Pharming Group N.V.

First quarter 2024 financial results and business update

May 8, 2024

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

Pharming®

SPEAKERS

Sijmen de Vries, MD
Chief Executive Officer

Stephen Toor
Chief Commercial Officer

Anurag Relan, MD
Chief Medical Officer

Jeroen Wakkerman
Chief Financial Officer

Forward-looking statements

This presentation may contain forward-looking statements. Forward-looking statements are statements of future expectations that are based on management's current expectations and assumptions and involve known and unknown risks and uncertainties that could cause actual results, performance, or events to differ materially from those expressed or implied in these statements. These forward-looking statements are identified by their use of terms and phrases such as "aim", "ambition", ''anticipate'', ''believe'', ''could'', ''estimate'', ''expect'', ''goals'', ''intend'', ''may'', "milestones", ''objectives'', ''outlook'', ''plan'', ''probably'', ''project'', ''risks'', "schedule", ''seek'', ''should'', ''target'', ''will'' and similar terms and phrases. Examples of forward-looking statements may include statements with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, and Pharming's expectations regarding its projected working capital requirements and cash resources, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope, progress and expansion of Pharming's clinical trials and ramifications for the cost thereof; and clinical, scientific, regulatory, commercial, competitive and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2023 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2023, filed with the U.S. Securities and Exchange Commission, the events and circumstances discussed in such forward-looking statements may not occur, and Pharming's actual results could differ materially and adversely from those anticipated or implied thereby. All forward-looking statements contained in this presentation are expressly qualified in their entirety by the cautionary statements contained or referred to in this section. Readers should not place undue reliance on forward-looking statements. Any forward-looking statements speak only as of the date of this presentation and are based on information available to Pharming as of the date of this presentation. Pharming does not undertake any obligation to publicly update or revise any forward-looking statement as a result of new information, future events or other information.

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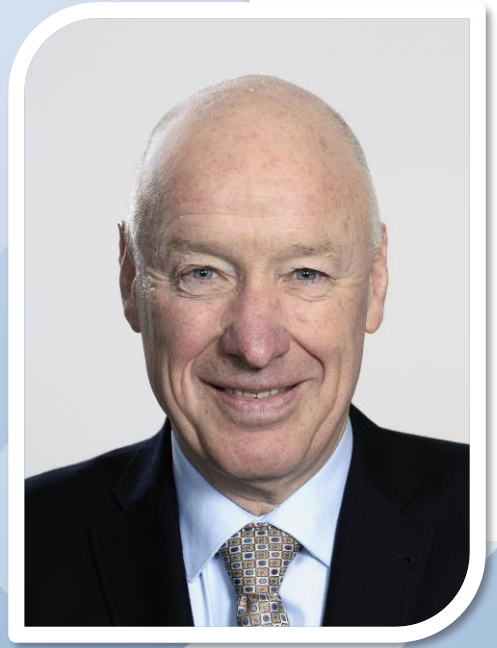
CEO



Sijmen de Vries, MD

Chief Executive Officer

Introduction

Building a leading global rare disease biopharma company

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Market RUCONEST® for acute HAE attacks in key markets – U.S. focus

Positive cash flow from RUCONEST® revenue funds Joenja® (leniolisib) launches & pipeline development

- FY23 revenue US$227.1M
- 1Q24 revenue US$46.0M (+8%)
- Increase in patients and prescribers driving growth
- Patients reliant on RUCONEST® despite increased therapy options

Global approvals and commercialization of Joenja® (leniolisib) for APDS

Successful commercialization of Joenja® (leniolisib) – first and only FDA approved treatment for APDS – U.S. launch April 2023

- Revenue FY23 US$18.2M
  1Q24 US$9.6M (+21% vs. 4Q23)
- Strong focus on patient finding
- Israel approval (April 2024)
- Regulatory reviews ongoing in EUR, U.K., CAN, AUS
- Pediatric and Japan clinical trials

Ongoing pipeline development and management of rare disease assets

Advance internal projects and rare disease in-licensing and acquisition strategy

- Leniolisib development for PIDs with immune dysregulation beyond APDS – preparing Ph2
- BD focus on clinical programs in immunology, hematology, respiratory and gastroenterology
- OTL- 105 discontinued

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

Joenja® (leniolisib) franchise – multi-year growth potential

Joenja® U.S. (APDS)	Leniolisib (APDS)	Leniolisib for Primary Immunodeficiencies (PIDs)
• Marketed (12+) • Significant portion of identified patients on paid therapy • Ongoing patient finding and VUS resolution efforts	• Patients on early access/ named patient programs • Global expansion / regulatory reviews • Pediatric studies	• Phase II POC trial in PIDs with immune dysregulation linked to PI3Kδ signaling • Symptoms similar to APDS

