



## **Pharming Group N.V.**

Second quarter and first half  
2023 financial results

**August 3, 2023**

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



**Sijmen de Vries, MD**  
Chief Executive Officer



**Anurag Relan, MD**  
Chief Medical Officer



**Stephen Toor**  
Chief Commercial Officer



**Jeroen Wakkerman**  
Chief Financial Officer

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**Sijmen de Vries, MD**  
Chief Executive Officer

## Introduction



**Market RUCONEST® in all key international markets – U.S. focus**



**Positive cash flow from RUCONEST® helps fund Joenja® (leniolisib) and pipeline development and management**

- ◆ RUCONEST® returned to revenue growth in 2Q23
- ◆ Continue to be on track for low single digit revenue growth




**Global approvals and commercialization of Joenja® (leniolisib)**



**Successful commercialization of Joenja® (leniolisib) for APDS and additional rare disease indications**

- ◆ MAR: FDA approval for Joenja®  
APR: Strong 2Q start U.S. launch
- ◆ Regulatory reviews ongoing in EUR, CAN, AUS, ISR
- ◆ Pediatric clinical program ongoing



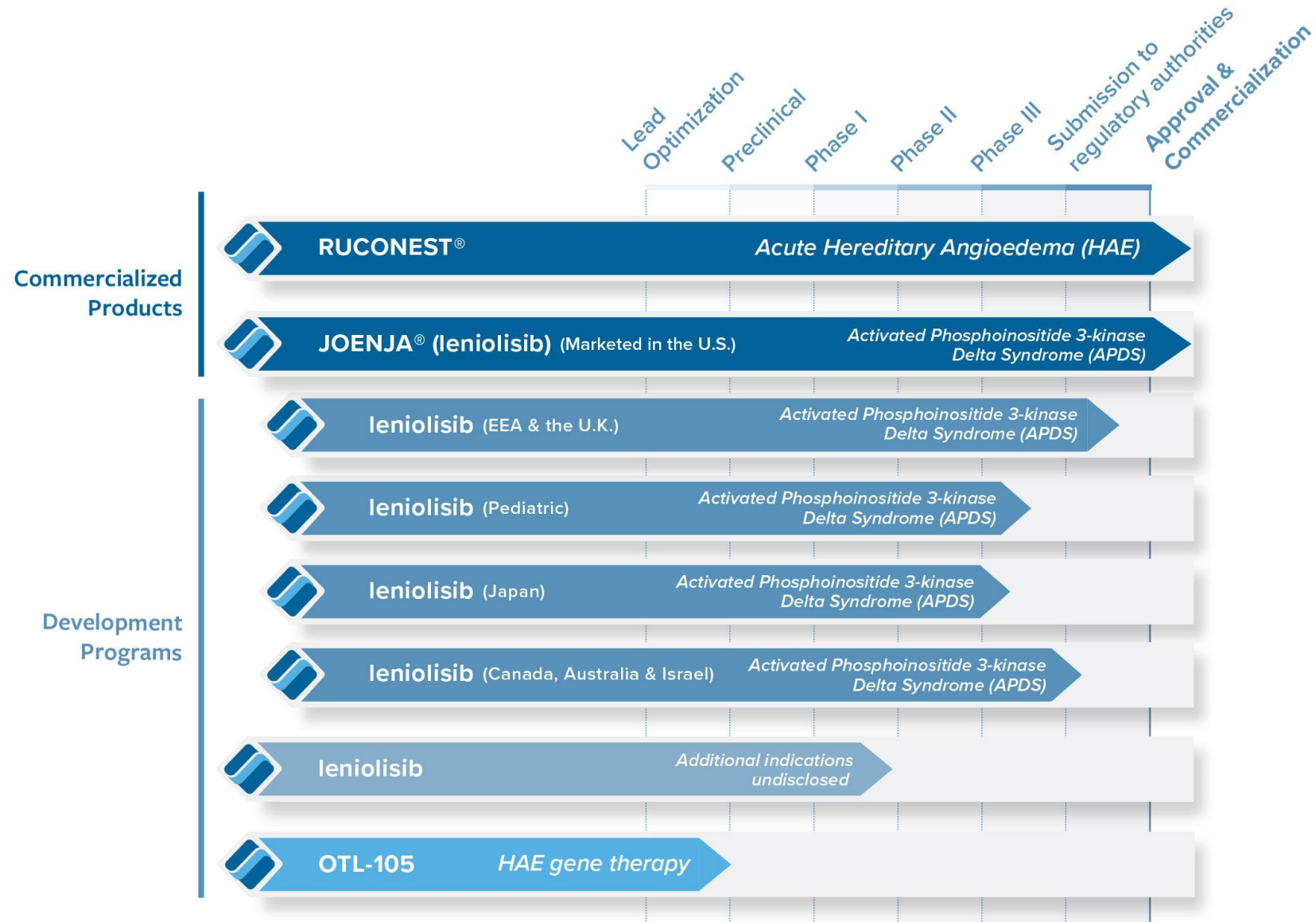
**Ongoing pipeline development and management of rare disease assets**



**Advance internal projects and potential acquisitions of new, mid to late-stage assets through in-licensing and M&A**

- ◆ Advanced 2<sup>nd</sup> indication for leniolisib (2H23 disclosure)
- ◆ Investments and continued focus on in-licensing or acquisitions of mid to late-stage opportunities in rare diseases.

