



Pharming Group N.V.

Third quarter 2024 financial
results and business update

October 24, 2024

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



Sijmen de Vries, MD
Chief Executive Officer



Stephen Toor
Chief Commercial Officer



Anurag Relan, MD
Chief Medical Officer



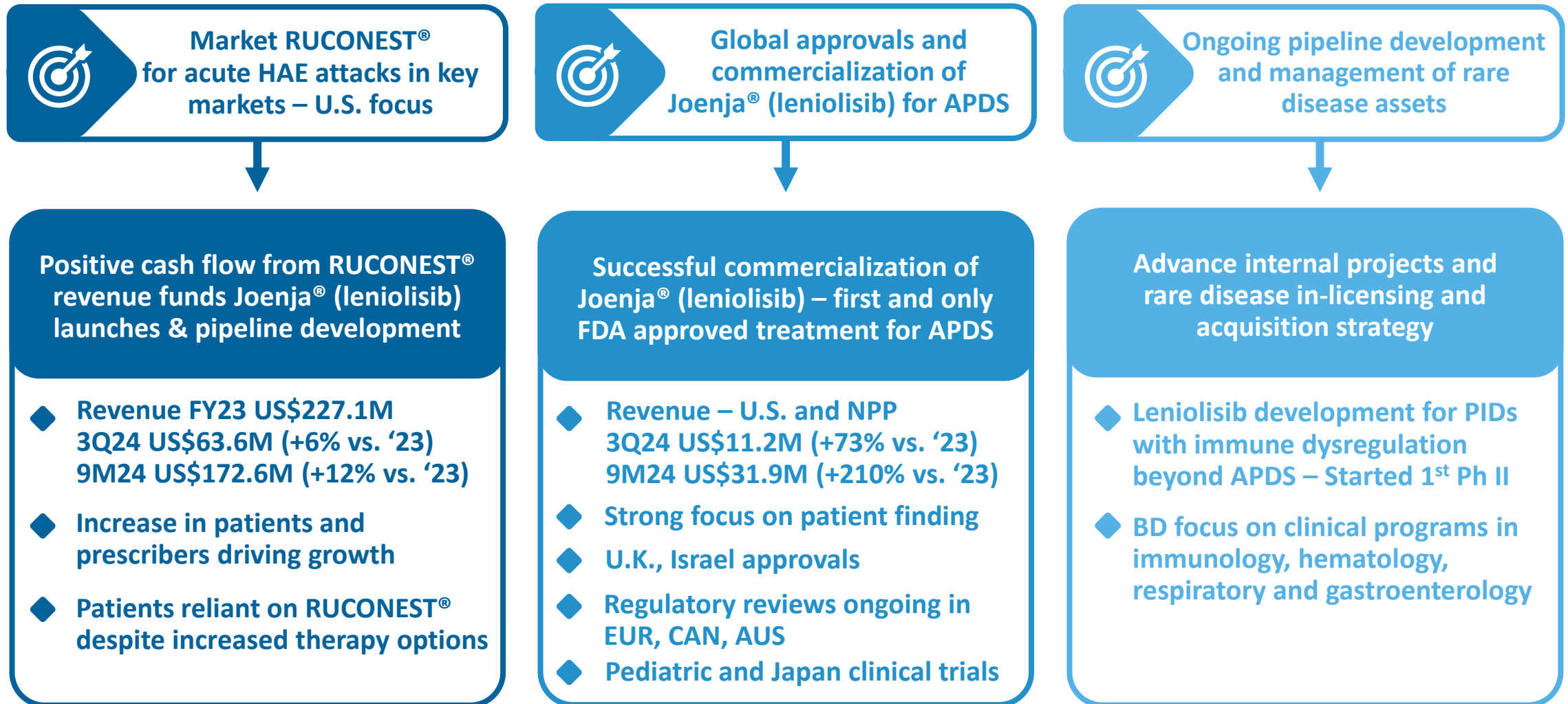
Jeroen Wakkerman
Chief Financial Officer

