Pharming Group N.V.

Second quarter and first half 2024 financial results and business update

August 1, 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

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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

- Revenue FY23 US$227.1M
  2Q24 US$63.0M (+23% vs. 2Q23)
  1H24 US$109.0M (+16% vs. 1H23)
- 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
  2Q24 US$11.1M (+16% vs. 1Q24)
  1H24 US$20.7M (+44% vs. 2H23)
- Strong focus on patient finding
- Israel approval
- 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

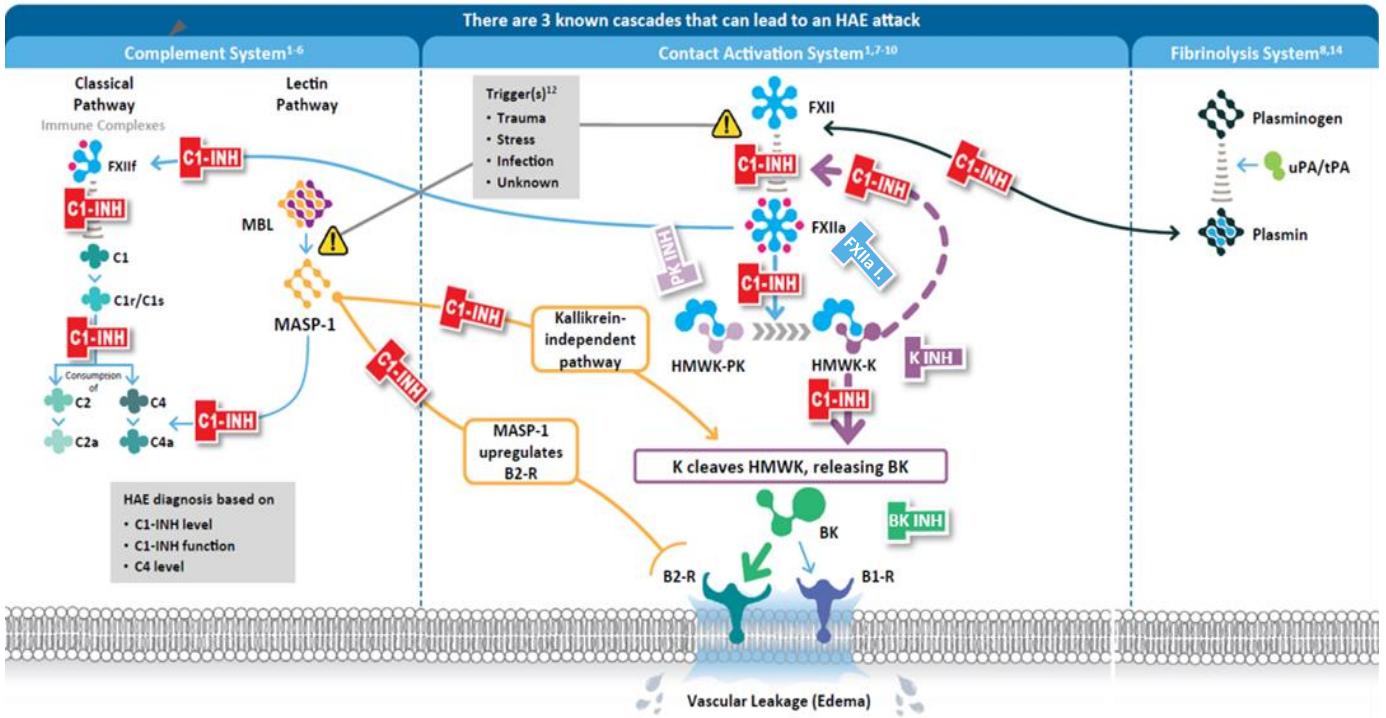
- Leniolisib development for PIDs with immune dysregulation beyond APDS – preparing Ph2
- BD focus on clinical programs in immunology, hematology, respiratory and gastroenterology

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

Disease overview

TARGET	BRAND NAME	GENERIC NAME	STATUS	TYPE
C1 Inhibitor	Ruconest	C1 esterase inhibitor (recombinant)	Marketed	OD
	Berinert	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	deucrictibant (PHVS416)	Phase 3	OD
	n/a	deucrictibant (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.

