



Pharming Group N.V.

Third quarter 2023
financial results

October 26, 2023

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



Sijmen de Vries, MD
Chief Executive Officer



Stephen Toor
Chief Commercial Officer



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Chief Medical 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 >\$200 million TTM sales RUCONEST® funds Joenja® (leniolisib) launches & pipeline development

- ◆ RUCONEST® strong revenue growth 3Q23 +18% vs 2Q23 and +11% vs 3Q22
- ◆ 9M23 RUCONEST® revenue +2% vs 9M22
- ◆ On track for low single digit revenue growth for 2023




Global approvals and commercialization of Joenja® (leniolisib)



Successful commercialization of Joenja® (leniolisib) for APDS

- ◆ MAR: FDA approval for Joenja® SEP: Strong 3Q U.S. revenues US\$6.5M / YTD US\$10.3M
- ◆ Regulatory reviews ongoing in EUR, CAN, AUS, ISR
- ◆ Pediatric clinical program ongoing



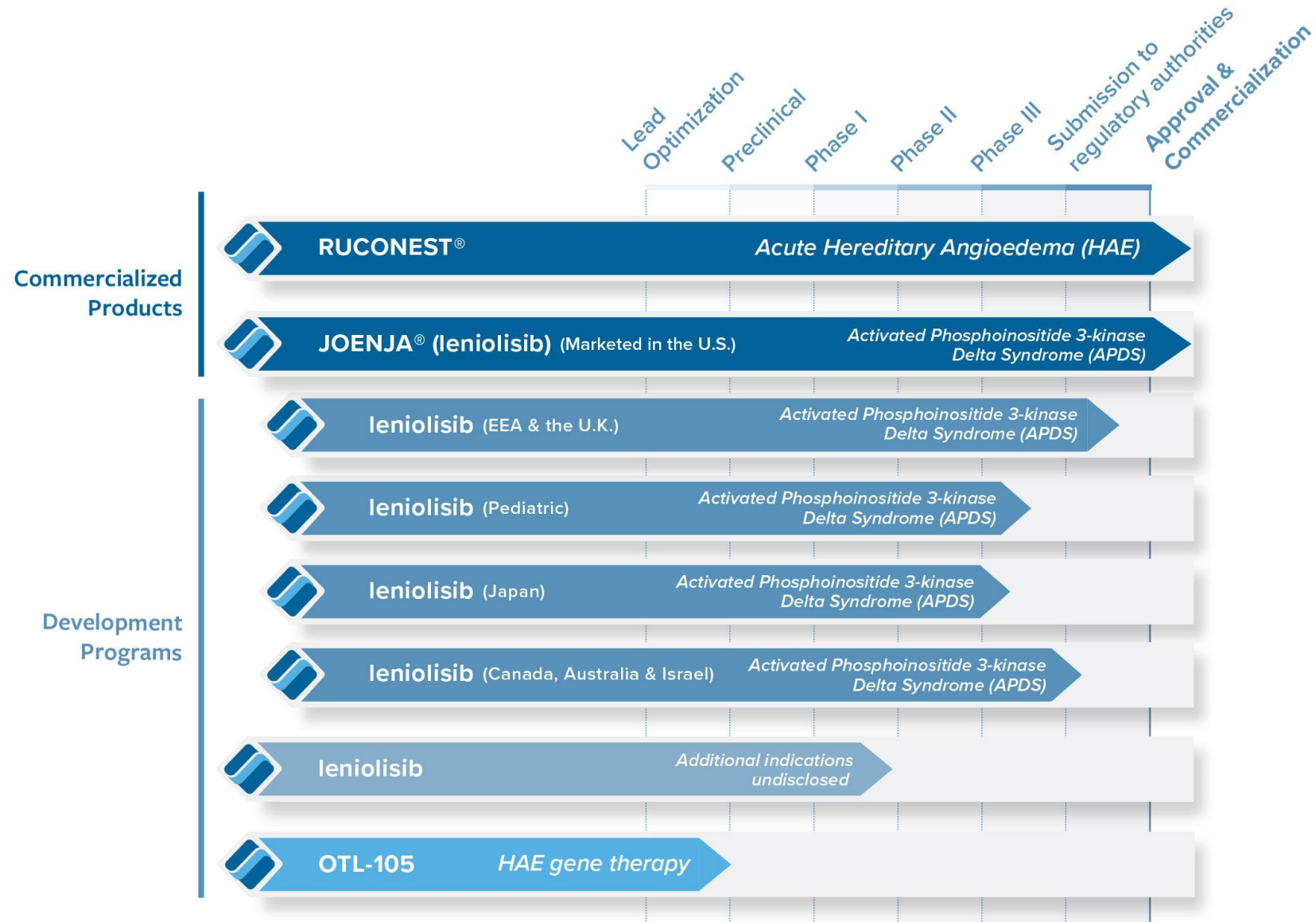
Ongoing pipeline development and management of rare disease assets