Prevalence: **~1.5 / million ~2,000 patients** | **~5 / million**

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CCO

Stephen Toor

Chief Commercial Officer

Commercial update

RUCONEST® (rhC1INH): trusted treatment cornerstone for HAE

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- The only recombinant treatment that targets the root cause of HAE by replacing missing or dysfunctional C1-INH
- Second most prescribed product for acute attacks
- 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 days[2]
- Strong U.S. in-market demand –
  New enrollments up 25% in FY23
  Almost 70 enrollments in 1Q24
- Performing well in leading U.S. revenue indicators: active patients, vials shipped, physicians prescribing (744, +15 vs. 2023)
- Revenue:
  FY23 US$227.1M (+10%)
  1Q24 US$46.0M (+8%)
- Continued growth in 2024, strong positioning vs. acute orals in late-stage development

References: 1. RUCONEST®. Prescribing information. Pharming Healthcare Inc; 2020. 2. Bernstein JA, et al. *Ann Allergy Asthma Immunol.* 2017;118(4):452-453. 3. Data on file. Pharming Healthcare Inc; 2019
The most common adverse reactions (incidence ≥2%) were headache, nausea and diarrhea. The most serious adverse reaction reported in clinical trials was anaphylaxis.

Joenja® U.S. launch: strong commercial execution

- Strong commercial execution 12 months into U.S. launch
- Continue to enroll and add patients on paid therapy in 1Q24
  83 patients on paid therapy at end 1Q24, with 5 additional enrollments pending authorization
  >50 diagnosed patients (12+) not yet enrolled and >50 pediatric
- 1Q24 revenue US$9.6M (+21% vs. 4Q23)
  Includes US$1.1M Europe and RoW revenue
- ~500 APDS patients in the U.S.* with >220 diagnosed at end 1Q24
  +15 diagnosed patients in 1Q24, including patients diagnosed via VUS resolution
- Significant focus on genetic family testing
- Variant of uncertain significance (VUS) validation studies to complete in 4Q24
  focused on >1100 patients identified in the U.S. with VUSs

* Prevalence estimated at 1.5 patients per million population, based on available literature
As of December 31, 2023, Pharming has identified >840 diagnosed APDS patients in global markets
>730 of these patients are in key global launch markets in the U.S., Europe, the U.K., Japan, Asia Pacific, Middle East, and Canada with total prevalence of ~2000 APDS patients

Joenja® (leniolisib) franchise – multi-year growth potential

Joenja® U.S. (APDS)	Leniolisib (APDS)	Leniolisib for Primary Immunodeficiencies (PIDs)
• Marketed (12+) • 83 patients on paid therapy / 5 pending • Found >220 of ~500 patients • >50 diagnosed patients (12+) not yet enrolled and >50 pediatric • Ongoing patient finding and VUS resolution efforts	• Global expansion / regulatory reviews • Pediatric studies • Found >840 patients globally • 138 patients on therapy (access programs and clinical studies)	• Phase II POC trial in PIDs with immune dysregulation linked to PI3Kδ signaling • Similar to APDS
Prevalence:	**~1.5 / million ~2,000 patients**	**~5 / million**

- Joenja® U.S. and Europe / RoW access program revenues support 2024 guidance
- U.S. Pricing: 30-day supply $47,220, Annual cost (WAC) $566,640
- Global expansion focused on Europe, U.K., Japan, Asia Pacific, Middle East, and Canada

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CMO

Anurag Relan, MD
Chief Medical Officer

Joenja® (leniolisib) for APDS

leniolisib for PIDs

U.S. launch of Joenja®: a much-needed treatment for APDS patients and another achievement for Pharming

Joenja® (leniolisib) is a prescription medicine that is used to treat activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older

In a randomized placebo-controlled trial of patients with APDS

- Joenja® met both primary end points with significant efficacy results
- Demonstrated significant improvement in other secondary and exploratory parameters

There were no drug-related serious adverse events or study withdrawals in Joenja® trials

Joenja® reported additional findings from an ongoing long-term open-label extension study interim analysis: reductions/discontinuations in IRT and reduction in infection rates

Extension study interim analysis demonstrated safety consistent with the randomized, controlled trial. We continue to collect observational long-term data on lymphadenopathy, naive B cells and IgM

Please see Important Safety Information and full Prescribing Information available at joenja.com

Rao VK, et al. Blood. 2023;141(9):971-983
Rao VK, et al. Poster presented at: 64th Annual American Society of Hematology Annual Meeting; December 10-13, 2022; New Orleans, LA.