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 • Growth potential from patient finding and VUS efforts	• Patients on early access/ named patient programs • Global expansion / regulatory reviews • Pediatric studies / label expansion	• Phase II POC trial in PIDs with immune dysregulation linked to PI3Kδ signaling • Symptoms similar to APDS • Seeking regulatory feedback on third PID indication

- APDS global prevalence:
  ~1.5 patients / million
  ~2,400 patients
- PIDs with immune dysregulation (PI3Kδ) global prevalence:
  ~5 patients / 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
- 2nd most prescribed product for acute attacks
  Typical patient: failed icatibant (BK inh) and on prophy Tx (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 days[2]
- Strong U.S. in-market demand –
  New enrollments up 25% in FY23
  Over 100 enrollments in 2Q24 (vs. almost 70 in 1Q24)
- Performing well in leading U.S. revenue indicators: active patients, vials shipped, physicians prescribing (765, +36 vs. 2023)
- Revenue:
  FY23 US$227.1M (+10%)
  2Q24 US$63.0M (+23%)
  1H24 US$109.0M (+16%)
- 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 15 months into U.S. launch
- Continue to enroll and add patients on paid therapy in 2Q24
  91 patients on paid therapy at end 2Q24, with 2 additional enrollments pending authorization
- 2Q24 revenue US$11.1M (+16% vs. 1Q24)
  Includes US$0.9 M Europe and RoW
- 1H24 revenue US$20.7M (+44% vs. 2H23)
  Includes US$2.0M Europe and RoW
- ~500 APDS patients in the U.S.* with >230 diagnosed as of June 30, 2024
  +10 diagnosed patients in 2Q24, 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 >1200 patients identified in the U.S. with VUSs

* Prevalence estimated at 1.5 patients per million population, based on available literature
As of June 30, 2024, Pharming has identified >870 diagnosed APDS patients in global markets
>780 of these patients are in key global launch markets in the U.S., Europe, the U.K., Japan, Asia Pacific, Middle East, Latin America and Canada with total prevalence of ~2,400 APDS patients

Joenja® (leniolisib) franchise – strong 3-5 year growth potential

Joenja® U.S. (APDS)	Leniolisib (APDS)	Leniolisib for Primary Immunodeficiencies (PIDs)
• Marketed (12+) • Found >230 of ~500 patients • 91 patients on paid therapy / 2 pending • >50 diagnosed patients (12+) not yet enrolled and >50 pediatric • Growth potential from patient finding and VUS efforts	• Found >870 patients globally • Global expansion / regulatory reviews • Pediatric studies / label expansion (>25% patients) • 150 patients in EAP, clinical studies, and NPP	• Phase II POC trial in PIDs with immune dysregulation linked to PI3Kδ signaling • Similar to APDS • Seeking regulatory feedback on third PID indication

Prevalence: **~1.5 / million ~2,400 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, Latin America 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®: first and only approved therapy for APDS, corrects the underlying immune defect

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

APDS is a complex syndrome caused by pathogenic variants of the PI3Kδ enzyme, with significant mortality

Joenja® is an oral, selective PI3Kδ inhibitor designed to help regulate the hyperactive signaling pathway

FDA approval (March 2023) based on randomized pivotal study and OLE study
U.S. launch (April 2023)

Joenja® is an oral immune modulator targeting the root cause of APDS

- Normalizes the hyperactive PI3Kδ pathway to correct the underlying immune defect in APDS patients
- Helps address both immune deficiency and immune dysregulation

No drug-related serious adverse events or study withdrawals in Joenja® trials
Clinical data and tolerability for long term treatment

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 (>1,200 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.

VUS by the numbers

VUSs frustrate patients and doctors, limiting diagnosis of genetic diseases such as APDS

Pharming is aware of **~1,200 US patients** harboring *PIK3CD/R1* VUSs

- This figure will continue to grow over time
- VUS are identified at ~4x the rate of likely pathogenic/pathogenic (LP/P) variants
- Similar VUS frequencies expected worldwide
- Published literature, which includes more than 1.5 million patients, showed that 20% of reclassified VUSs are upgraded to LP/P
- Pilot study in 25 VUS patient samples - findings consistent with APDS identified in 5 patients (20%) including patient preparing for enrollment

No systemic initiatives exist to resolve *PIK3CD/R1* VUSs, yet these patients remain a significant opportunity to identify incremental patients with APDS

Joenja® – looking beyond FDA approval

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

Israel marketing authorization received April 30, 2024

U.K MHRA decision expected in the fourth quarter 2024*

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

Japan clinical study: Patient enrollment is now complete
- PMDA filing following completion of appropriate clinical trials

Pediatric studies
- 4 to 11 years - Enrollment completed
- 1 to 6 years - 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. Pharming received MHRA Day 70 Request for Further Information on July 3, 2024. There were no major objections. Upon Pharming's satisfactory response to MHRA requests, it is expected that the MHRA will issue its decision in the fourth quarter of 2024.