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

- ◆ Advanced development plans for 2nd leniolisib indication – further details by end 2023
- ◆ 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® | 35 years



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Stephen Toor

Chief Commercial Officer

Commercial update



Revenues increased 11% in 3Q23 (US\$60.2m) vs 3Q22
Revenues increased 2% in 9M23 (US\$153.8m) vs 9M22



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 new patient enrollments for 3 straight quarters



On track for low single digit revenue growth for 2023

Strong commitment to HAE community



Strong patient organization support since 2000

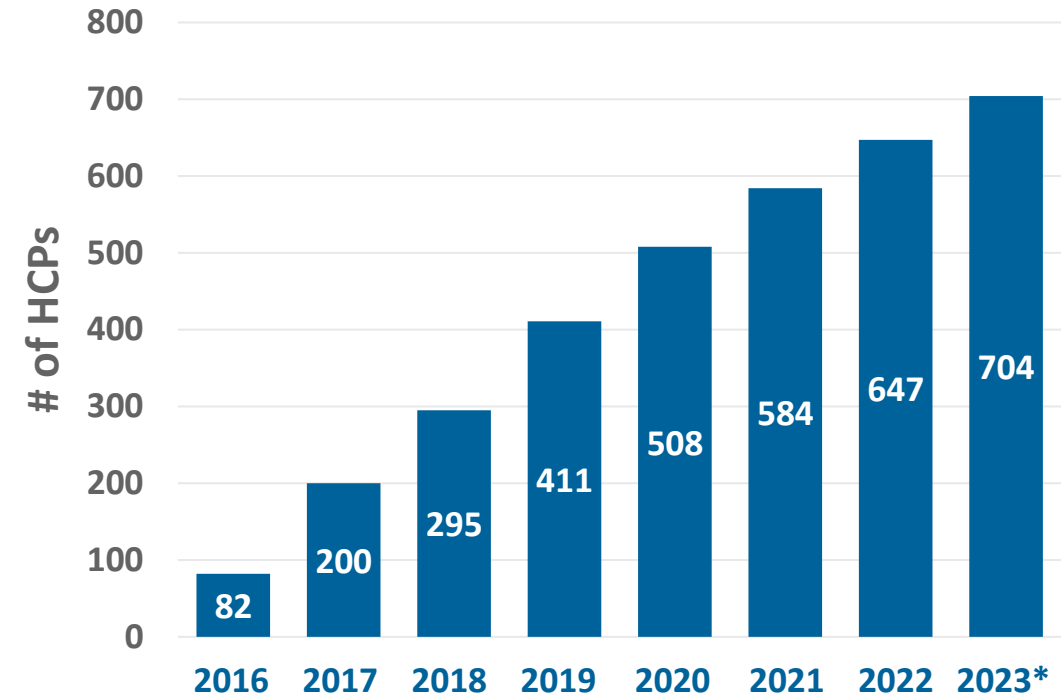


More than 700 U.S. physicians (and growing) prescribing RUCONEST®



>2,000 patients with HAE have been prescribed RUCONEST®

of unique U.S. physicians prescribing



*Data thru September 30, 2023





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



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
- 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

- ◆ Strong commercial execution 6 months into U.S. launch
- ◆ Continue to add enrollments
76 enrollments, of which 63 patients on paid therapy at end 3Q23
- ◆ All but one pre-existing OLE/EAP patients enrolled or are on paid therapy
37 patients on paid therapy were previously untreated patients or naïve
- ◆ 3Q23 revenues: US\$6.5 million
9M23 revenues: US\$10.3 million
- ◆ Significant focus on genetic family testing
Ramp up in 4Q23 and 1Q24
- ◆ Productive ongoing engagement with both national and regional payers





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 δ) syndrome (APDS) affects >1500 patients*