# Pipeline – multiple commercial stage rare disease products



# Pharming strengthens leadership with new Chairman of the Board (nominated) and Chief Business Officer



## Dr. Richard Peters

Nominated as Non-Executive director and new Chairman of the Board, successor to Mr. Paul Sekhri

**Start date:** Upon appointment at upcoming EGM.

**Experience:** Over 30 years of experience in the healthcare industry and academia

**Role:** Chairman of the Board of Directors and Non-Executive Director



## Dr. Alexander Breidenbach, MBA

Chief Business Officer and new Member of the Executive Committee

**Start date:** September 1, 2023

**Experience:** More than 20 years of partnering, R&D and management experience in biosciences.

**Role:** Chief Business Officer (CBO)





**RUCONEST®:**  
**Established commercial business**





Dedicated sales force and marketing in U.S., Europe, and MENA



Market access teams



Patient support and reimbursement teams



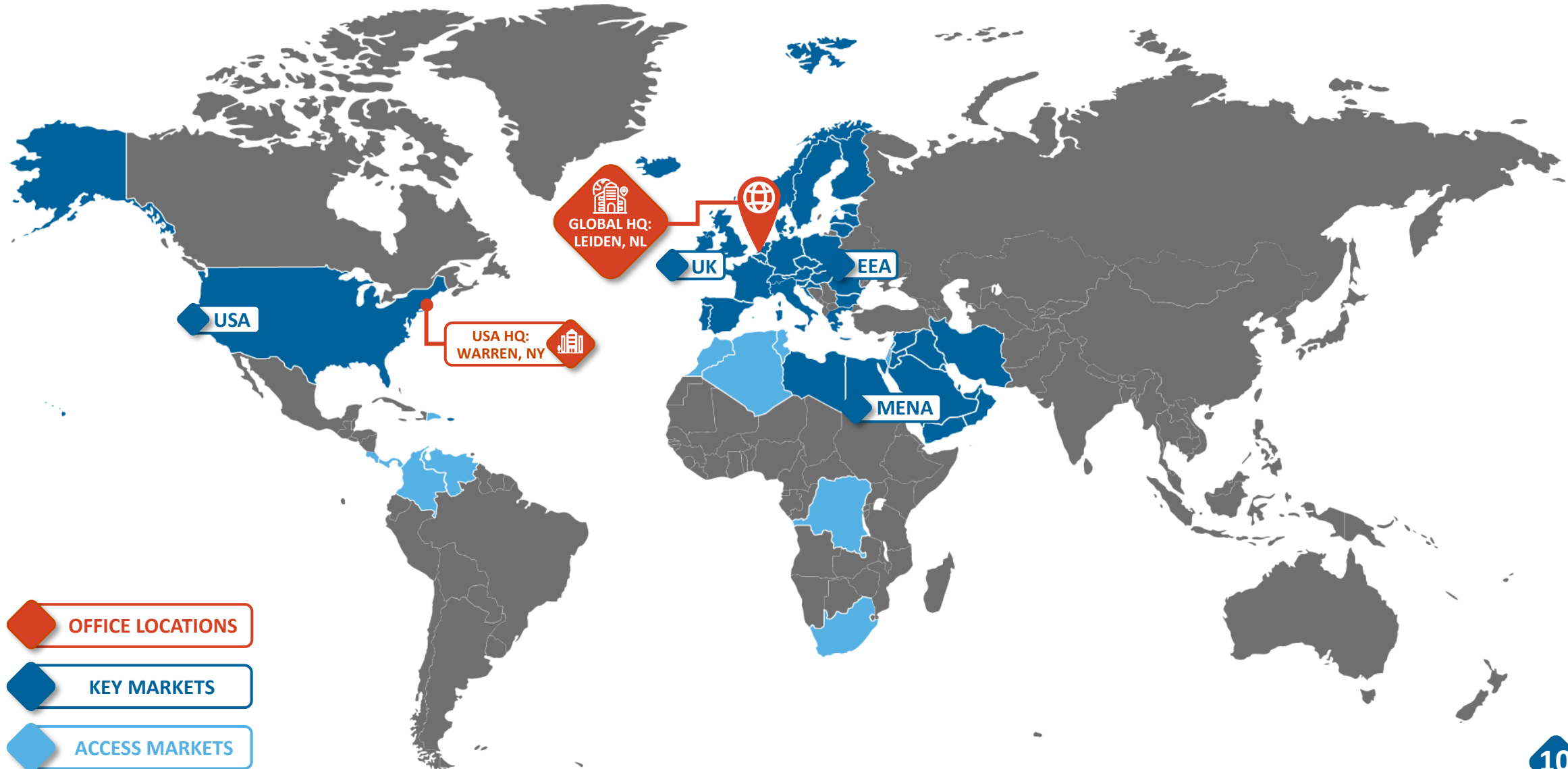
Disease educators and specialists for APDS and HAE



Medical Affairs teams



High conference penetration & Support for educational KOL speaker programs





RUCONEST® sales >US\$200m  
(trailing 12 months)



2Q23: RUCONEST® returned to growth  
Outlook of low single digit revenue growth for 2023



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



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



Second most prescribed product detailed for acute attacks



97% of acute attacks needed just one dose of RUCONEST®<sup>1</sup>



93% of attacks were stopped with RUCONEST® for at least three days<sup>2</sup>



Patients are well managed and feel confident to administer treatment themselves<sup>3</sup>



Pharming® | 35 years



CMO



**Anurag Relan, MD**  
Chief Medical Officer

**APDS**

**Joenja® (leniolisib)**

# APDS is a rare, primary immunodeficiency (PI) first characterized in 2013



Activated phosphoinositide 3-kinase delta (PI3K $\delta$ ) syndrome (APDS) affects >1500 patients\*

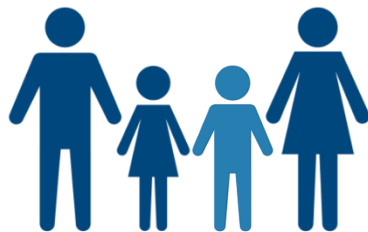
To date, Pharming has identified >640 of these patients in key global markets

(as of June 30, 2023, for U.S., Europe, U.K., Japan, Canada, Australia and Israel)



Until now, treatments for APDS have addressed the symptoms of the disease which manifest early in childhood, but not the root cause of APDS

Without an indicated treatment specifically for APDS, physicians could only manage symptoms