** Anticipate regulatory action in 2025 for Australia

Leniolisib for PIDs with immune dysregulation

- **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

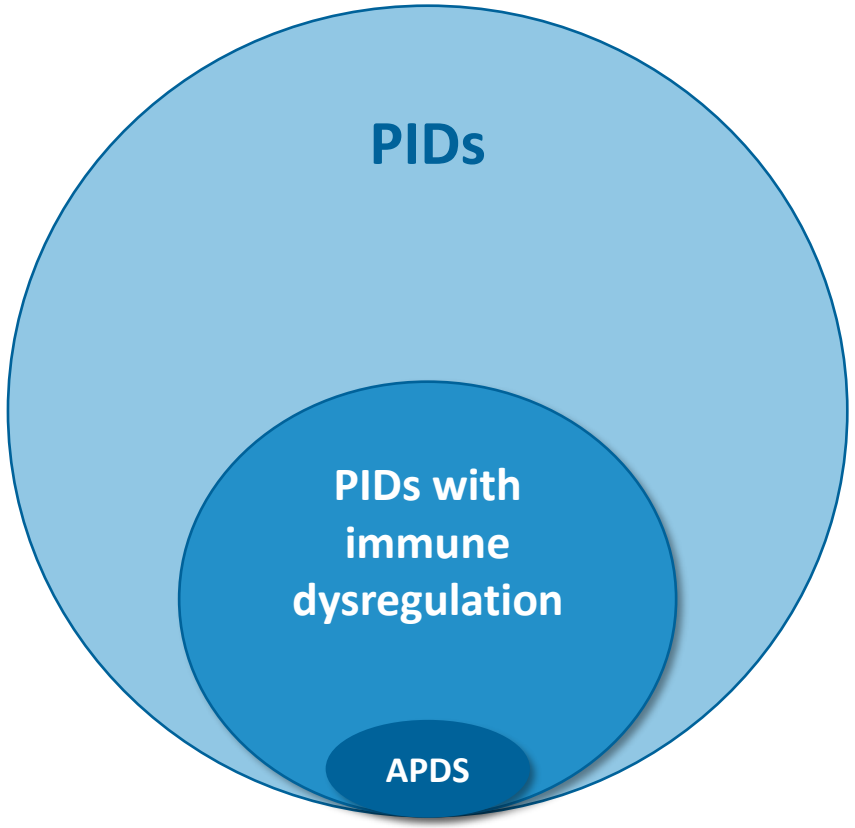
- **Pharming developing leniolisib for PIDs with immune dysregulation beyond APDS**

PIDs with immune dysregulation linked to PI3Kδ signaling

- Multiple PIDs with alterations in PI3Kδ signaling
- Clinical manifestations, disease onset and severity similar to APDS
- No approved therapies
- Phase II proof of concept clinical trial starting shortly

Next indication

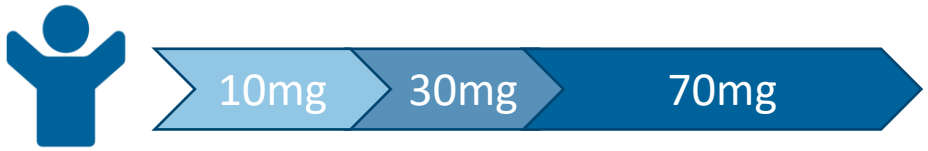
- Obtaining regulatory feedback on proposed clinical development plan



Not to scale with population sizes

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] (treatable population ~5/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)

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

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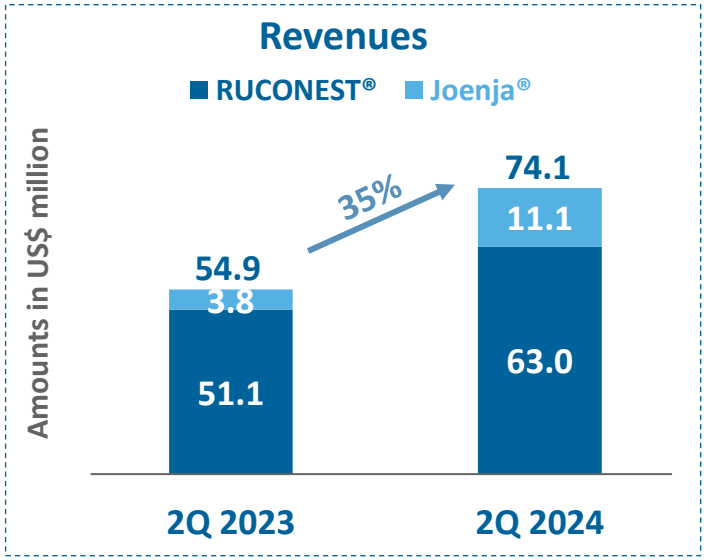
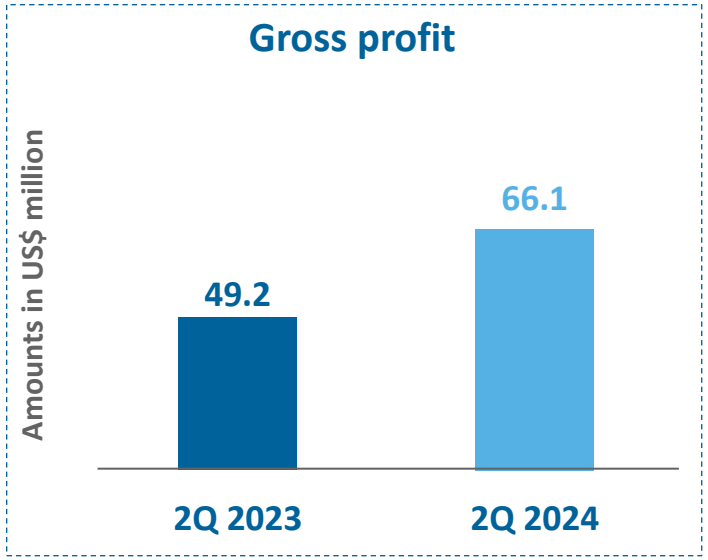
CFO