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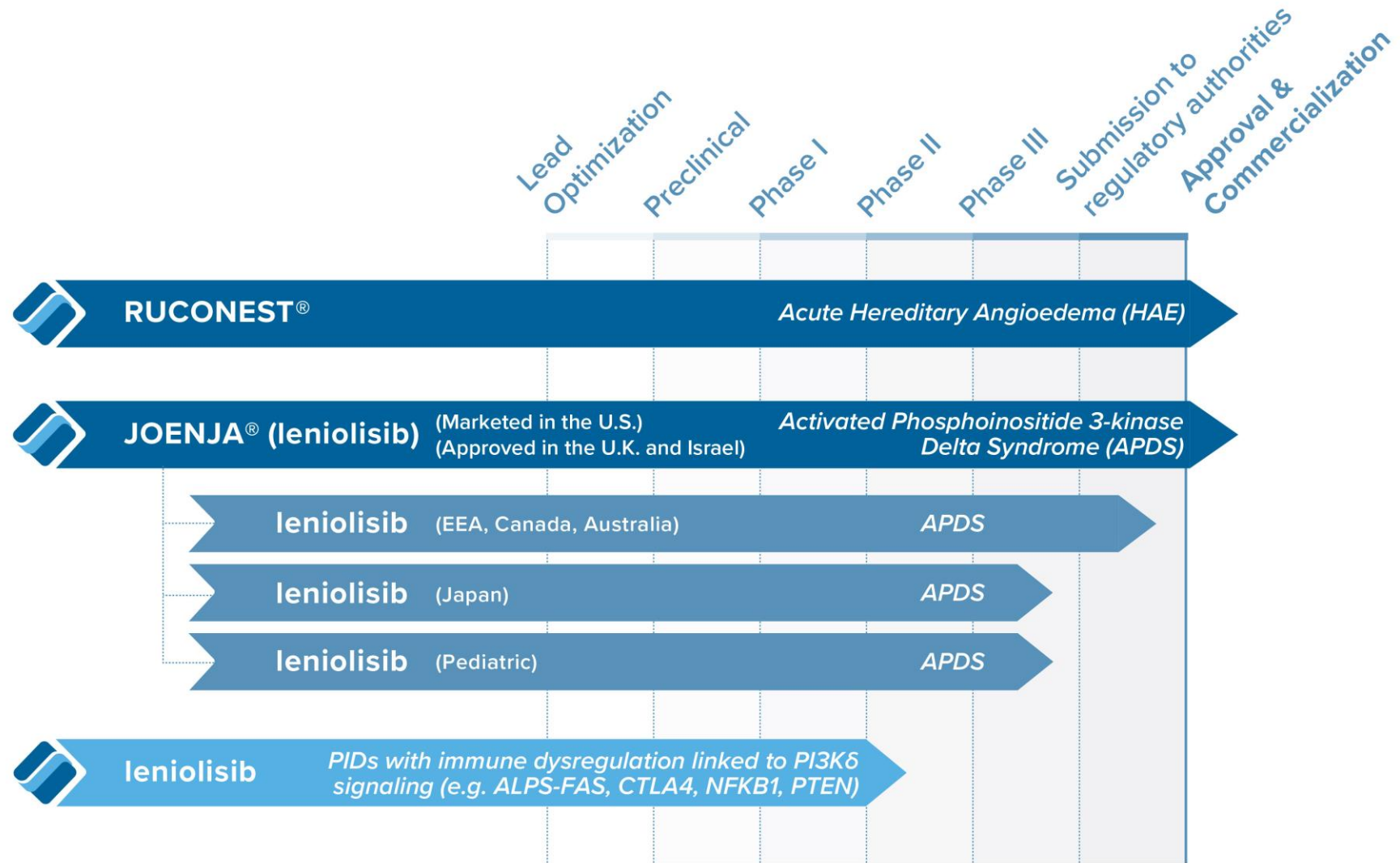
Sijmen de Vries, MD
Chief Executive Officer