Hiding in plain sight: Patient finding strategy

Medical education to raise awareness of APDS and share leniolisib data

- Conferences and congresses
- Abstracts
- Publications

AMCP NEXUS

American College of Allergy, Asthma & Immunology

IPIC2023 INTERNATIONAL PRIMARY IMMUNODEFICIENCIES CONGRESS

American Academy of Allergy Asthma & Immunology

Genetic testing

- Sponsored, no-cost testing program navigateAPDS by Pharming
- 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 (>1,100 patients in U.S.)**
- Variant curation (ClinGen, Genomenon)
- Functional testing (PI3K pathway activity)
- Multiplexed assays of variant effect (MAVE) studies
- Completion of studies during 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 1,100 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.

Joenja® – looking beyond FDA approval

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Europe – awaiting CHMP opinion on MAA

Israel marketing authorization received April 30, 2024

Japan clinical study: Patient enrollment is now complete

PMDA filing following completion of appropriate clinical trials

U.K., CAN, AUS submissions under regulatory review

Approvals in 2024-25* **

Pediatric study for 4 to 11 years

Enrollment completed

Pediatric study for 1 to 6 years ongoing

First patient dosed November 2023, enrollment continuing as planned

Expanded Access and Named Patient Programs

Initiate leniolisib development for PIDs with immune dysregulation (Phase II trial)

* In the U.K., Pharming filed an MAA on March 12, 2024 through the International Recognition Procedure (IRP) on the basis of FDA approval. The MAA was validated on April 17, 2024. The MHRA has 110 days from the date the IRP submission is validated, with an optional clock stop at Day 70, to review and issue its decision

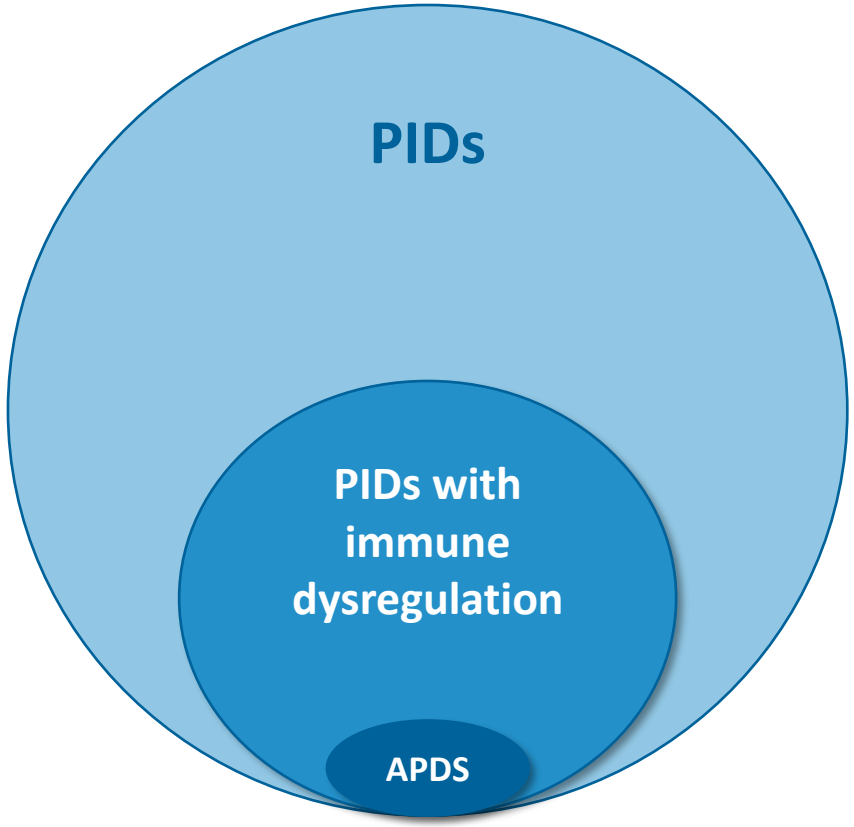
** Anticipate regulatory action in 2024 for Canada and in 2025 for Australia

Primary Immunodeficiencies (PIDs) with immune dysregulation

PIDs are a broad group of disorders[1] with key features:

- Genetic basis, i.e., not secondarily caused by another disease
  'Inborn Errors of Immunity' (IEI) is used interchangeably with PID
- An increased risk of infection may be the predominant manifestation, due to poor immune system function
- PID patients may have a predominance of **immune dysregulation**, for example: lymphoproliferation and autoimmunity[2]