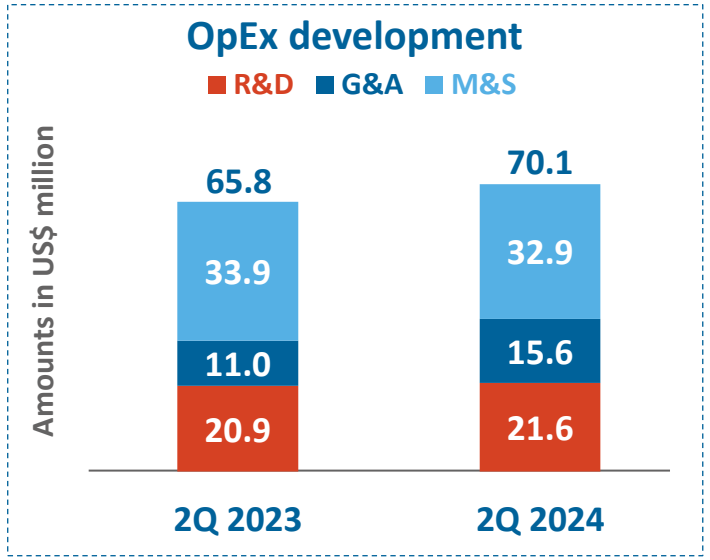
Jeroen Wakkerman
Chief Financial Officer

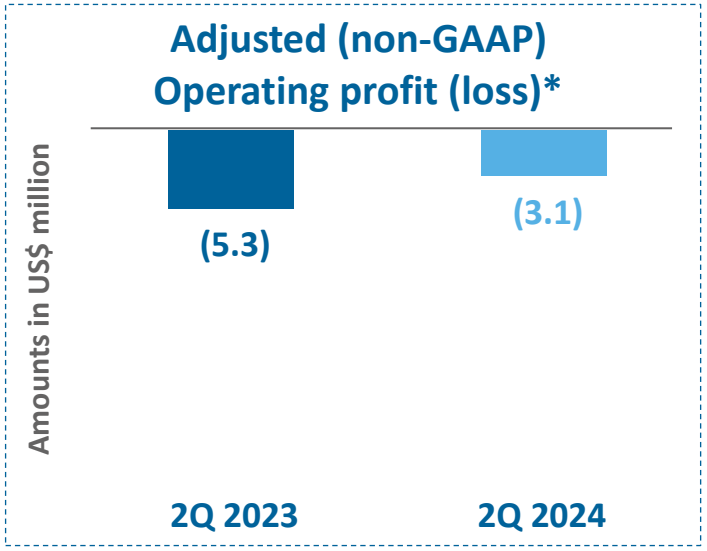
Financials

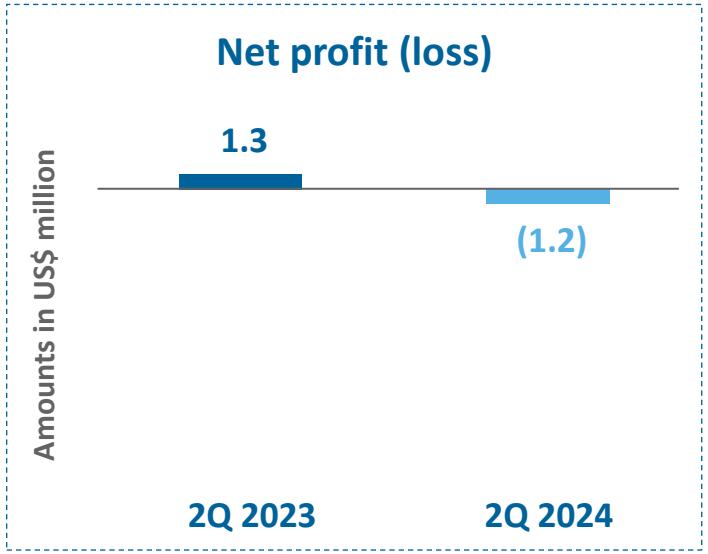
Financial highlights: 2Q 2024 vs 2Q 2023