Introduction



**2024 Total Revenue Guidance - \$280 – \$295M (14 – 20% growth)
Driven by Joenja®**

Pipeline – multiple commercial stage rare disease products

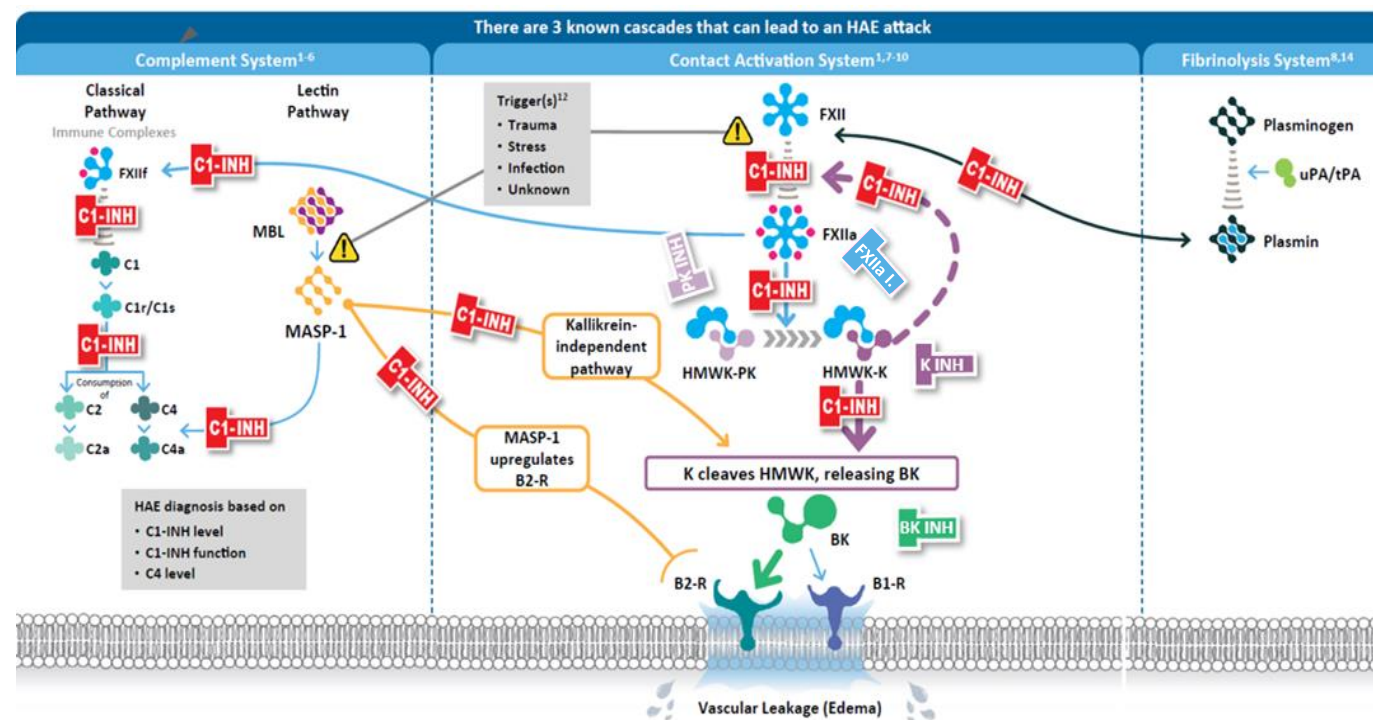


C1-INH targets the root cause of HAE

Addresses patient needs unmet by other therapies

TARGET			
BRAND NAME	GENERIC NAME	STATUS	TYPE
C1 Inhibitor			
Ruconest	C1 esterase inhibitor (recombinant)	Marketed	OD
Berinerit	C1 esterase inhibitor (human)	Marketed	OD
Haegarda	C1 esterase inhibitor (human)	Marketed	Prophy
Cinryze	C1 esterase inhibitor (human)	Marketed	Prophy
Pre-Kallikrein			
n/a	donidalorsen	Phase 3	Prophy
n/a	NTLA-2002	Phase 1/2	Prophy
Plasma Kallikrein			
Kalbitor	ecallantide	Marketed	OD
Orladeyo	berotralstat	Marketed	Prophy
Takhzyro	lanadelumab	Marketed	Prophy
n/a	sebetralstat	NDA	OD
n/a	STAR-0215	Phase 2	Prophy
FXIIa			
n/a	garadacimab	BLA	Prophy
Bradykinin B2			
Firazyr	icatibant	Marketed	OD
n/a	deucricitabant (PHVS416)	Phase 3	OD
n/a	deucricitabant (PHVS719)	Phase 2	Prophy

Overview of Marketed and In-Development Therapies and Their Targets Within the Three Known Cascades Leading to HAE Attacks



Source: Cascade Adapted from a clinical cascade developed in partnership with Dr. Allen Kaplan. This is a current scientific understanding of the cascades. Clinical implications are unknown.



Stephen Toor

Chief Commercial Officer

Commercial update

RUCONEST® (rhC1INH): trusted treatment cornerstone for HAE



The only recombinant treatment that targets the root cause of HAE by replacing missing or dysfunctional C1-INH



2nd most prescribed product for acute attacks
Typical patient: failed icatibant (BK inh) and on prophylaxis (K inh)



Well-tolerated and effective treatment option for acute hereditary angioedema (HAE) - including breakthrough attacks



97%: needed just 1 dose of RUCONEST®¹
93%: acute attacks stopped with RUCONEST® for at least 3 days²



Strong U.S. in-market demand –
New enrollments up 25% in FY23
~100 enrollments in 3Q24



Performing well in leading U.S. revenue indicators: patients on therapy, vials shipped, physicians prescribing (786, +57 vs. 2023)



Revenue:
FY23 US\$227.1M (+10%)
9M24 US\$172.6M (+12%)



Strong growth in 2024, well positioned vs. acute orals in late-stage development

Joenja[®] (leniolisib) – strong execution and growth opportunity



Joenja [®] U.S. (APDS)	Leniolisib (APDS)	Leniolisib for Primary Immunodeficiencies (PIDs)
<ul style="list-style-type: none"> Marketed (12+) Found >230 of ~500 patients 93 patients on paid therapy + 5 pending >30 diagnosed patients (12+) not yet enrolled and >60 pediatric Growth potential from patient finding and VUS efforts 	<ul style="list-style-type: none"> Found >870 patients globally Global expansion / regulatory reviews Pediatric studies / label expansion (>25% patients) 164 patients in EAP, clinical studies, and NPP 	<ul style="list-style-type: none"> Phase II POC trial in PIDs with immune dysregulation linked to PI3Kδ signaling Similar to APDS Seeking regulatory feedback on third PID indication
<p>Prevalence: ~1.5 / million ~2,400 patients</p>		<p>~7 / million</p>

- ◆ Revenues:
 - U.S. commercial sales
 - Europe / RoW access program
- ◆ 3Q24 revenue US\$11.3M
- ◆ 9M24 revenue US\$31.9M
- ◆ U.S. Pricing
 - 30-day supply \$47,220
 - Annual cost (WAC) \$566,640
- ◆ Global expansion:
 - Europe, U.K., Japan, Asia Pacific, Middle East, Latin America and Canada



Anurag Relan, MD
Chief Medical Officer

**Joenja[®] (leniolisib)
for APDS**

leniolisib for PIDs



Medical education to raise awareness of APDS and share leniolisib data

- ◆ Conferences and congresses
- ◆ Abstracts
- ◆ Publications



NEXUS



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American
College
of Allergy, Asthma
& Immunology



American Academy of
**Allergy Asthma
& Immunology**



Genetic testing

- ◆ Sponsored, no-cost testing program 
- ◆ Assistance from Genetic counselors
- ◆ Partnering with genetic testing companies to identify APDS patients



Family testing

- ◆ Inherited disease* but most APDS patients do not have diagnosed family members
- ◆ Cooperating with clinicians to educate/encourage family testing
- ◆ Genetic testing offered through partner Genome Medical



VUS resolution

- ◆ Validation studies with various laboratories to confirm which Variants of Uncertain Significance (VUSs) should be classified as APDS
- ◆ Diagnose additional APDS patients amongst those who have clinical symptoms and a VUS test result (>1,200 patients in U.S.)**
- ◆ Variant curation (ClinGen, Genomenon)
- ◆ Functional testing (PI3K pathway activity)
- ◆ Multiplexed assays of variant effect (MAVE) studies (complete 4Q24)

*APDS genes are autosomal dominant meaning there is a 50% chance that a blood relative of an APDS patient may also carry that gene and in turn have APDS.

**To date Pharming has identified more than 1,200 patients in the U.S. with VUSs. As results become available, patients with validated variants could be diagnosed with APDS and be eligible for Joenja® treatment.