APDS is an example of a PID with immune dysregulation



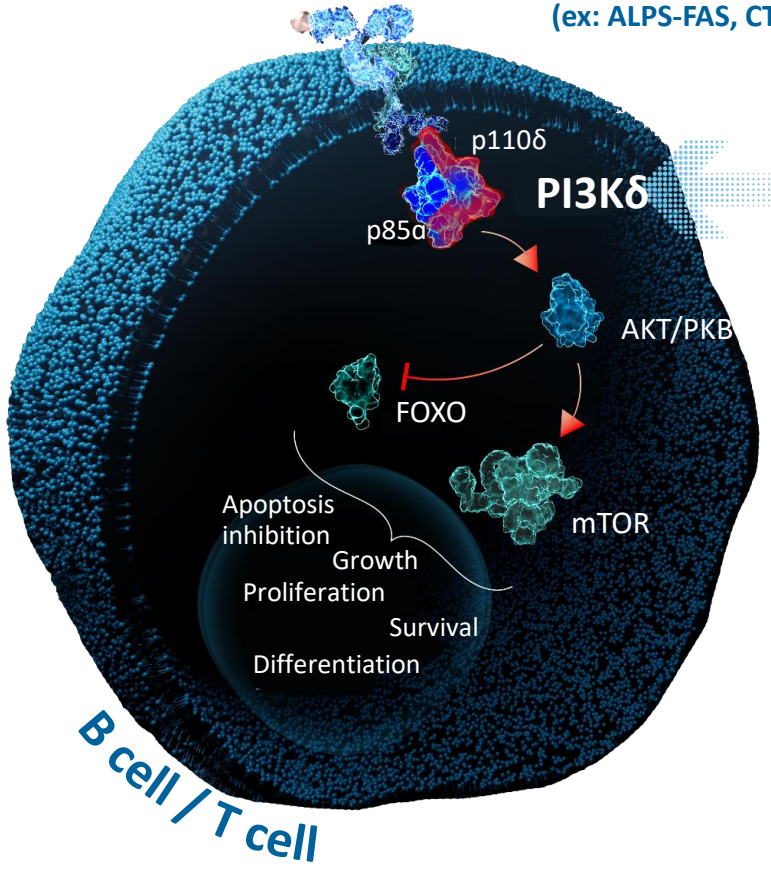
Not to scale with population sizes

1. Bousfiha et al 2022 IUIS categorization
2. Chan and Torgerson 2020 Curr Opin Allergy Clin Immunol 20(6): 582-590

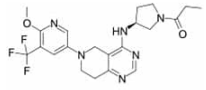
Given importance of PI3Kδ in B & T cells, immune dysregulation in PIDs can occur via alterations in PI3Kδ signaling

Altered PI3Kδ signaling can occur in multiple PID genetic disorders beyond APDS

(ex: ALPS-FAS, CTLA4, PTEN) [1-4]



leniolisib



High unmet medical need

- no approved therapies other than Joenja® (leniolisib) for APDS:

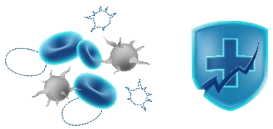
SOC immunosuppressives (e.g. rapamycin) have limited efficacy and significant tolerability concerns

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

Clinical manifestations, disease onset and severity similar to APDS [5-8]

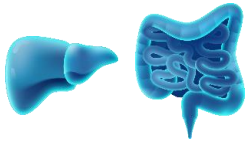
Lymphoproliferation

- Lymphadenopathy
- Splenomegaly/hepatomegaly
- Nodular lymphoid hyperplasia



Autoimmunity

- Cytopenias
- Autoimmune disorders
- Autoinflammation



GI Disease

- Autoimmune enteropathy
- Nodular regenerative hyperplasia

Pulmonary Disease

- GLILD
- Bronchiectasis

Infections

- Sinopulmonary
- Herpesvirus

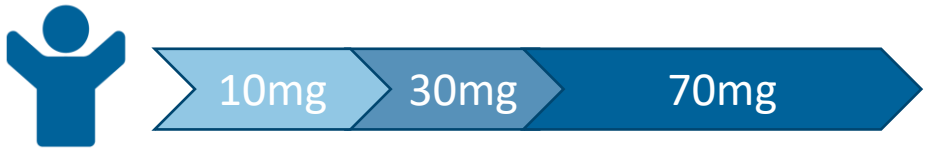
Lymphoma

FOXO, forkhead box O; mTOR, mammalian target of rapamycin; PI3Kδ, phosphoinositide 3-kinase delta; PKB, protein kinase B.