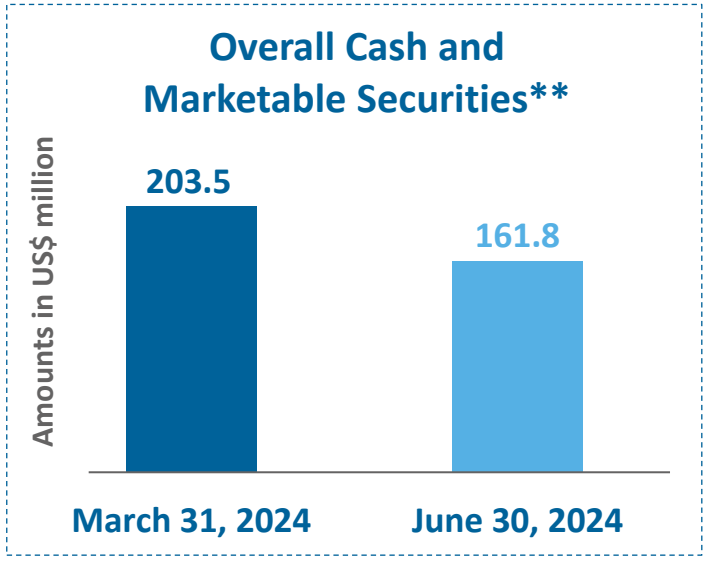






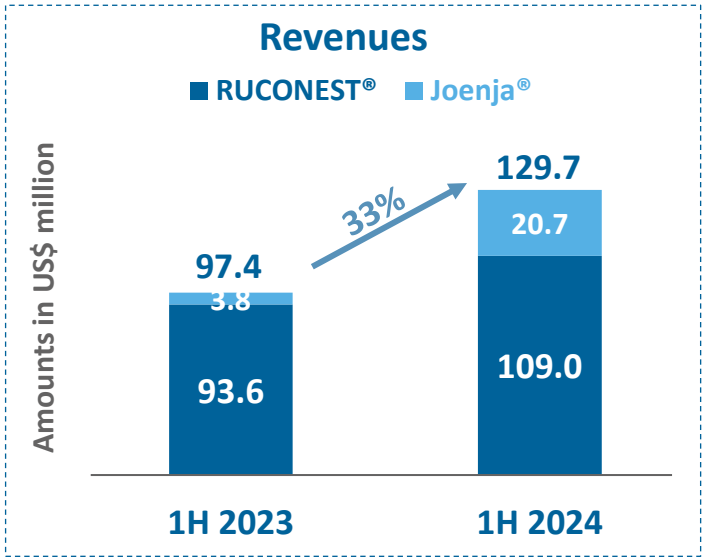


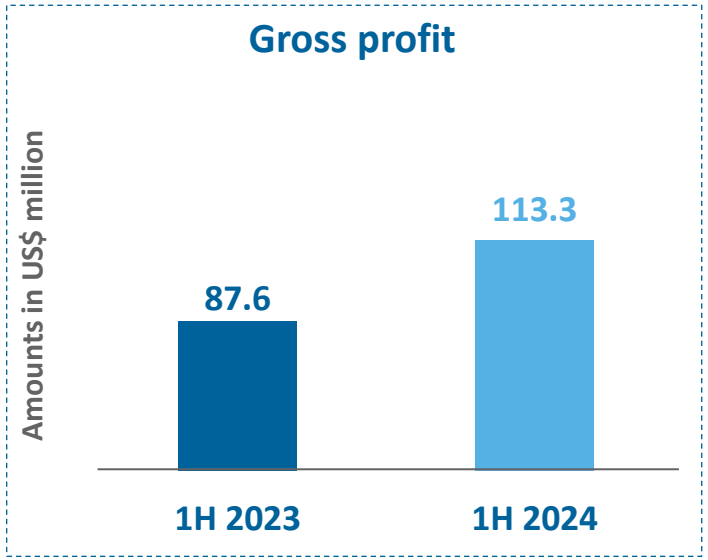


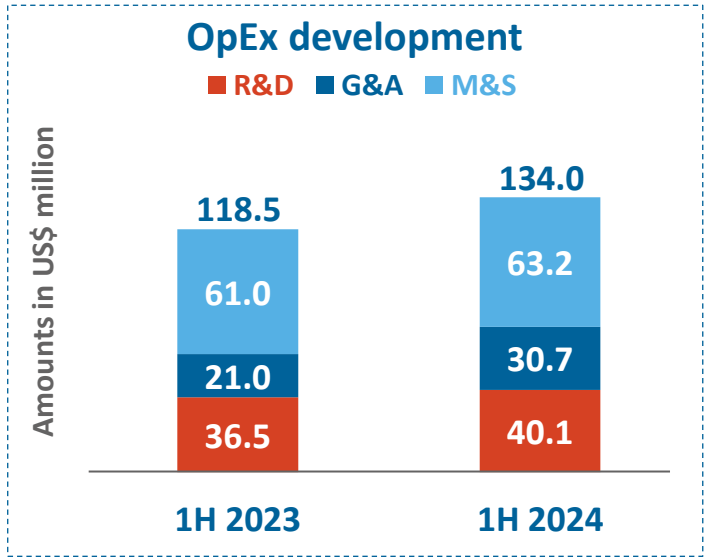


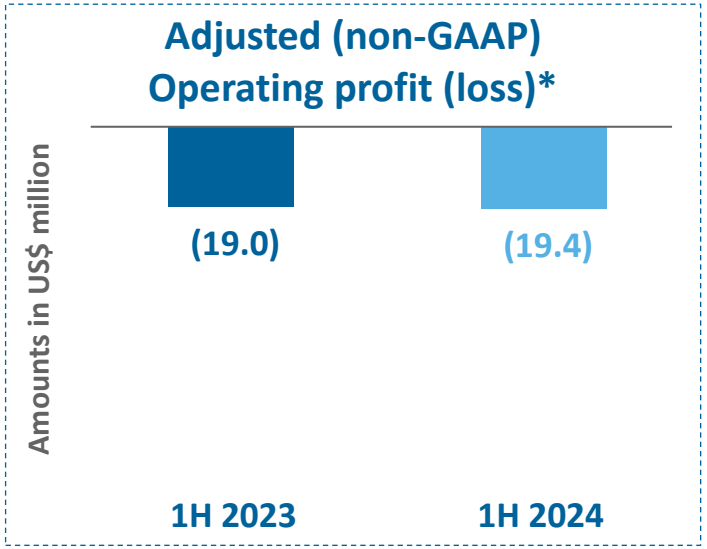
* Operating profit (loss) for 2Q 2023 excludes milestone payments for Joenja® (US$10.5 million) and gain on sale of