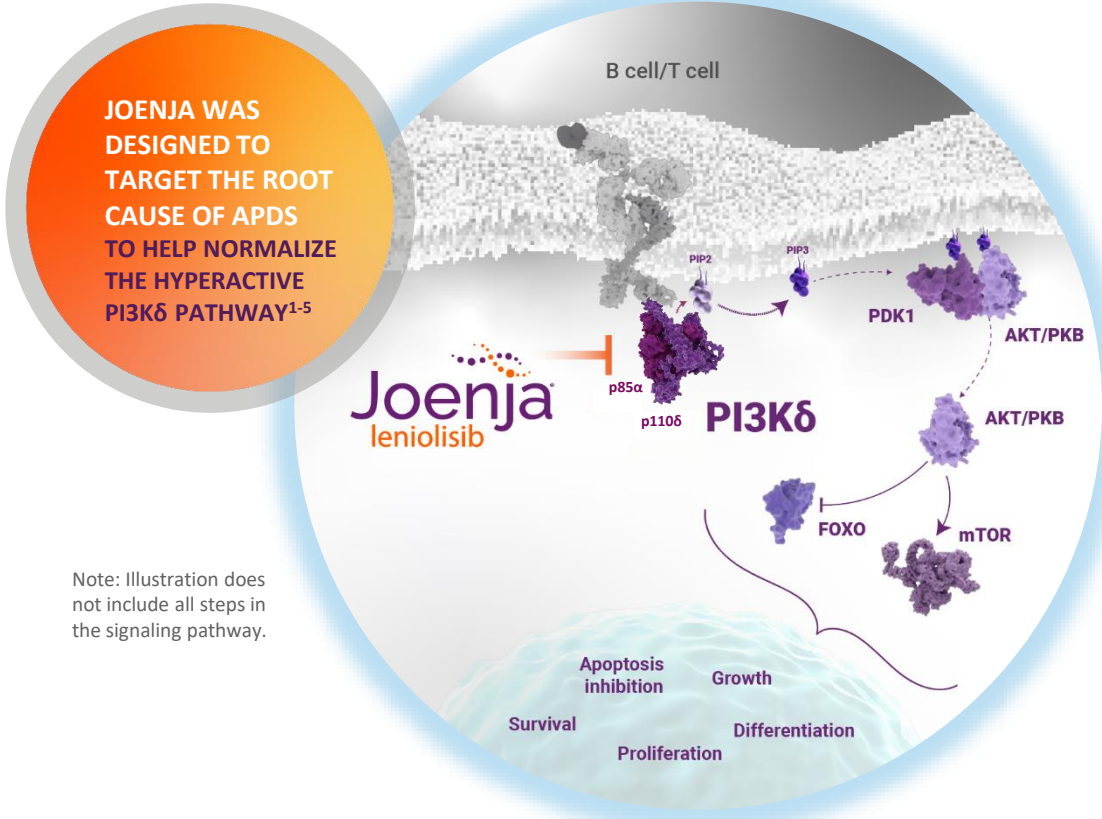
The signs and symptoms of APDS vary widely, even among family members with the same genetic variant, resulting in potential delays in diagnosis and care



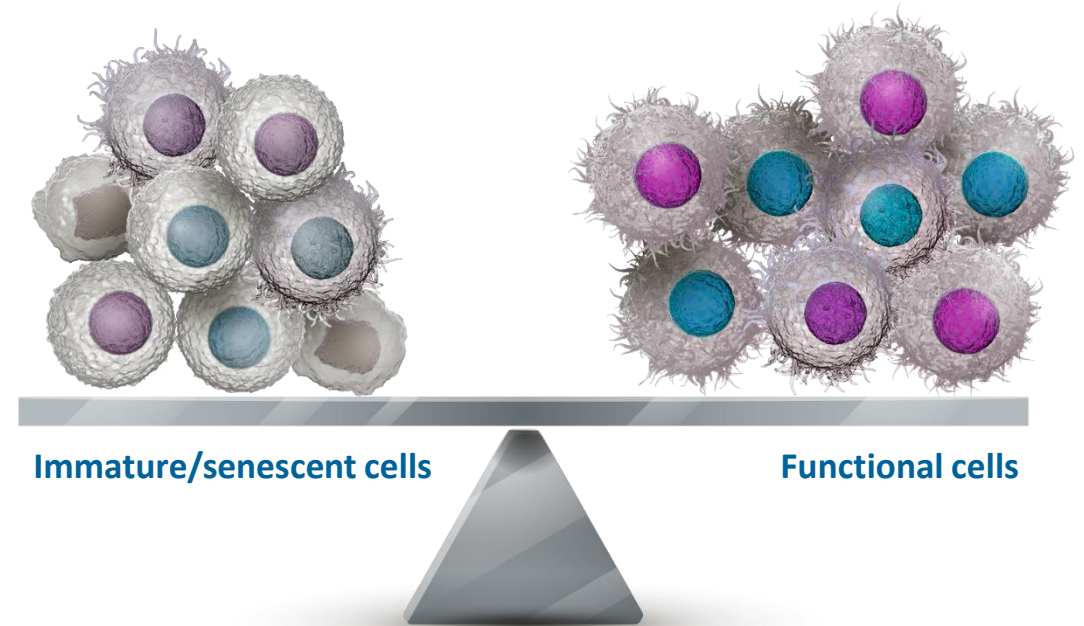
A genetic test can provide a definitive diagnosis of APDS

\*Size based on estimate of 1.5 APDS patients per million (based on available literature) for U.S., Europe, U.K., Japan, Canada, Australia and Israel

# Joenja<sup>®</sup>: immune modulator that targets the root cause of APDS



Joenja<sup>®</sup> facilitates a balanced PI3Kδ pathway to support proper immune function<sup>6</sup>



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.

