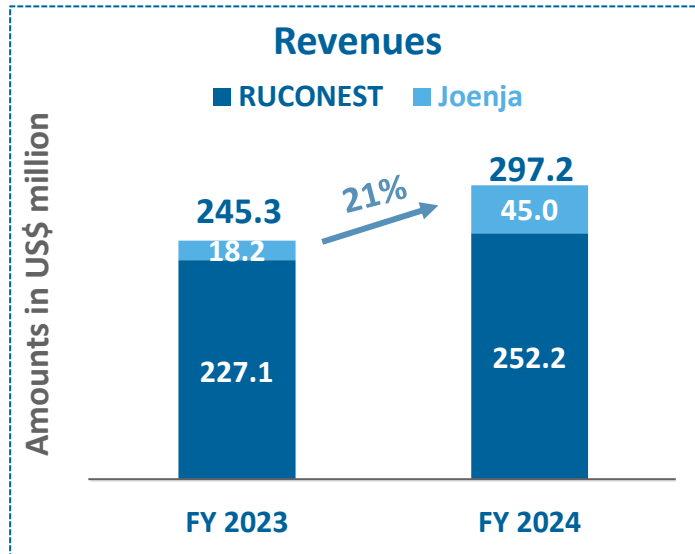
Financials

Financial highlights: 4Q 2024 vs 4Q 2023



* G&A expenses for 4Q 2024 include one-off items - impairment of the DSP facility (2024: US\$5.1 million, 2023: US\$4.7 million) and Abliva AB acquisition legal and advisory fees (US\$1.1 million).

Financial highlights: FY 2024 vs FY 2023



* Operating profit (loss) for 2023 excludes milestone payments for Joenja® (US\$10.5 million) and gain on sale of Priority Review Voucher to Novartis (US\$21.3 million).

** US\$30.4 million of the US\$45.6 million decrease in overall cash and marketable securities is due to convertible bond refinancing.

Acquisition Terms

- ❖ Acquisition through a public tender offer under Swedish Takeover Act and Nasdaq Stockholm Takeover Rules
- ❖ Offer price of SEK 0.45 in cash for each share in Abliva AB, totalling approximately \$66.1M USD *

Financial Details

- ❖ Acquisition of shares with available cash
- ❖ KL1333 in-licensed by Abliva from Yungjin Pharm, which is entitled to milestone and royalty payments **

Timing

- ❖ Announced ownership exceeding 90% on February 20 and initiated delisting activities
- ❖ Initiated compulsory acquisition procedure for remaining Abliva shares
- ❖ Abliva application for delisting was approved on March 3 and last day of trading will be March 17

Transaction illustrates our strategy of developing a high-value pipeline

*Based on an exchange rate of 0.0911 SEK / USD from 13 December 2024

**Worldwide rights, excl. Japan and South Korea primarily for the treatment of genetic mitochondrial disease; single-digit to low double-digit royalties on net sales, plus development and commercial milestone payments

Revenue and operating expenses:

	FY 2025 Guidance	Notes
Total Revenues	US\$315 - 335 million	6 - 13% growth
Operating Expenses (pre-Abliva impact)	Flat vs. FY 2024	
Operating Expenses (Abliva-related)	~US\$30 million	Preliminary estimate including R&D and non-recurring transaction and integration costs

Financial impact of Abliva acquisition:

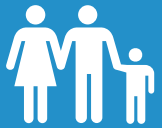
- Available cash and future cash flows expected to cover KL1333 development and pre-launch costs and current pipeline investments.
- Business combination - substantially all value concentrated in a single asset, KL1333.
- Following delisting as expected in March 2025, the acquisition would be reflected in our financial statements beginning with the current quarter. At this point we expect the US\$66.1M acquisition price to be allocated to the fair value of the acquired identifiable assets and liabilities, with any excess to be recorded as goodwill.