Priority Review Voucher to Novartis (US$21.1 million).
** Of the US$41.7 million decrease in overall cash and marketable securities, US$30.1 million is due to convertible bond refinancing and US$12.4 million due to increase in receivables.

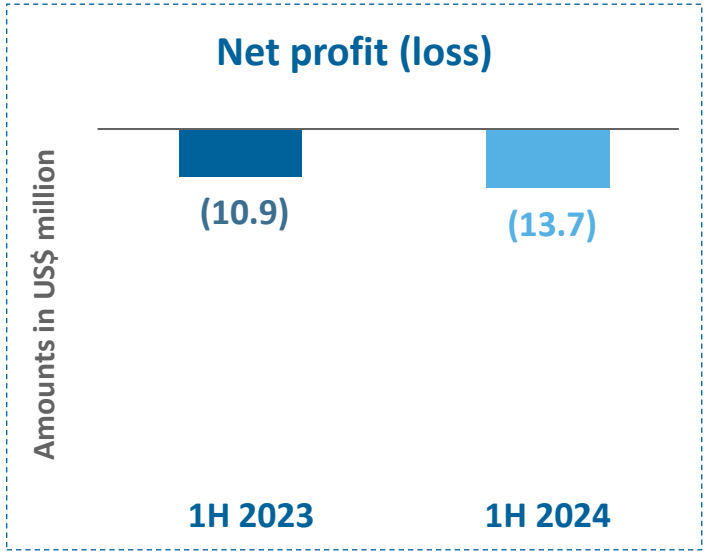
Financial highlights: 1H 2024 vs 1H 2023