# FDA approval of Joenja<sup>®</sup>: a much-needed treatment for patients with APDS and another win for Pharming

Joenja<sup>®</sup> (leniolisib) is a prescription medicine that is used to treat activated phosphoinositide 3-kinase delta (PI3K $\delta$ ) syndrome (APDS) in adults and pediatric patients 12 years of age and older

In a randomized placebo-controlled trial of patients with APDS

- Joenja<sup>®</sup> 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<sup>®</sup> trials



Joenja<sup>®</sup> 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

Pharming is off to a strong start with Joenja<sup>®</sup> delivered to patients within 2 weeks of FDA approval

## All patients with IEI/PID

### ~200 patients identified with APDS in the U.S.

- ◆ Disease state awareness
- ◆ Familial testing
- ◆ Educational programs
- ◆ Abstracts and manuscripts
- ◆ Clinician and patient support

### Undiagnosed APDS patients

- ◆ A.I. methods to i.d. APDS patients seeing Immunologists, GI, Heme/Onc, and Pulm providers
- ◆ Comprehensive genetic testing (*navigateAPDS*) and immunophenotyping

### Potential APDS patients with gene VUS

- ◆ Variant of Uncertain Significance (VUS) resolution
  - ◆ Literature mining
  - ◆ Facilitating data sharing among clinical laboratories
  - ◆ Functional testing
  - ◆ Familial testing (de novo, segregation)





Europe – CHMP opinion on MAA expected 4Q23 (approval ~ 2 months later)



UK – MHRA filing expected 4Q23 (approval ~2 months later)



Japan clinical study open for enrollment  
First patient expected in the third quarter



Regulatory submissions filed in additional markets: CAN, AUS, ISR



Named patient program partnership



Pediatric patients enrolling in the 4 to 11 year old study



Progress in identifying additional indications for development of leniolisib beyond APDS.  
More details in 3Q/4Q23



Initiation of second pediatric study in children 1 to 6 years in 3Q23



May: responses submitted for CHMP Day 120 list of questions

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July: receives the CHMP's Day 180 list of outstanding issues

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CHMP will consult an Ad-hoc Expert Group (AEG) given the rarity of the disease and the unmet medical need for the treatment of APDS patients

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AEG will be consulted at a closed meeting also involving Pharming representatives including leniolisib investigators and APDS patients

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CHMP opinion expected in 4Q 2023, with approval following ~2 months later\*

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Pharming® | 35 years



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**Stephen Toor**

Chief Commercial Officer

**Commercial update**



Revenues increased 20% in 2Q23 (US\$51.1m) vs 1Q23  
2% revenue increase 2Q23 v 2Q22



Performed well in leading revenue indicators in the U.S. including active patients, vials shipped, and number of physicians prescribing



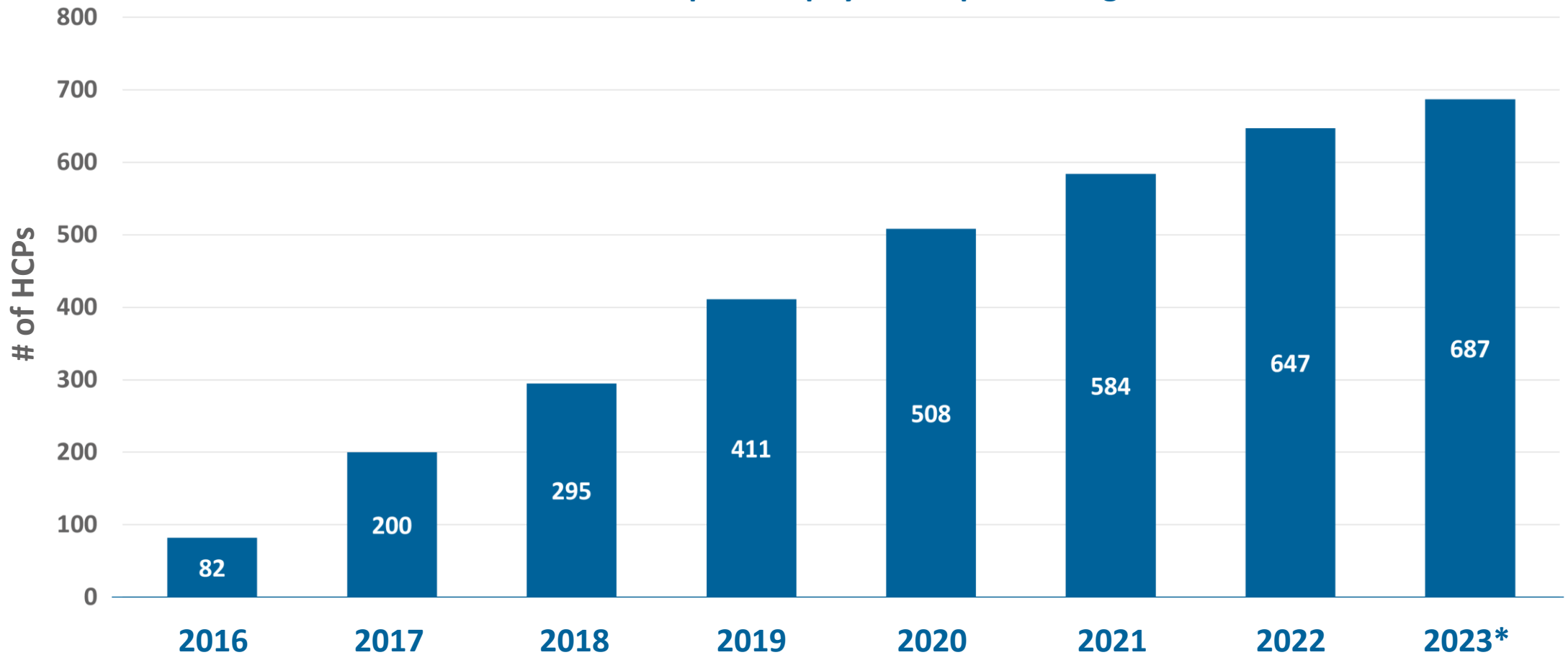
Strong U.S. in-market demand – over 70 quarterly new patient enrollments in 1Q23 and 2Q23



Continue to guide for low, single digit growth for remainder of 2023

# U.S. physicians prescribing RUCONEST<sup>®</sup> continues to grow

# of unique U.S. physicians prescribing



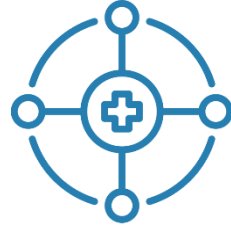
\*Data thru June 30, 2023



## Commercial Field Team

Rare Disease Team of 27  
focused on  
Allergy/Immunology

Institutional Team of 27  
focused on multiple  
specialties



## Patient Identification

- Work with HCPs to further identify patients and get them tested
- APDS clinical educators assist with family mapping



All about **APDS**  
Activated PI3K Delta Syndrome



## Support Services

- Dedicated support, education and resources for patients and caregivers through the APDS Assist patient support program
- APDS Care Coordinators provide support for onboarding, coverage assistance and financial support resources