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



U.K. marketing authorization received September 25, 2024



Japan clinical study: Patient enrollment is now complete
PMDA filing mid-2025



Expanded Access and Named Patient Programs



Israel marketing authorization received April 30, 2024



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



Pediatric studies
4 to 11 years - Enrollment complete
1 to 6 years - Enrollment continuing
Filing to begin 2H 2025



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

* Anticipate regulatory action in 2025 for Australia

◆ Primary Immunodeficiencies (PIDs) are a broad group of disorders with key potential features:

- Genetic basis
- Immune dysfunction → increased risk of infection
- Immune dysregulation → lymphoproliferation and autoimmunity
- High morbidity and mortality

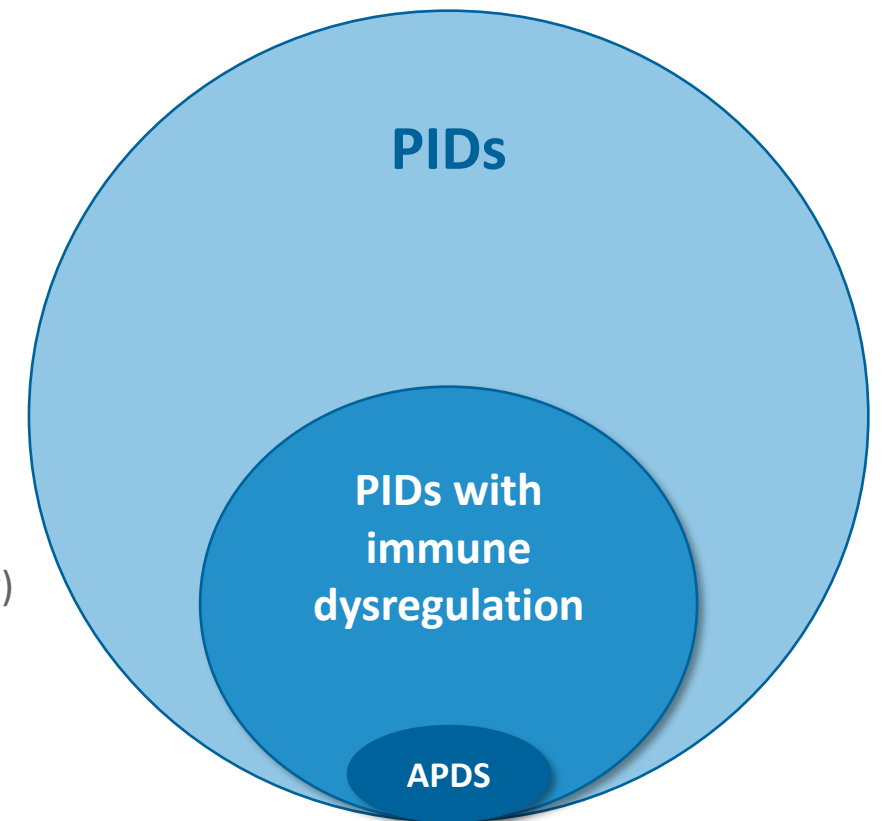
◆ Pharming developing leniolisib for PIDs with immune dysregulation beyond APDS

PIDs with immune dysregulation linked to PI3K δ signaling

- Multiple PIDs with alterations in PI3K δ signaling (including ALPS-FAS, CTLA4 haploinsufficiency, NFKB1 haploinsufficiency and PTEN deficiency)
- Clinical manifestations, disease onset and severity similar to APDS
- No approved therapies
- Prevalence ~7/million (approximately five times that of APDS)
- Phase II proof of concept clinical trial started October 2024

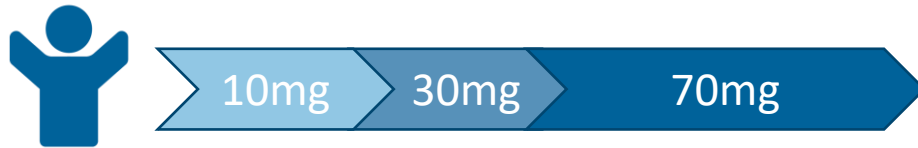
Next indication

- Obtaining regulatory feedback on proposed clinical development plan



Not to scale with population sizes

Phase II proof of concept clinical trial – single arm, open-label, dose range-finding study (N=12)



- Patients with PIDs linked to PI3K δ signaling, e.g. ALPS-FAS¹, CTLA4 haploinsufficiency², NFKB1 haploinsufficiency³, PTEN deficiency⁴ (treatable population ~7/million)
- Primary: Safety & Tolerability
- Secondary/Exploratory: PK/PD, efficacy measures
- 10/30/70 mg: 4/4/12 wks treatment, respectively
- Pick Best Dose regimen for Phase III



National Institute of
Allergy and
Infectious Diseases

Lead Investigator: Gulbu Uzel, M.D., Senior Research Physician

Co-Investigator: V. Koneti Rao, M.D., FRCPA, Senior Research Physician
Primary Immune Deficiency Clinic (ALPS Clinic)