1. Volkl et al. Blood 2016; 128(2):227-238. 2.Tsujita, et al. J Allergy Clin Immunol. 2016;138(6):1872-80. 3. Browning et al. J Med Genet. 2015;52(12):856-59. 4. Heindl et al. Gastroenterology 2012;142:1093-96. 5. Coulter TI, et al. *J Allergy Clin Immunol*. 2017;139(2):597-606. 6. Rao VK and Oliveria JB. Blood 2011; 118(22):5741-51. 7. Westerman-Clark et al 2021; Schwab C, Gabrysch A, Olbrich P, Patiño V, Warnatz K, et al. J Allergy Clin Immunol. 2018;142(6):1932-1946. 8. Eissing M, Ripken L, Schreibelt G, Westdorp H, Ligtenberg M, Netea-Maier R, Netea MG, de Vries IJM, Hoogerbrugge N. Transl Oncol. 2019;12(2):361-367

PIDs linked to PI3Kδ signaling – Phase II study design

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



- Patients with PIDs linked to PI3Kδ signaling, e.g. ALPS-FAS[1], CTLA4 haploinsufficiency[2], PTEN deficiency[3]
- Primary: Safety & Tolerability
- Secondary/Exploratory: PK/PD, efficacy measures
- 10/30/70 mg: 4/4/12 wks treatment, respectively
- Pick Best Dose regimen for Ph3

NIH 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)

1. Rao VK and Oliveria JB. How I treat autoimmune lymphoproliferative syndrome. Blood 2011; 118(22):5741-51
2. Westerman-Clark et al 2021; Schwab C, Gabrysch A, Olbrich P, Patiño V, Warnatz K, et al. Phenotype, penetrance, and treatment of 133 cytotoxic T-lymphocyte antigen 4-insufficient subjects. J Allergy Clin Immunol. 2018;142(6):1932-1946
3. Eissing M, Ripken L, Schreibelt G, Westdorp H, Ligtenberg M, Netea-Maier R, Netea MG, de Vries IJM, Hoogerbrugge N. PTEN Hamartoma Tumor Syndrome and Immune Dysregulation. Transl Oncol. 2019;12(2):361-367

PIDs linked to PI3Kδ signaling – patient prevalence

Epidemiology of PIDs linked to PI3K signaling suggests treatable population of ~5/million[1]

Patients identified to date included in table below

Genetic PID Type	Publication/cohort/registry	Cohort Size
ALPS-FAS	NIH protocol cohort ESID registry[2] Price et al 2014[3]	~500 236 150
CTLA4	Egg et al 2022[4] Schwab et al 2018[5] NIH protocol cohort ESID registry[2]	173 133 ~100 38
PTEN	All PTEN PID patients reported across publications	~88 [6]

1. Estimate of 5 patients per million is based on Pharming literature review, KOL feedback and review of patient registries. Estimate based on proportion of ALPS-FAS and CLTA4 haploinsufficiency patients deemed to be candidates for treatment.
2. Thalhammer et al J Allergy Clin Immunol 2021;148:1332-41
3. Price et al. Blood. 2014;123:1989-1999
4. Egg et al. J Allergy Clin Immunol 2022;149:736-746
5. Schwab et al. J Allergy Clin Immunol 2018;142:1932-1946
6. PTEN PID patient number tabulation from Pharming unpublished literature review completed Feb 2023. Patients may be double counted if reported in more than 1 publication.

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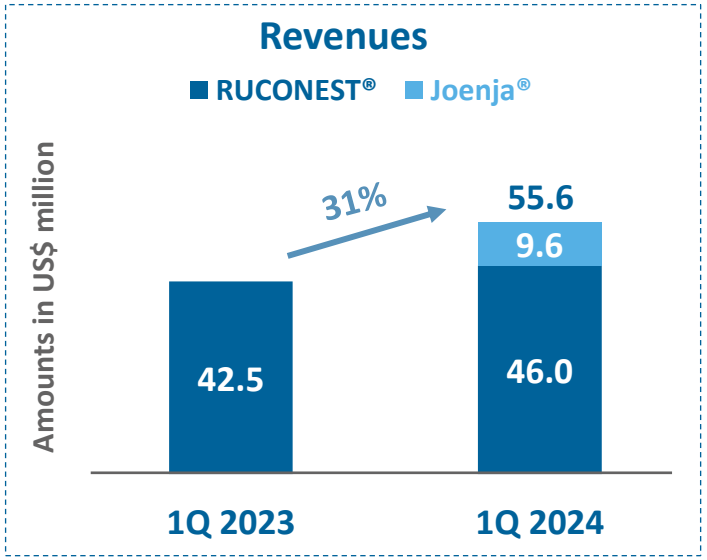
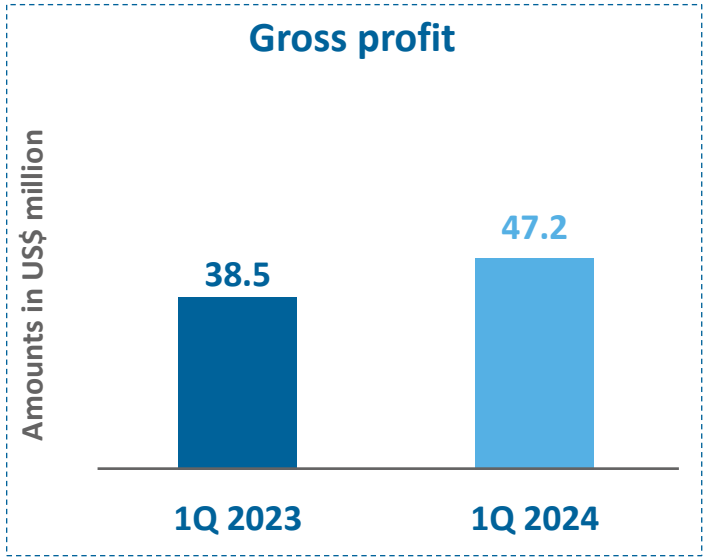
CFO