## Patient Access

- Partnered exclusively with PANTHERx Specialty Pharmacy
- Starter and Bridge program enables rapid access while navigating coverage
- Copay Assistance and Patient Assistance Programs for eligible patients ensure affordability to care



**Precision medicine targeting rare and genetically-defined patient population**



**First and only treatment indicated for APDS addressing high unmet need**



**Demonstrated efficacy and safety profile**



**Significant burden of disease**

## ◆ Innovation:

- Pharming is committed to providing patients with rare disease the solutions they need

## ◆ Value:

- APDS is a progressive disease
- Joenja<sup>®</sup> designed to treat the root cause of APDS treating both immune deficiency and dysregulation

## ◆ Patient Access:

- Dedicated support and education resources through the APDS Assist patient support program
- APDS Assist to help patients navigate coverage to ensure all eligible patients receive access to treatment

## ◆ Support:

- Pharming is committed to the APDS community through active grassroots engagement with advocacy groups such as the IDF and Jeffrey Modell Foundation

**Annual Cost (WAC) – US\$547,500**

- ◆ MAR: FDA approval  
APR: First commercial shipment to patients
- ◆ Strong start to U.S. launch in 2Q23: 60 enrollments, of which 43 patients on paid therapy
- ◆ 19 of ~25 U.S. EAP/OLE patients are now on paid therapy.  
24 patients on paid therapy were previously untreated patients or naïve
- ◆ 2Q23 revenues: US\$3.8 million
- ◆ Productive ongoing engagement with both national and regional payers
- ◆ The sales team continue to drive new patient enrollments



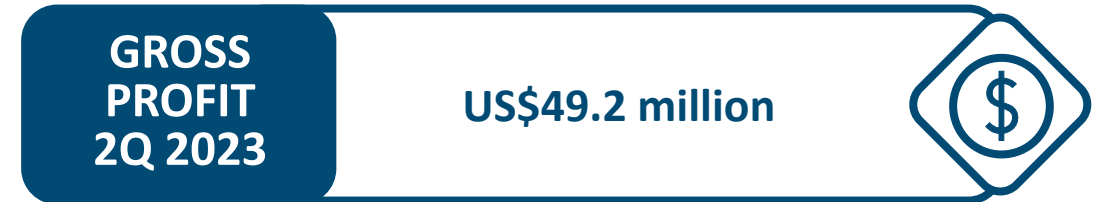
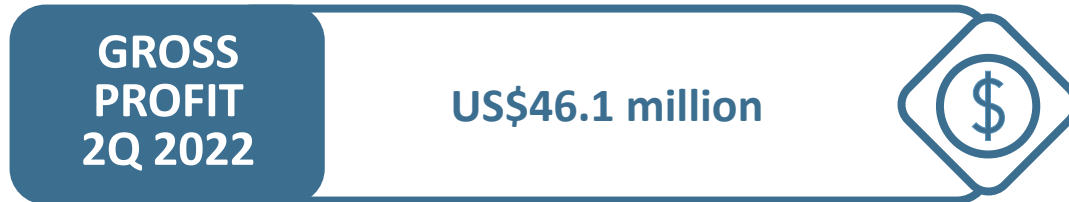
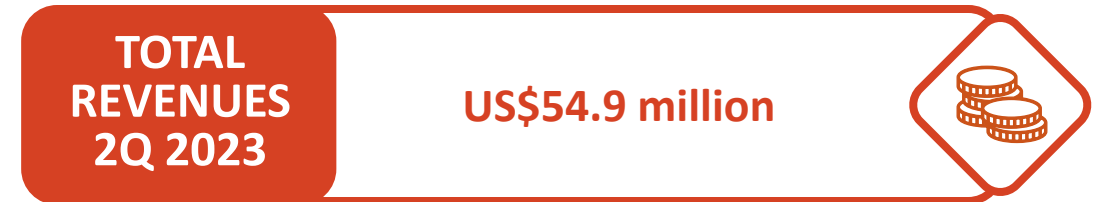




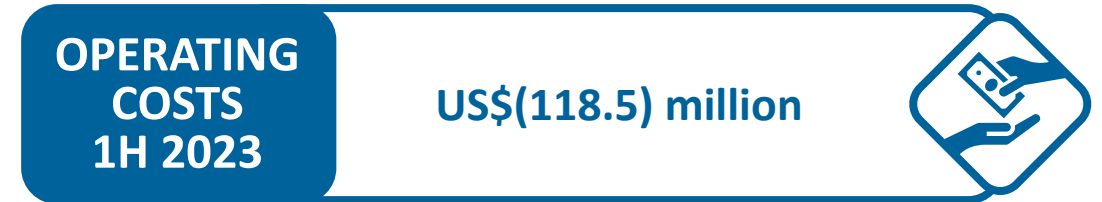
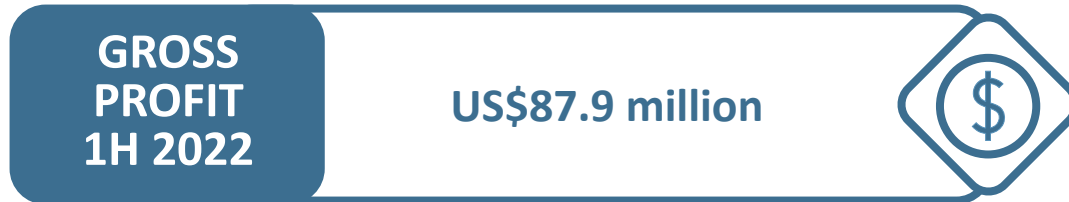
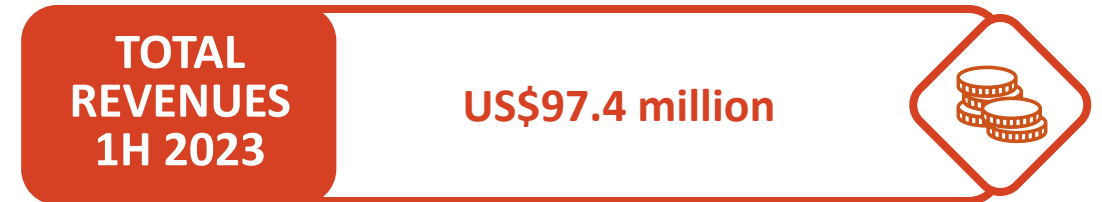
**Jeroen Wakkerman**  
Chief Financial Officer