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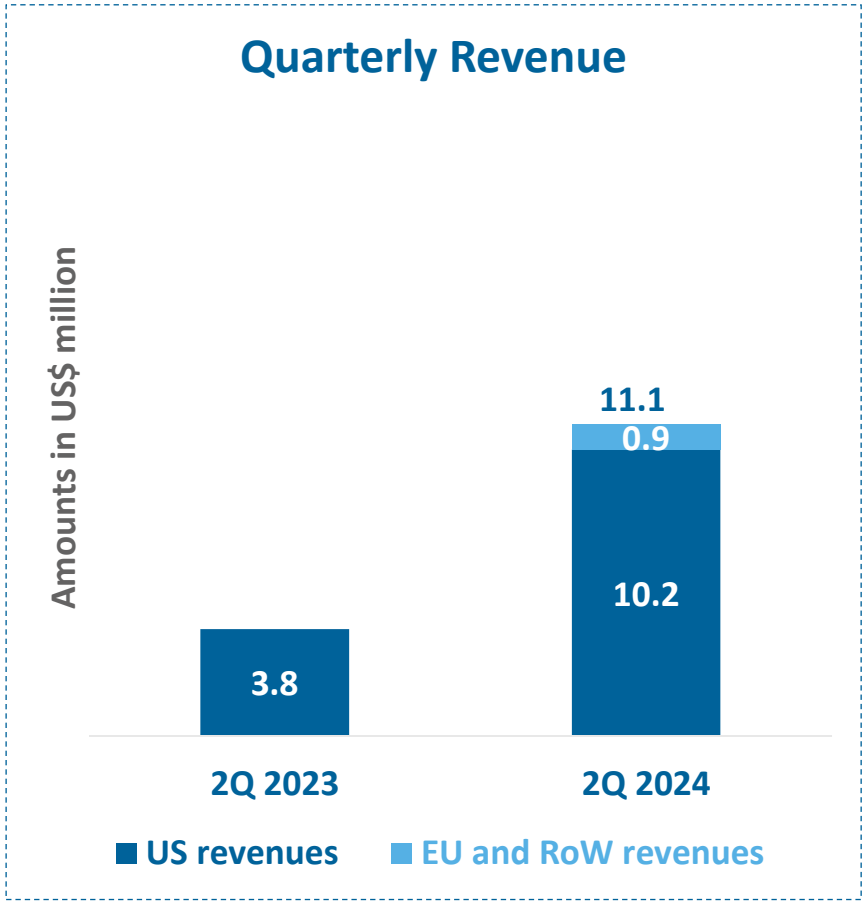
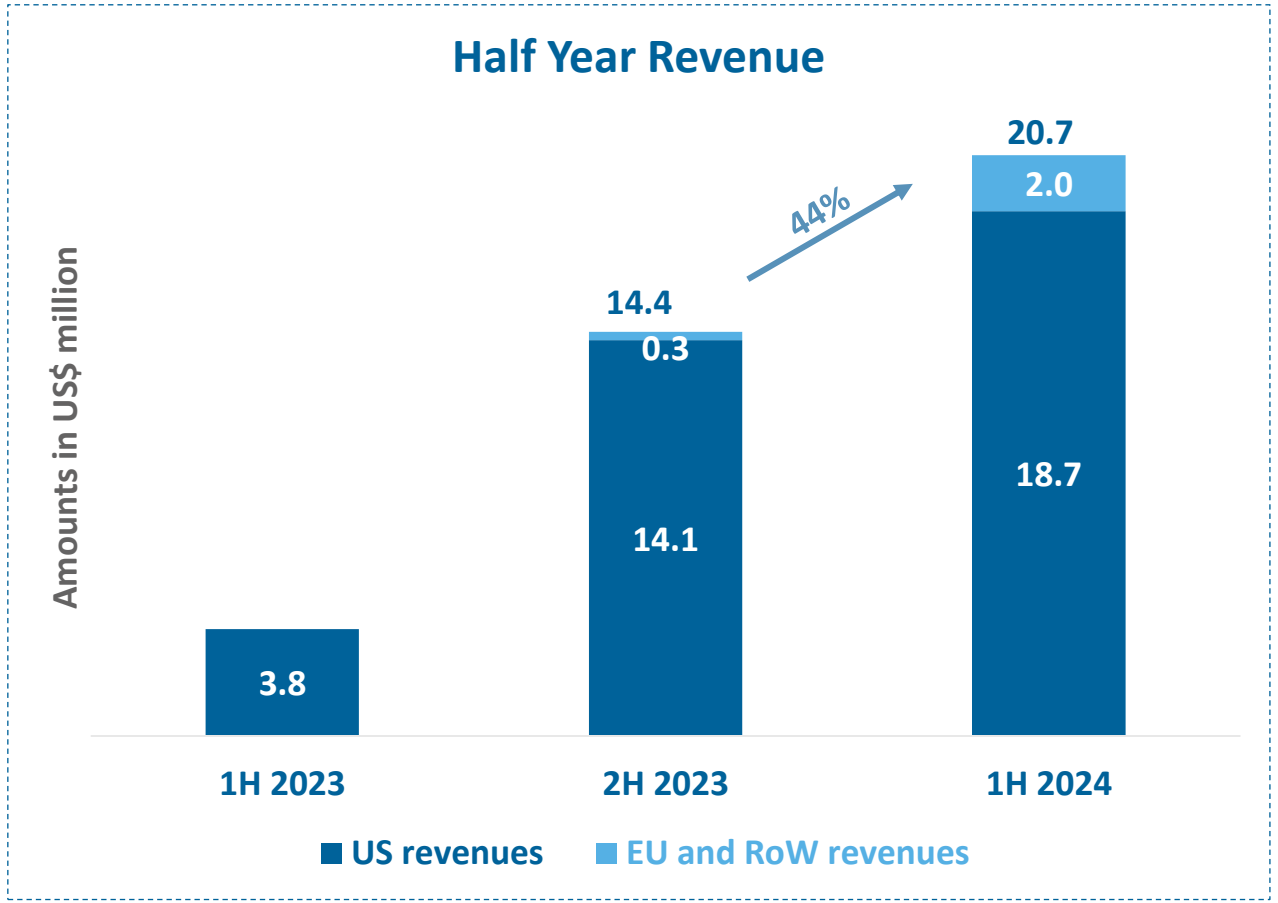
** US$30.1 million of the US$53.2 million decrease in overall cash and marketable securities is due to convertible bond refinancing.