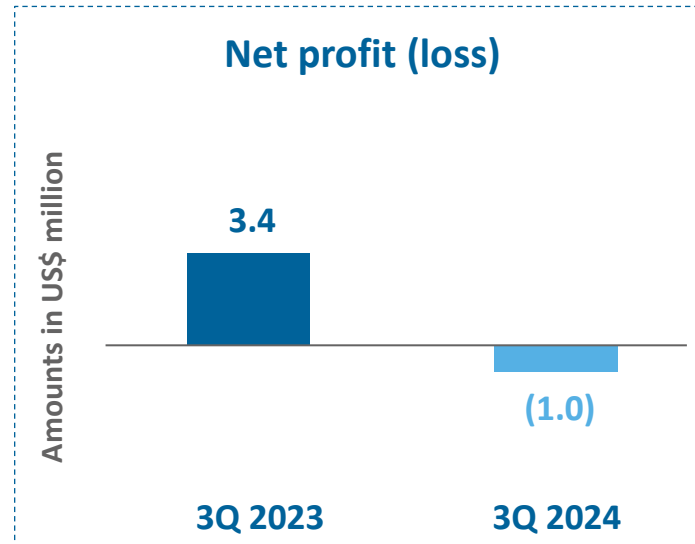
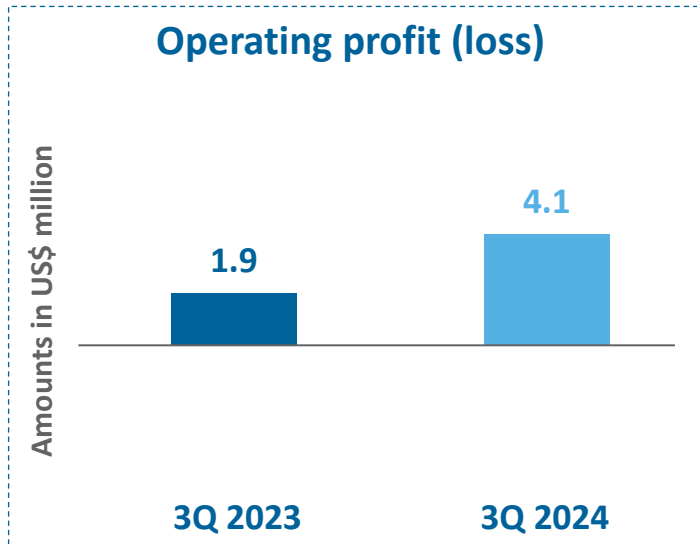
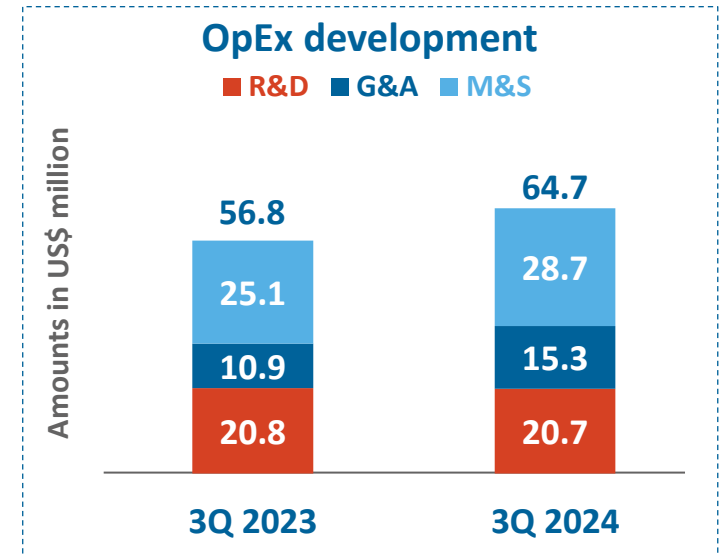
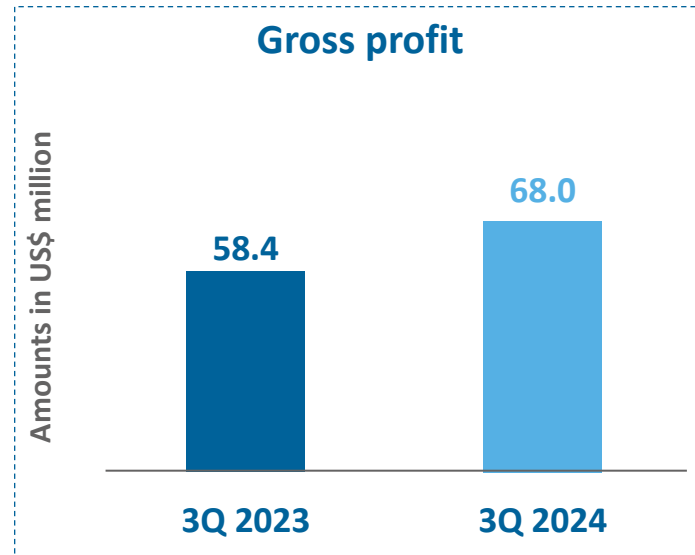
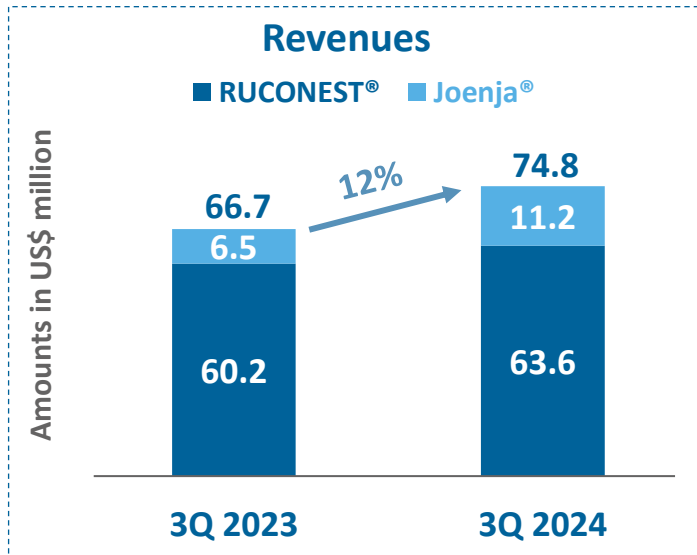
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