## Financials

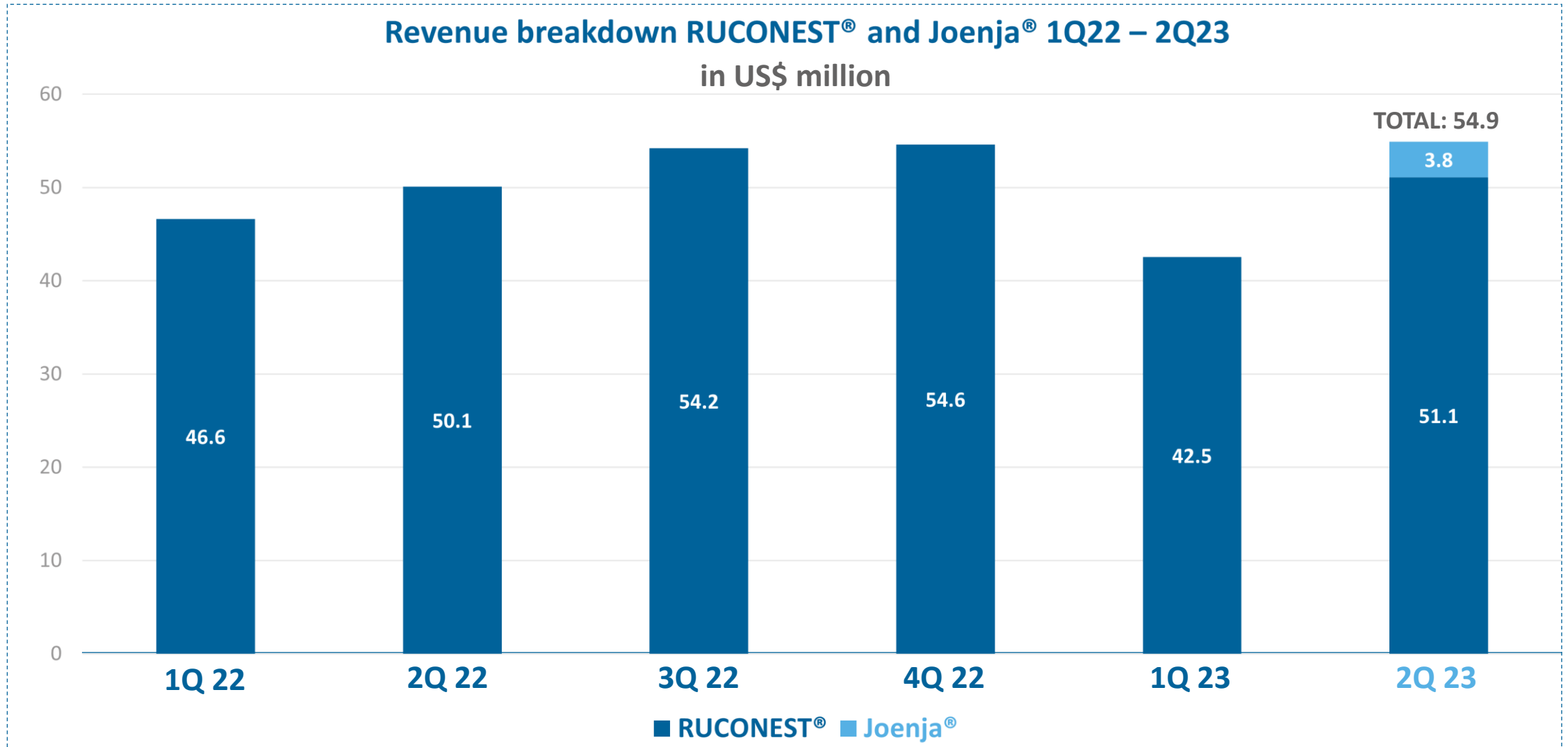
# Financial highlights: 2Q 2023 vs 2Q 2022



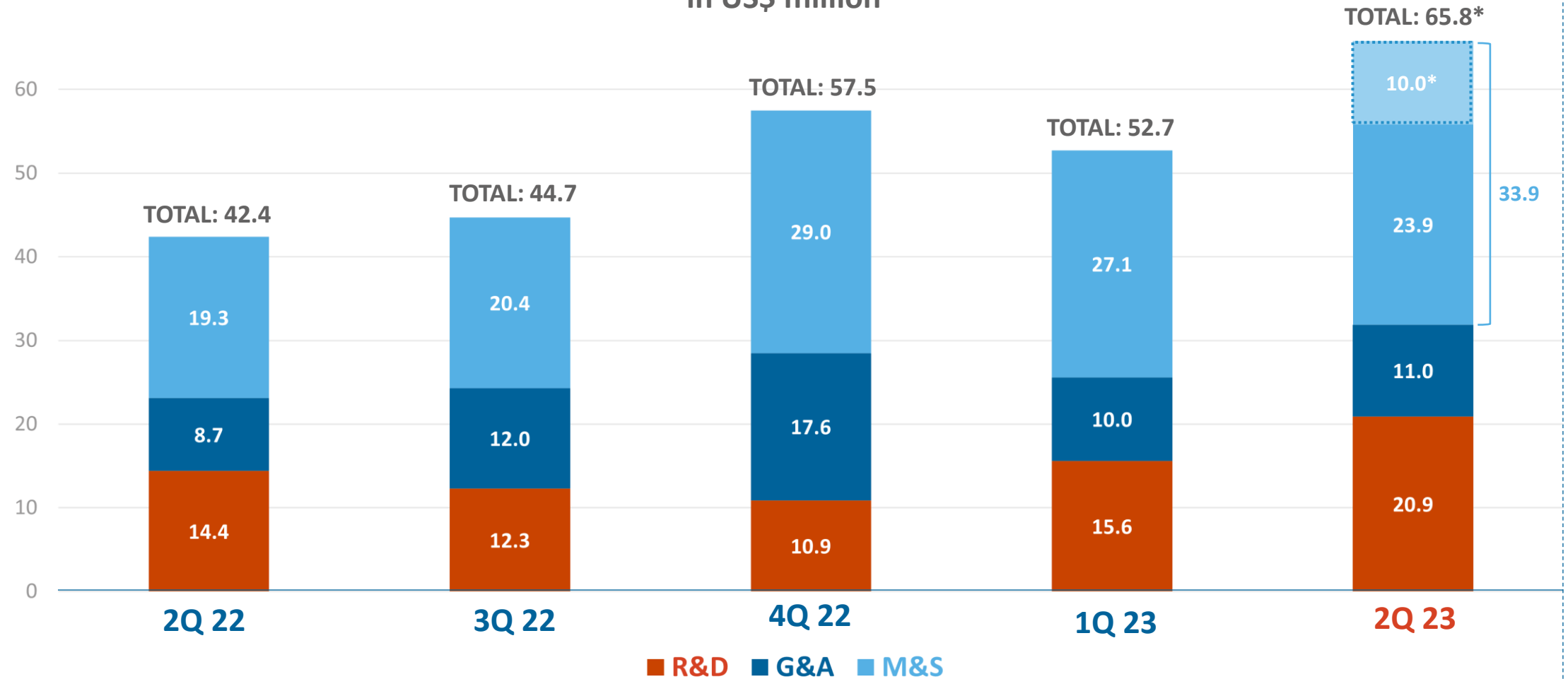
# Financial highlights: 1H 2023 vs 1H 2022