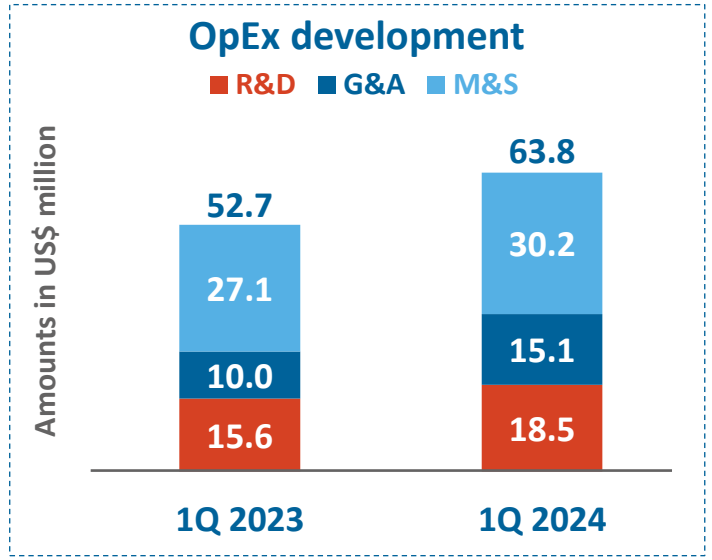
Jeroen Wakkerman
Chief Financial Officer

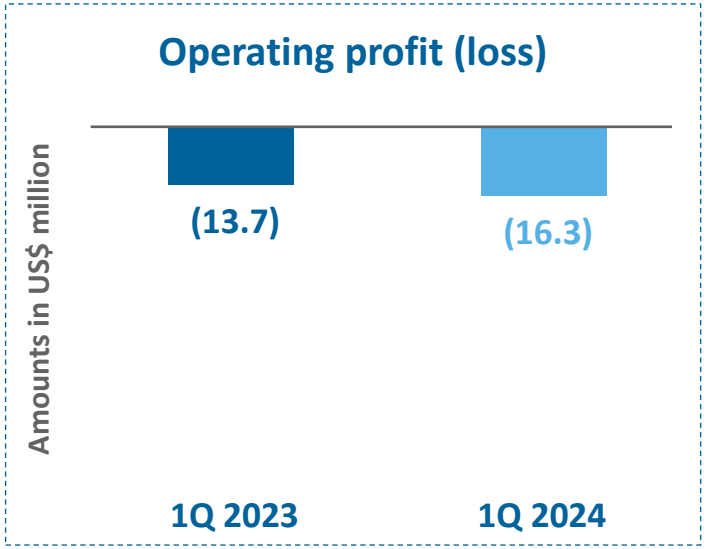
Financials

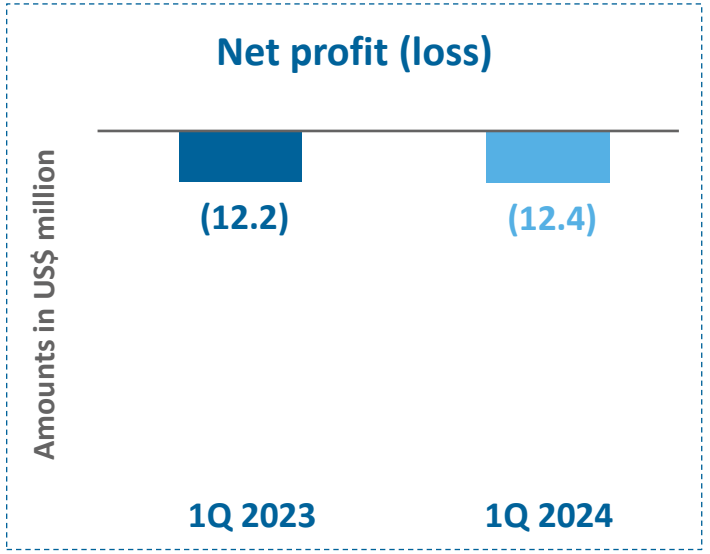
Financial highlights: 1Q 2024 vs 1Q 2023