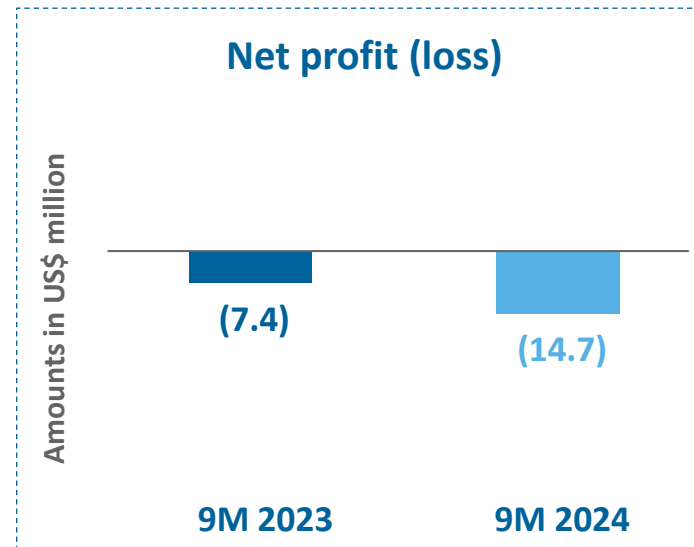
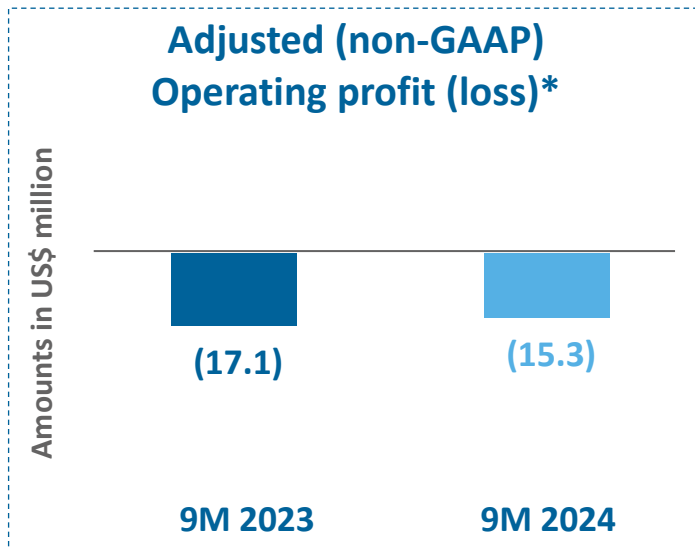
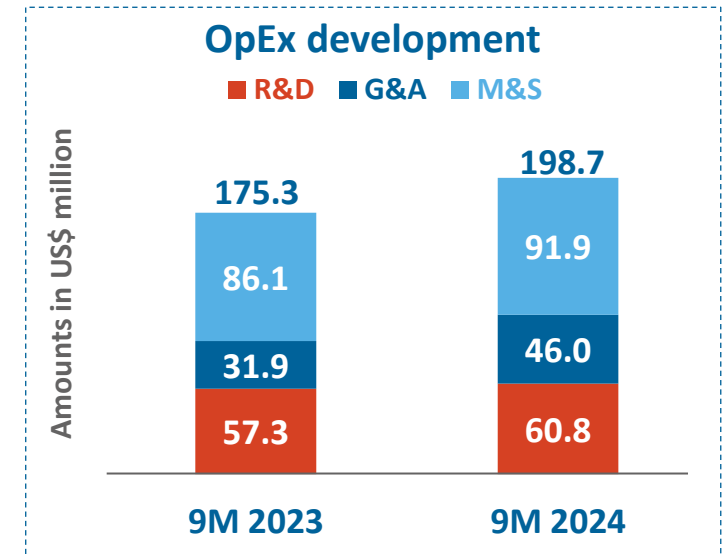
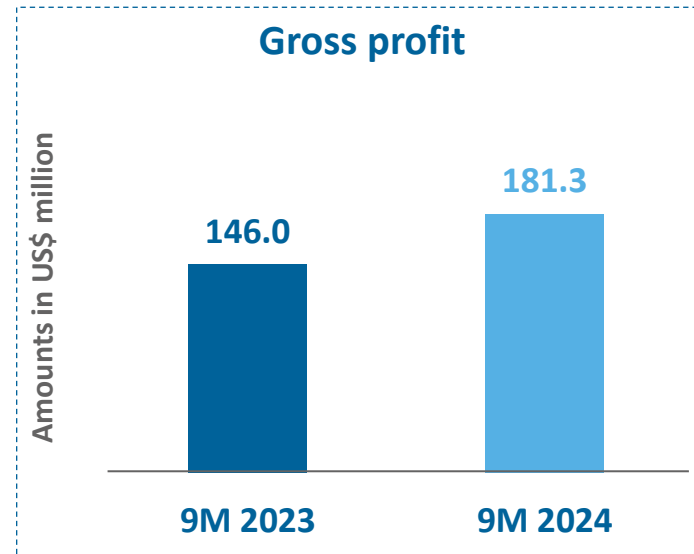
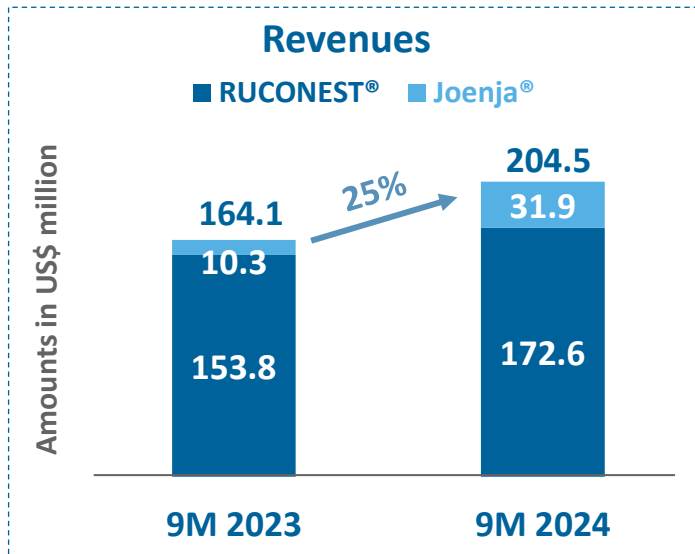
Jeroen Wakkerman
Chief Financial Officer