# Quarterly revenue breakdown by product

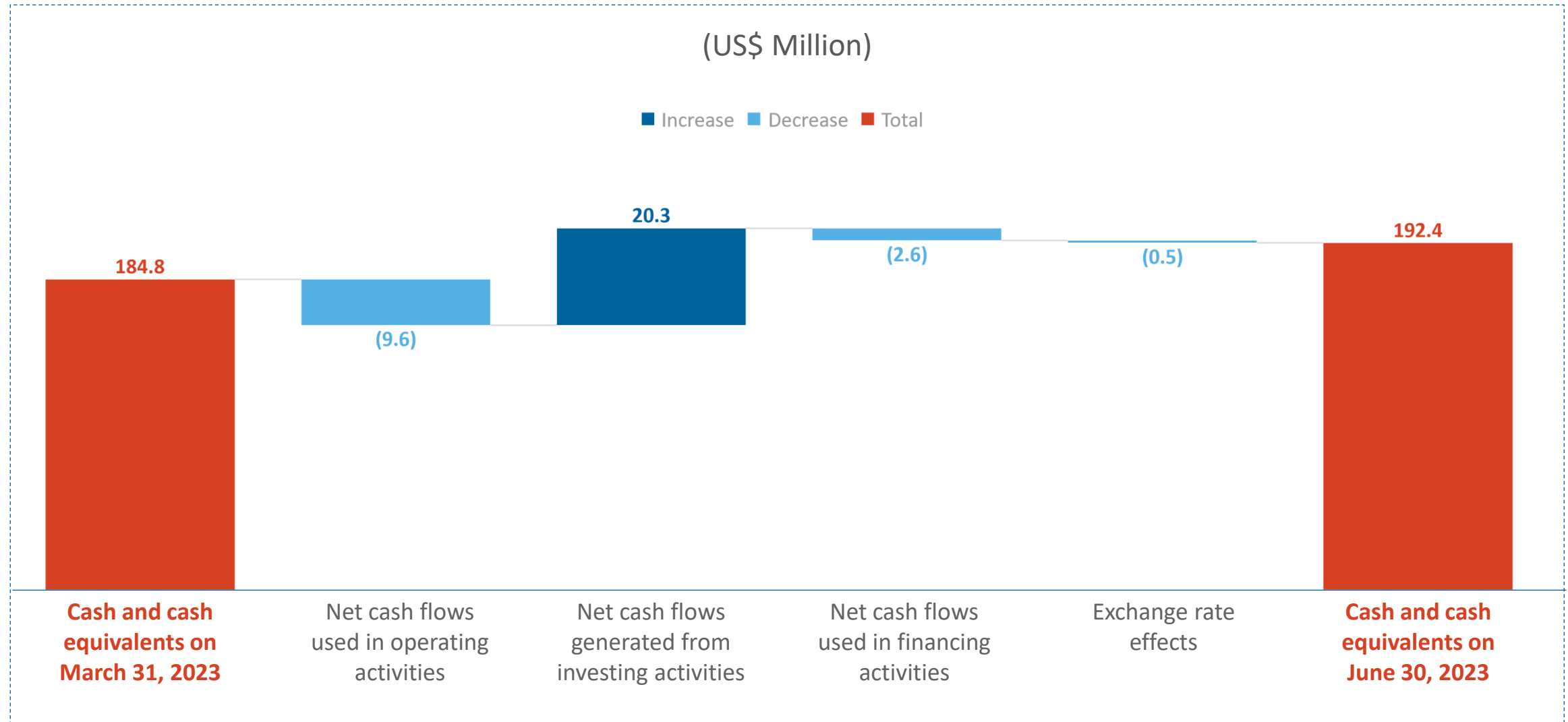


Cost category breakdown 2Q22 – 2Q23  
in US\$ million



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

# 2Q 2023: Cashflow March 31, 2023 – June 30, 2023





Continued low single digit growth in RUCONEST® revenues



Joenja® approved by FDA March 24, 2023, commercializing in U.S. since early April 2023



CHMP opinion in 4Q23, marketing authorization in Europe ~2 months later\*



File leniolisib with UK's MHRA following ECDRP route\*



Continued operating cost investments to accelerate future growth

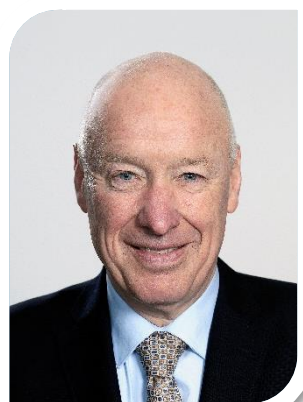


Further details on our plans to develop leniolisib in additional indications to be provided in 2H 2023



Investment and continued focus on in-licensing or acquisitions of mid to late-stage opportunities in rare diseases

\*Subject to positive outcomes of the EMA CHMP review.