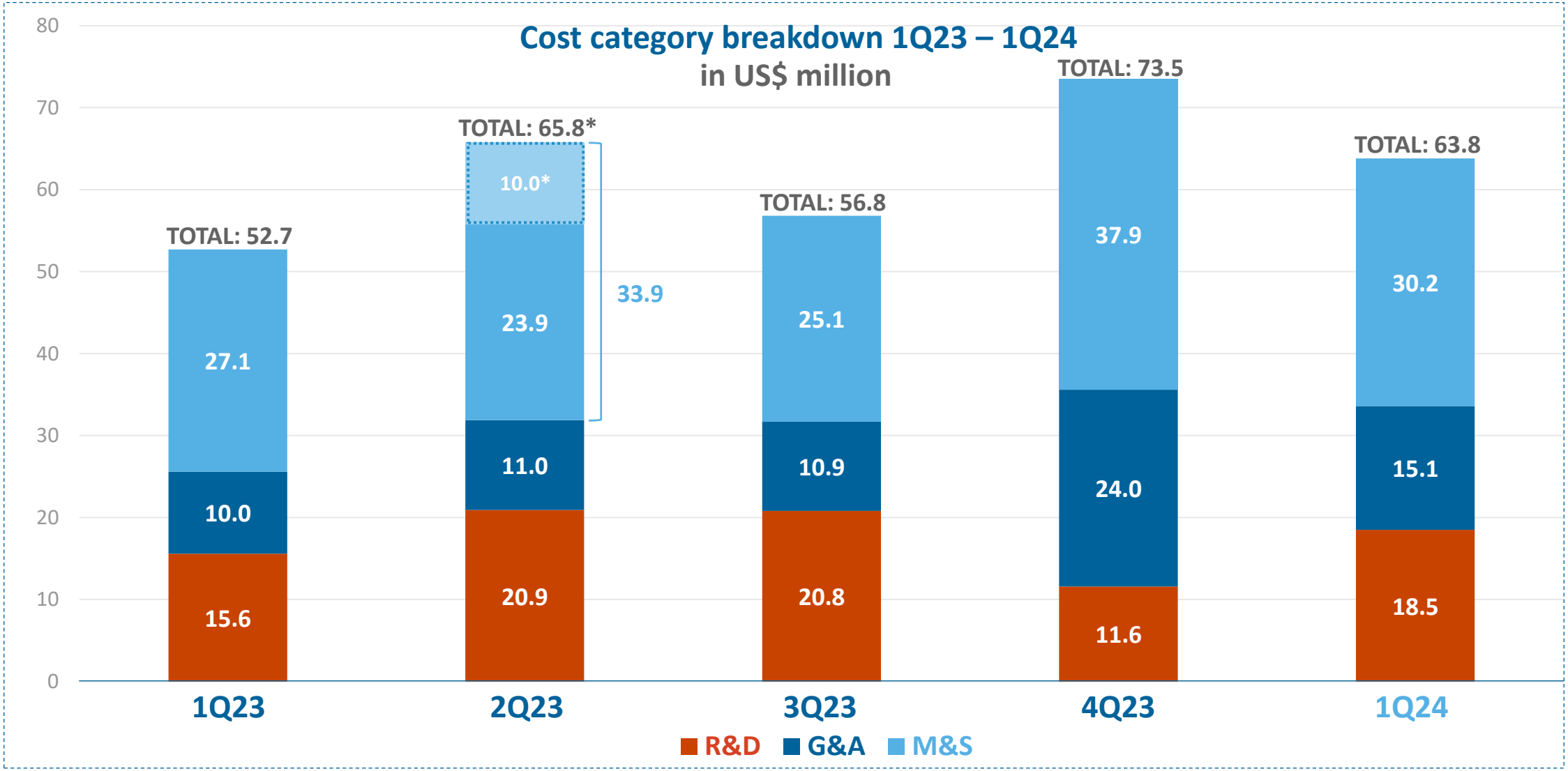




Revenue breakdown by product and geographic segment

Amounts in US$ millions	1Q 2024			1Q 2023		
	RUCONEST®	Joenja®	Total	RUCONEST®	Joenja®	Total
Revenues						
US	44.8	8.5	**53.3**	40.9	-	**40.9**
Europe and RoW	1.2	1.1	**2.3**	1.6	-	**1.6**
Total Revenues	**46.0**	**9.6**	**55.6**	**42.5**	**-**	**42.5**