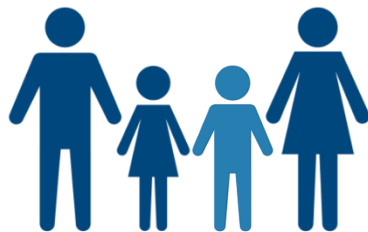
Pharming has identified >640 of these patients in key launch markets

(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

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

Joenja® (leniolisib) is a prescription medicine that is used to treat activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) in adults 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

Strong start to Joenja® launch with 76 enrollments & 63 patients on paid therapy as of September 30, 2023



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



Japan clinical study – 1st patient enrolled Aug 2023



Named patient program ongoing



Provide details on development plans for 2nd indication for leniolisib in 4Q23



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



AUS, CAN, ISR submissions processing as anticipated

CAN & AUS approval 2Q24***
ISR approval 1H24***



Pediatric study for 4 to 11 years: enrollment majority (11/15) complete



Pediatric study for 1 to 6 years:
Trial now recruiting

* Received CHMP Day 180 list of outstanding issues in July. 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. Approval is subject to positive outcomes of the EMA CHMP review.

** Subject to positive outcomes of the EMA CHMP review

*** Subject to positive AUS, CAN, ISR decisions



Medical education to raise awareness of APDS and share leniolisib data

- ◆ Conferences and congresses
- ◆ Abstracts
- ◆ Publications



Genetic testing

- ◆ Sponsored, no-cost testing program



- ◆ Genetic counselors to assist with testing and reviewing results
- ◆ Partnering with genetic testing companies to identify previously and newly diagnosed APDS patients



Family testing

- ◆ Inherited disease* but most APDS patients do not have diagnosed family members
- ◆ Patients may not be aware of genetics or have access to specialty physicians
- ◆ Cooperating with clinicians to encourage family testing
- ◆ Patients can request a genetic test through partner Genome Medical (if suspect APDS for themselves or family members)
- ◆ Reduces barrier for easier testing of those suspected with APDS

*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.

Helping diagnose APDS patients: Variant of Uncertain Significance (VUS) resolution

Genetic testing frequently leads to inconclusive results - previously unseen genetic variants:



Patients have clinical symptoms compatible with APDS, but genetic variant test is inconclusive



Frustrating for patients and clinicians

Need to determine if Variant of Uncertain Significance (VUS) causes APDS

Pharming initiatives/partnerships to resolve VUSs



Variant Curation

- ◆ ClinGen expert panels develop gene/disease specific thresholds and criteria for classifying variants
- ◆ Partnership with Genomenon to develop Genomic Landscape (comprehensive, systematic review of all published variant data)



Functional testing

- ◆ Improve access to directly measure PI3K pathway activity in patient blood samples
- ◆ Sharing of results via public databases (ClinVar)



Multiplexed assays of variant effect (MAVE)

- ◆ Test nearly all possible variants in a single experiment
- ◆ Generate variant effect map, including variants already found and those not yet found (proactive)



◆ AMCP Nexus (October 2023)

- *A Real-world Comparison of Health Care Resource Utilization and Health Care Costs Among Patients With Activated PI3K-Delta Syndrome Versus a Control Cohort of Patients Without Activated PI3K-Delta Syndrome in the United States*



◆ ACAAI (November 2023)

- *Mortality in Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome, a Systematic Literature Review*



IPIC2023

**INTERNATIONAL
PRIMARY
IMMUNODEFICIENCIES
CONGRESS**

◆ IPIC (November 2023)

- *Results of a second interim analysis of an ongoing single-arm open-label extension study of leniolisib in activated PI3K delta syndrome: long-term efficacy and safety through to March 2023.*
- *Complicated course of activated PI3K delta syndrome-1 ameliorated by leniolisib: a case study.*
- *Gastrointestinal manifestations in patients with activated PI3K delta syndrome (APDS) treated with leniolisib.*
- *Assessing long-term treatment with leniolisib and its effects on bronchiectasis in patients with activated PI3K delta syndrome (APDS).*

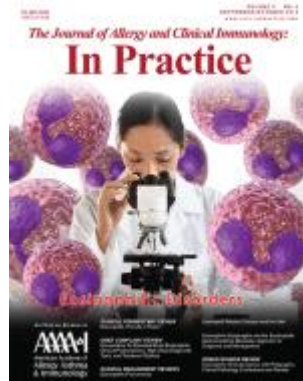


Rao VK, et al. (Sep 2023)

Interim Analysis: Open-Label Extension Study of Leniolisib for Patients with APDS.