Financials

Financial highlights: 3Q 2024 vs 3Q 2023

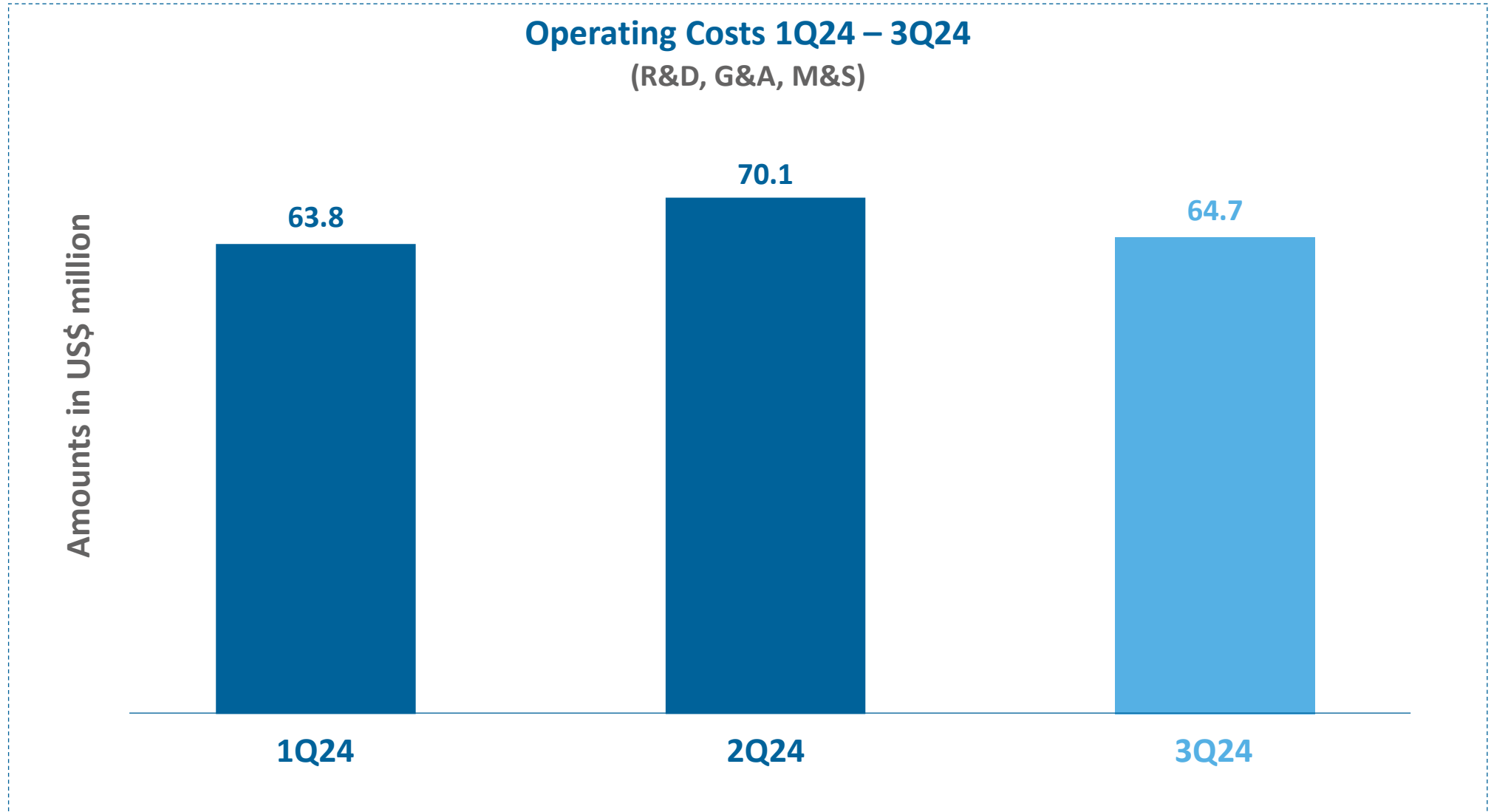


Financial highlights: 9M 2024 vs 9M 2023



* Operating profit (loss) for 9M 2023 excludes milestone payments for Joenja® (US\$10.5 million) and gain on sale of Priority Review Voucher to Novartis (US\$21.1 million).

** US\$30.4 million of the US\$41.6 million decrease in overall cash and marketable securities is due to convertible bond refinancing.



	FY 2024 Revenue Guidance	% Growth vs. FY 2023
Total Revenues	US\$280 - 295 million	14-20%

- ◆ Joenja[®] significant driver of revenue growth, continued RUCONEST[®] growth
- ◆ Joenja[®] revenue assumptions:
 - Continued growth in patients on paid therapy
 - Continued high (monthly) adherence (compliance) rates >85%
 - U.S. Pricing: 30-day supply \$47,220, Annual cost (WAC) \$566,640, GTN Discount ~15%
- ◆ OpEx adjustments to continue in 4Q



Total revenues between US\$280 and US\$295 million (14% to 20% growth).



Joenja® (leniolisib) U.S.: Continued progress finding additional APDS patients, supported by family testing and VUS validation efforts, and subsequently converting patients to paid therapy.



Leniolisib ex-U.S.: Increasing revenues through our Named Patient Program and other funded early access programs in key global markets.



Completion of leniolisib clinical trials to support regulatory filings for approval in Japan and pediatric label expansion in key global markets.



Progress towards regulatory approvals for leniolisib in the EEA, Canada and Australia.



Advancing the Phase II clinical trial for leniolisib in PIDs with immune dysregulation linked to PI3K δ signaling to significantly expand the long-term commercial potential of leniolisib.



Continued focus on potential acquisitions and in-licensing of clinical stage opportunities in rare diseases (e.g. immunology, hematology, respiratory and gastroenterology).