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2025 guidance: US\$315 - 335 million

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Appendix

Statement of profit and loss

Amounts in US\$ '000	2024	2023
Revenues	297,200	245,316
Costs of sales	(35,399)	(25,212)
Gross profit	261,801	220,104
Other income	2,177	23,349
Research and development	(83,161)	(68,914)
General and administrative	(70,619)	(55,877)
Marketing and sales	(118,819)	(124,049)
Other Operating Costs	(272,599)	(248,840)
Operating profit (loss)	(8,621)	(5,387)
Fair value gain (loss) on revaluation	4,990	(930)
Other finance income	6,820	3,663
Other finance expenses	(9,944)	(9,069)
Finance result, net	1,866	(6,336)
Share of net profits (loss) in associates using the equity method	(1,758)	(289)
Profit (loss) before tax	(8,513)	(12,012)
Income tax credit (expense)	(2,514)	1,464
Profit (loss) for the period	(11,027)	(10,548)
Basic earnings per share (US\$)	(0.016)	(0.016)
Diluted earnings per share (US\$)	(0.016)	(0.016)

Balance sheet – assets

Amounts in US\$ '000	December 31, 2024	December 31, 2023
Non-current assets		
Intangible assets	61,039	71,267
Property, plant and equipment	7,752	9,689
Right-of-use assets	16,382	23,777
Long-term prepayments	90	92
Deferred tax assets	31,090	29,761
Investment accounted for using the equity method	466	2,285
Investments in equity instruments designated as at FVTOCI	—	2,020
Investment in debt instruments designated as at FVTPL	3,767	6,093
Restricted cash	1,505	1,528
Total non-current assets	122,091	146,512
Current assets		
Inventories	55,724	56,760
Trade and other receivables	55,079	46,158
Marketable securities	112,949	151,683
Cash and cash equivalents	54,944	61,741
Total current assets	278,696	316,342
Total assets	400,787	462,854

Equity		
Share capital	7,769	7,669
Share premium	488,990	478,431
Other reserves	(222)	(2,057)
Accumulated deficit	(274,675)	(265,262)
Shareholders' equity	221,862	218,781
Non-current liabilities		
Convertible bonds	78,154	136,598
Lease liabilities	26,968	29,507
Total non-current liabilities	105,122	166,105
Current liabilities		
Convertible bonds	4,245	1,824
Trade and other payables	66,611	72,528
Lease liabilities	2,947	3,616
Total current liabilities	73,803	77,968
Total equity and liabilities	400,787	462,854

Amounts in \$'000	2024	2023
Profit (loss) before tax	(8,513)	(12,012)
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>		
Depreciation, amortization, impairment of non-current assets	16,070	15,925
Equity settled share based payments	11,248	9,251
Fair value loss (gain) on revaluation	(4,990)	930
Gain on disposal from PRV sale	—	(21,279)
Disposal of leases	22	—
Other finance income	(6,820)	(3,663)
Other finance expenses	9,887	9,069
Share of net profits in associates using the equity method	1,758	289
Other	—	(1,079)
Operating cash flows before changes in working capital	18,662	(2,569)
<i>Changes in working capital:</i>		
Inventories	(503)	(14,434)
Trade and other receivables	(6,783)	(18,539)
Payables and other current liabilities	(2,769)	16,228
Restricted cash	(17)	(216)
Total changes in working capital	(10,072)	(16,961)

Interest received	5,201	2,883
Income taxes received (paid)	(15,584)	(655)
Net cash flows generated from (used in) operating activities	(1,793)	(17,302)
Capital expenditure for property, plant and equipment	(790)	(1,437)
Proceeds on PRV sale	—	21,279
Investment intangible assets	(6)	(27)
Disposal of investment designated as at FVOCI	2,098	—
Purchases of marketable securities	(284,314)	(382,014)
Proceeds from sale of marketable securities	314,630	232,811
Net cash flows generated from (used in) investing activities	31,618	(129,388)
Payment of lease liabilities	(4,008)	(4,038)
Interests on lease liabilities	(1,141)	(1,088)
Net proceeds of issued convertible bonds	104,539	—
Repurchase of convertible bonds	(134,924)	—
Interests on convertible bonds	(4,457)	(4,046)
Settlement of share based compensation awards	5,579	8,133
Net cash flows generated from (used in) financing activities	(34,412)	(1,039)
Increase (decrease) of cash	(4,587)	(147,729)
Exchange rate effects	(2,210)	2,128
Cash and cash equivalents at the beginning of the period	61,741	207,342
Total cash and cash equivalents at December 31	54,944	61,741