Joenja® revenue breakdown





2024 Financial guidance

	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 adherence (compliance) rates ~85%
  - U.S. Pricing: 30-day supply $47,220, Annual cost (WAC) $566,640, GTN Discount ~15%
- 2H 2024 OpEx – adjustments / savings due to EMA delay

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	notes	1H 2024	1H 2023
Revenues	7	**129,679**	**97,438**
Costs of sales	9	**(16,367)**	**(9,799)**
Gross profit	7	**113,312**	**87,639**
Other income	8	**1,257**	**22,507**
Research and development		(40,118)	(36,534)
General and administrative		(30,707)	(20,963)
Marketing and sales		(63,177)	(61,013)
Other Operating Costs	9	**(134,002)**	**(118,510)**
Operating profit (loss)		**(19,433)**	**(8,364)**
Fair value gain (loss) on revaluation	18	5,138	—
Other finance income	10	2,935	799
Other finance expenses	10	(4,490)	(5,254)
Finance result, net		**3,583**	**(4,455)**
Share of net profits (loss) in associates using the equity method	12	**(834)**	**(469)**
Profit (loss) before tax		**(16,684)**	**(13,288)**
Income tax credit (expense)	11	3,018	2,399
Profit (loss) for the period		**(13,666)**	**(10,889)**
Basic earnings per share (US$)	19	(0.020)	(0.017)
Diluted earnings per share (US$)	19	(0.020)	(0.017)

Balance sheet – assets

Amounts in US$ '000	notes	June 30, 2024	December 31, 2023
Non-current assets			
Intangible assets		66,572	71,267
Property, plant and equipment		8,617	9,689
Right-of-use assets		22,107	23,777
Long-term prepayments		90	92
Deferred tax assets	13	39,049	29,761
Investment accounted for using the equity method	12	1,404	2,285
Investments in equity instruments designated as at FVTOCI	12	—	2,020
Investment in debt instruments designated as at FVTPL	12	5,959	6,093
Restricted cash	16	1,498	1,528
Total non-current assets		**145,296**	**146,512**
Current assets			
Inventories	14	59,190	56,760
Trade and other receivables		51,119	46,158
Marketable securities	15	113,181	151,683
Cash and cash equivalents	16	47,142	61,741
Total current assets		**270,632**	**316,342**
Total assets		**415,928**	**462,854**

Balance sheet – liabilities

Amounts in US$ '000	notes	June 30, 2024	December 31, 2023
Equity			
Share capital		7,748	7,669
Share premium		486,850	478,431
Other reserves		6,390	(2,057)
Accumulated deficit		(280,051)	(265,262)
Shareholders' equity	17	**220,937**	**218,781**
Non-current liabilities			
Convertible bonds	18	87,323	136,598
Lease liabilities		27,731	29,507
Total non-current liabilities		**115,054**	**166,105**
Current liabilities			
Convertible bonds	18	3,147	1,824
Trade and other payables		72,967	72,528
Lease liabilities		3,823	3,616
Total current liabilities		**79,937**	**77,968**
Total equity and liabilities		**415,928**	**462,854**

Cash flow (1/2)

Amounts in $'000	1H 2024	1H 2023
Profit (loss) before tax	**(16,684)**	**(13,288)**
Adjustments to reconcile net profit (loss) to net cash used in operating activities:		
Depreciation, amortization, impairment of non-current assets	5,628	5,468
Equity settled share based payments	5,687	3,970
Fair value loss (gain) on revaluation	(5,138)	—
Gain on disposal from PRV sale	—	(21,080)
Other finance income	(2,935)	(799)
Other finance expenses	4,450	5,254
Share of net profits in associates using the equity method	834	469
Other	—	(1,743)
Operating cash flows before changes in working capital	**(8,158)**	**(21,749)**
Changes in working capital:		
Inventories	(3,115)	(10,717)
Trade and other receivables	(4,963)	(5,539)
Payables and other current liabilities	(2,255)	4,833
Restricted cash	—	410
Total changes in working capital	**(10,333)**	**(11,014)**
Interest received	2,370	799
Income taxes received (paid)	(4,747)	(442)
Net cash flows generated from (used in) operating activities	**(20,868)**	**(32,406)**

Cash flow (2/2)

Amounts in $'000	1H 2024	1H 2023
Capital expenditure for property, plant and equipment	(294)	(986)
Proceeds on PRV sale	—	21,080
Disposal of investment designated as at FVOCI	1,964	—
Purchases of marketable securities	(112,453)	(87,347)
Proceeds from sale of marketable securities	147,841	—
Net cash flows generated from (used in) investing activities	**37,058**	**(67,253)**
Payment of lease liabilities	(2,093)	(2,570)
Net proceeds of issued convertible bonds	104,802	—
Repurchase of convertible bonds	(134,922)	—
Interests on convertible bonds	(2,024)	(2,023)
Settlement of share based compensation awards	3,462	(666)
Net cash flows generated from (used in) financing activities	**(30,775)**	**(5,259)**
Increase (decrease) of cash	**(14,585)**	**(104,918)**
Exchange rate effects	(14)	2,601
Cash and cash equivalents at the beginning of the period	61,741	207,342
Total cash and cash equivalents at June 30	**47,142**	**105,026**