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This presentation, a recording and a transcript of this call will be made available on the company's website

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Pharming Group N.V.

Appendix

Statement of profit and loss

Amounts in US\$ '000	3Q 2024	3Q 2023	9M 2024	9M 2023
Revenues	74,849	66,661	204,528	164,099
Costs of sales	(6,819)	(8,295)	(23,186)	(18,094)
Gross profit	68,030	58,366	181,342	146,005
Other income	777	304	2,034	22,811
Research and development	(20,721)	(20,753)	(60,839)	(57,287)
General and administrative	(15,292)	(10,886)	(45,999)	(31,849)
Marketing and sales	(28,686)	(25,123)	(91,863)	(86,136)
Other Operating Costs	(64,699)	(56,762)	(198,701)	(175,272)
Operating profit (loss)	4,108	1,908	(15,325)	(6,456)
Fair value gain (loss) on revaluation	21	—	5,159	—
Other finance income	825	1,251	3,760	2,050
Other finance expenses	(2,998)	633	(7,488)	(4,621)
Finance result, net	(2,152)	1,884)	1,431)	(2,571)
Share of net profits (loss) in associates using the equity method	(442)	(485)	(1,276)	(954)
Profit (loss) before tax	1,514	3,307	(15,170)	(9,981)
Income tax credit (expense)	(2,548)	157	470	2,556
Profit (loss) for the period	(1,034)	3,464	(14,700)	(7,425)
Basic earnings per share (US\$)	(0.002)	0.005	(0.022)	(0.011)
Diluted earnings per share (US\$)	(0.002)	0.005	(0.022)	(0.011)

Balance sheet – assets

Amounts in US\$ '000	September 30, 2024	December 31, 2023
Non-current assets		
Intangible assets	67,096	71,267
Property, plant and equipment	8,692	9,689
Right-of-use assets	21,975	23,777
Long-term prepayments	93	92
Deferred tax assets	36,752	29,761
Investment accounted for using the equity method	1,016	2,285
Investments in equity instruments designated as at FVTOCI	—	2,020
Investment in debt instruments designated as at FVTPL	6,150	6,093
Restricted cash	1,548	1,528
Total non-current assets	143,322	146,512
Current assets		
Inventories	62,227	56,760
Trade and other receivables	48,199	46,158
Marketable securities	111,104	151,683
Cash and cash equivalents	60,662	61,741
Total current assets	282,192	316,342
Total assets	425,514	462,854

Equity		
Share capital	7,750	7,669
Share premium	487,079	478,431
Other reserves	9,334	(2,057)
Accumulated deficit	(278,371)	(265,262)
Shareholders' equity	225,792	218,781
Non-current liabilities		
Convertible bonds	92,099	136,598
Lease liabilities	27,784	29,507
Total non-current liabilities	119,883	166,105
Current liabilities		
Convertible bonds	3,319	1,824
Trade and other payables	72,638	72,528
Lease liabilities	3,882	3,616
Total current liabilities	79,839	77,968
Total equity and liabilities	425,514	462,854

Amounts in \$'000	3Q 2024	3Q 2023	9M 2024	9M 2023
Profit (loss) before tax	1,514	3,307	(15,170)	(9,981)
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>				
Depreciation, amortization, impairment of non-current assets	2,743	2,902	8,371	8,370
Equity settled share based payments	2,918	1,965	8,605	5,935
Fair value loss (gain) on revaluation	(21)	—	(5,159)	—
Gain on disposal from PRV sale	—	—	—	(21,080)
Other finance income	(182)	(1,251)	(3,117)	(2,050)
Other finance expenses	2,315	(633)	6,765	4,621
Share of net result in associates using the equity method	442	485	1,276	954
Other	—	1,055	—	(1,130)
Operating cash flows before changes in working capital	9,729	7,830	1,571	(14,361)
<i>Changes in working capital:</i>				
Inventories	(2,133)	(396)	(5,248)	(11,113)
Trade and other receivables	2,919	(7,363)	(2,044)	(12,902)
Payables and other current liabilities	6,560	3,242	4,305	8,075
Restricted cash	—	(47)	—	363
Total changes in working capital	7,346	(4,563)	(2,987)	(15,577)

Cash flow (2/2)

Interest received	1,784	260	4,154	1,059
Income taxes received (paid)	(9,117)	—	(13,864)	—
Net cash flows generated from (used in) operating activities	9,742	3,527	(11,126)	(28,879)
Capital expenditure for property, plant and equipment	(366)	(147)	(660)	(1,133)
Proceeds on PRV sale	—	—	—	21,080
Investment intangible assets	—	23	—	23
Disposal of investment designated as at FVOCI	8	—	1,972	—
Purchases of marketable securities	(109,796)	(144,554)	(222,249)	(231,901)
Proceeds from sale of marketable securities	114,504	86,451	262,345	86,451
Net cash flows generated from (used in) investing activities	4,350	(58,227)	41,408	(125,480)
Payment of lease liabilities	(918)	(1,007)	(2,485)	(3,022)
Interests on lease liabilities	(258)	(270)	(784)	(825)
Net proceeds of issued convertible bonds	(263)	—	104,539	—
Repurchase of convertible bonds	(9)	—	(134,931)	—
Interests on convertible bonds	(8)	(2,029)	(2,032)	(4,052)
Settlement of share based compensation awards	23	8,546	3,485	7,880
Net cash flows generated from (used in) financing activities	(1,433)	5,240	(32,208)	(19)
Increase (decrease) of cash	12,659	(49,460)	(1,926)	(154,378)
Exchange rate effects	861	(913)	847	1,689
Cash and cash equivalents at the beginning of the period	47,142	105,026	61,741	207,342
Total cash and cash equivalents at September 30	60,662	54,653	60,662	54,653