**Sijmen de Vries, MD**

Chief Executive Officer



**Anurag Relan, MD**

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Chief Financial Officer





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

[www.pharming.com](http://www.pharming.com) | [investor@pharming.com](mailto:investor@pharming.com)

NASDAQ: **PHAR** | Euronext Amsterdam: **PHARM**

Bloomberg: **PHAR.AS**



**Pharming Group N.V.**

# Appendix

# Statement of profit and loss

Amounts in \$ '000	notes	1H 2023	1H 2022
<b>Revenues</b>	7	<b>97,438</b>	<b>96,763</b>
<b>Costs of sales</b>	9	<b>(9,799)</b>	<b>(8,906)</b>
<b>Gross profit</b>		<b>87,639</b>	<b>87,857</b>
<b>Other income</b>	8	<b>22,507</b>	<b>14,955</b>
Research and development		(36,534)	(29,296)
General and administrative		(20,963)	(16,421)
Marketing and sales		(61,013)	(36,449)
<b>Other Operating Costs</b>	9	<b>(118,510)</b>	<b>(82,166)</b>
<b>Operating profit (loss)</b>		<b>(8,364)</b>	<b>20,646</b>
Other finance income	10	799	6,474
Other finance expenses	10	(5,254)	(2,780)
<b>Finance gain (cost) net</b>		<b>(4,455)</b>	<b>3,694</b>
Share of net profits (loss) in associates using the equity method	12	(469)	(550)
<b>Profit (loss) before tax</b>		<b>(13,288)</b>	<b>23,790</b>
Income tax credit (expense)	11	2,399	(4,587)
<b>Profit (loss) for the period</b>		<b>(10,889)</b>	<b>19,203</b>
Basic earnings per share (US\$)	18	(0.017)	0.029
Diluted earnings per share (US\$)	18	(0.017)	0.027

Amounts in \$ '000	notes	June 30, 2023	December 31, 2022
<b>Non-current assets</b>			
Intangible assets		73,413	75,121
Property, plant and equipment		9,910	10,392
Right-of-use assets		29,436	28,753
Long term prepayments		91	228
Deferred tax assets	13	27,010	22,973
Investments accounted for using the equity method	12	2,070	2,501
Investments in equity instruments designated as at FVTOCI	12	640	403
Investments in debt instruments designated as at FVTPL	12	6,940	6,827
Restricted cash	15	1,722	1,099
<b>Total non-current assets</b>		<b>151,232</b>	<b>148,297</b>
<b>Current assets</b>			
Inventories	14	53,042	42,326
Trade and other receivables		33,158	27,619
Restricted cash	15	—	213
Cash and cash equivalents	15	192,373	207,342
<b>Total current assets</b>		<b>278,573</b>	<b>277,500</b>
<b>Total assets</b>		<b>429,805</b>	<b>425,797</b>

# Balance sheet – liabilities

Amounts in \$ '000	notes	June 30, 2023	December 31, 2022
<b>Equity</b>			
Share capital		7,540	7,509
Share premium		464,363	462,297
Legal reserves		(6,037)	(8,737)
Accumulated deficit		(265,494)	(256,431)
<b>Shareholders' equity</b>	<b>16</b>	<b>200,372</b>	<b>204,638</b>
<b>Non-current liabilities</b>			
Convertible bonds	17	134,183	131,618
Lease liabilities		30,298	29,843
<b>Total non-current liabilities</b>		<b>164,481</b>	<b>161,461</b>
<b>Current liabilities</b>			
Convertible bonds	17	1,797	1,768
Trade and other payables		59,299	54,465
Lease liabilities		3,856	3,465
<b>Total current liabilities</b>		<b>64,952</b>	<b>59,698</b>
<b>Total equity and liabilities</b>		<b>429,805</b>	<b>425,797</b>

Amounts in \$'000	1H 2023	1H 2022
<b>Profit (loss) before tax</b>	<b>(13,288)</b>	<b>23,790</b>
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>		
Depreciation, amortization, impairment	5,468	4,263
Equity settled share-based payments	3,970	2,879
Gain on disposal of investment in associate	—	(12,708)
Gain on disposal from PRV sale	(21,080)	—
Other finance income	(799)	(6,474)
Other finance expense	5,254	2,780
Share of net profits in associates using the equity method	469	550
Other	(1,743)	—
<b>Operating cash flows before changes in working capital</b>	<b>(21,749)</b>	<b>15,080</b>
<i>Changes in working capital:</i>		
Inventories	(10,717)	(6,619)
Trade and other receivables	(5,539)	(2,895)
Payables and other current liabilities	4,833	2,601
Restricted Cash	410	(84)
<b>Total changes in working capital</b>	<b>(11,014)</b>	<b>(6,997)</b>
Interest received (paid)	799	(54)
Income taxes paid	(442)	(3,422)

Amounts in \$'000	1H 2023	1H 2022
<b>Net cash flows generated from (used in) operating activities</b>	<b>(32,406)</b>	<b>4,607</b>
Capital expenditure for property, plant and equipment	(986)	(729)
Proceeds on PRV sale	21,080	—
Investment intangible assets	—	(829)
Investment in associate	—	7,578
<b>Net cash flows generated from (used in) investing activities</b>	<b>20,094</b>	<b>6,020</b>
Payment of lease liabilities	(2,570)	(1,594)
Interests on loans	(2,023)	(2,052)
Settlement of share based compensation awards	(666)	306
<b>Net cash flows generated from (used in) financing activities</b>	<b>(5,259)</b>	<b>(3,340)</b>
<b>Increase (decrease) of cash</b>	<b>(17,570)</b>	<b>7,287</b>
Exchange rate effects	2,601	(9,247)
Cash and cash equivalents at January 1	207,342	191,924
<b>Total cash and cash equivalents at June 30</b>	<b>192,373</b>	<b>189,964</b>