Investment in Joenja® launch and leniolisib development



*2Q23 marketing and sales expenses includes US$10M milestone payments paid

Pharming 2024 Outlook

- Total revenues between US$280 and US$295 million (14% to 20% growth), with quarterly fluctuations expected.
- 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 from commercial availability or 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, the U.K., Canada and Australia.
- Initiate and advance a Ph II clinical trial for leniolisib in PIDs with immune dysregulation linked to PI3Kδ 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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Q&A

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

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Appendix

Statement of profit and loss

Amounts in US$ '000	1Q 2024	1Q 2023
Revenues	**55,586**	42,541
Costs of sales	**(8,386)**	(4,075)
Gross profit	**47,200**	38,466
Other income	**345**	579
Research and development	(18,521)	(15,620)
General and administrative	(15,087)	(9,981)
Marketing and sales	(30,249)	(27,107)
Other Operating Costs	**(63,857)**	**(52,708)**
Operating profit (loss)	**(16,312)**	**(13,663)**
Other finance income	1,779	123
Other finance expenses	(1,556)	(2,795)
Finance result, net	**223**	**(2,672)**
Share of net profits (loss) in associates using the equity method	**(535)**	**(339)**
Profit (loss) before tax	**(16,624)**	**(16,674)**
Income tax credit (expense)	4,176	4,466
Profit (loss) for the period	**(12,448)**	**(12,208)**
Basic earnings per share (US$)	(0.019)	(0.019)
Diluted earnings per share (US$)	(0.019)	(0.019)

Balance sheet – assets

Amounts in US$ '000	March 31, 2024	December 31, 2023
Non-current assets		
Intangible assets	68,299	71,267
Property, plant and equipment	9,013	9,689
Right-of-use assets	22,849	23,777
Long-term prepayments	90	92
Deferred tax assets	35,686	29,761
Investment accounted for using the equity method	1,707	2,285
Investments in equity instruments designated as at FVTOCI	—	2,020
Investment in debt instruments designated as at FVTPL	5,974	6,093
Restricted cash	1,500	1,528
Total non-current assets	**145,118**	**146,512**
Current assets		
Inventories	55,883	56,760
Trade and other receivables	38,697	46,158
Marketable securities	150,078	151,683
Cash and cash equivalents	51,892	61,741
Total current assets	**296,550**	**316,342**
Total assets	**441,668**	**462,854**

Balance sheet – liabilities

Amounts in US$ '000	March 31, 2024	December 31, 2023
Equity		
Share capital	7,681	7,669
Share premium	479,657	478,431
Other reserves	(4,001)	(2,057)
Accumulated deficit	(277,392)	(265,262)
Shareholders' equity	**205,945**	**218,781**
Non-current liabilities		
Convertible bonds	—	136,598
Lease liabilities	28,438	29,507
Total non-current liabilities	**28,438**	**166,105**
Current liabilities		
Convertible bonds	134,889	1,824
Trade and other payables	68,516	72,528
Lease liabilities	3,880	3,616
Total current liabilities	**207,285**	**77,968**
Total equity and liabilities	**441,668**	**462,854**

Cash flow (1/2)

Amounts in $'000	1Q 2024	1Q 2023
Profit (loss) before tax	**(16,624)**	**(16,674)**
Adjustments to reconcile net profit (loss) to net cash used in operating activities:		
Depreciation, amortization, impairment of non-current assets	5,921	2,306
Equity settled share based payments	2,427	1,558
Other finance income	(1,779)	(123)
Other finance expenses	1,556	2,795
Share of net profits in associates using the equity method	535	339
Other	783	(455)
Operating cash flows before changes in working capital	**(7,181)**	**(10,254)**
Changes in working capital:		
Inventories	877	(5,801)
Trade and other receivables	7,461	(5,313)
Payables and other current liabilities	(9,414)	(1,211)
Restricted cash	28	117
Total changes in working capital	**(1,048)**	**(12,208)**

Cash flow (2/2)

Amounts in $'000	1Q 2024	1Q 2023
Interest received	582	117
Income taxes received (paid)	—	(440)
Net cash flows generated from (used in) operating activities	**(7,647)**	**(22,785)**
Capital expenditure for property, plant and equipment	(80)	(215)
Disposal of investment designated as at FVOCI	1,971	—
Purchases of marketable securities	(94,778)	—
Proceeds from sale of marketable securities	93,551	—
Net cash flows generated from (used in) investing activities	**664**	**(215)**
Payment of lease liabilities	(1,324)	(1,312)
Interests on convertible bonds	(2,031)	(2,013)
Settlement of share based compensation awards	884	695
Net cash flows generated from (used in) financing activities	**(2,471)**	**(2,630)**
Increase (decrease) of cash	**(9,454)**	**(25,630)**
Exchange rate effects	(395)	3,068
Cash and cash equivalents at January 1	61,741	207,342
Total cash and cash equivalents at March 31	**51,892**	**184,780**