<https://doi.org/10.1016/j.jaci.2023.09.032>



Cant AJ, et al. (Sep 2023)

PI3K δ Pathway Dysregulation and Unique Features of Its Inhibition by Leniolisib in Activated PI3K δ Syndrome and Beyond.













<https://doi.org/10.1016/j.jaip.2023.09.016>



Jeroen Wakkerman
Chief Financial Officer

Financials

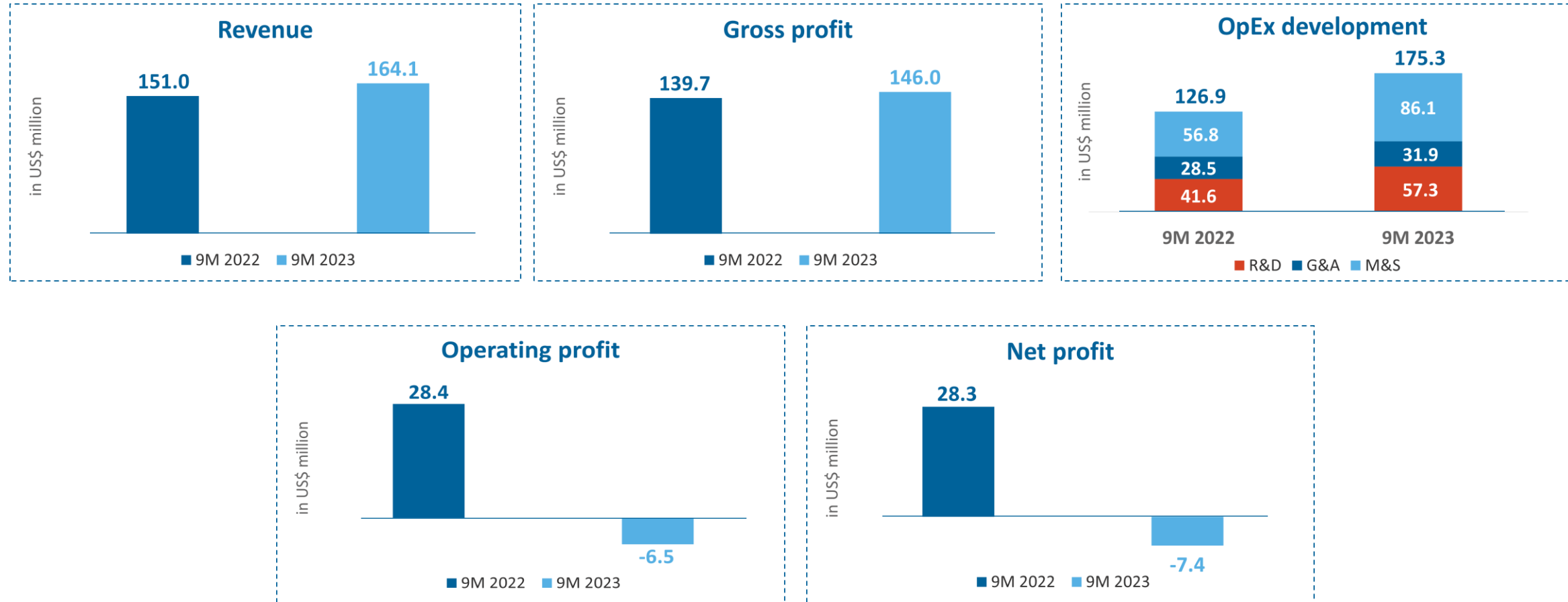
Financial highlights: 3Q 2023 vs 3Q 2022

TOTAL REVENUES 3Q 2022	US\$54.2 million		TOTAL REVENUES 3Q 2023	US\$66.7 million	
GROSS PROFIT 3Q 2022	US\$51.9 million		GROSS PROFIT 3Q 2023	US\$58.4 million	
OPERATING COSTS 3Q 2022	US\$(44.7) million		OPERATING COSTS 3Q 2023	US\$(56.8) million	
OPERATING PROFIT (LOSS) 3Q 2022	US\$7.8 million		OPERATING PROFIT (LOSS) 3Q 2023	US\$1.9 million	
NET PROFIT (LOSS) 3Q 2022	US\$9.1 million		NET PROFIT (LOSS) 3Q 2023	US\$3.5 million	

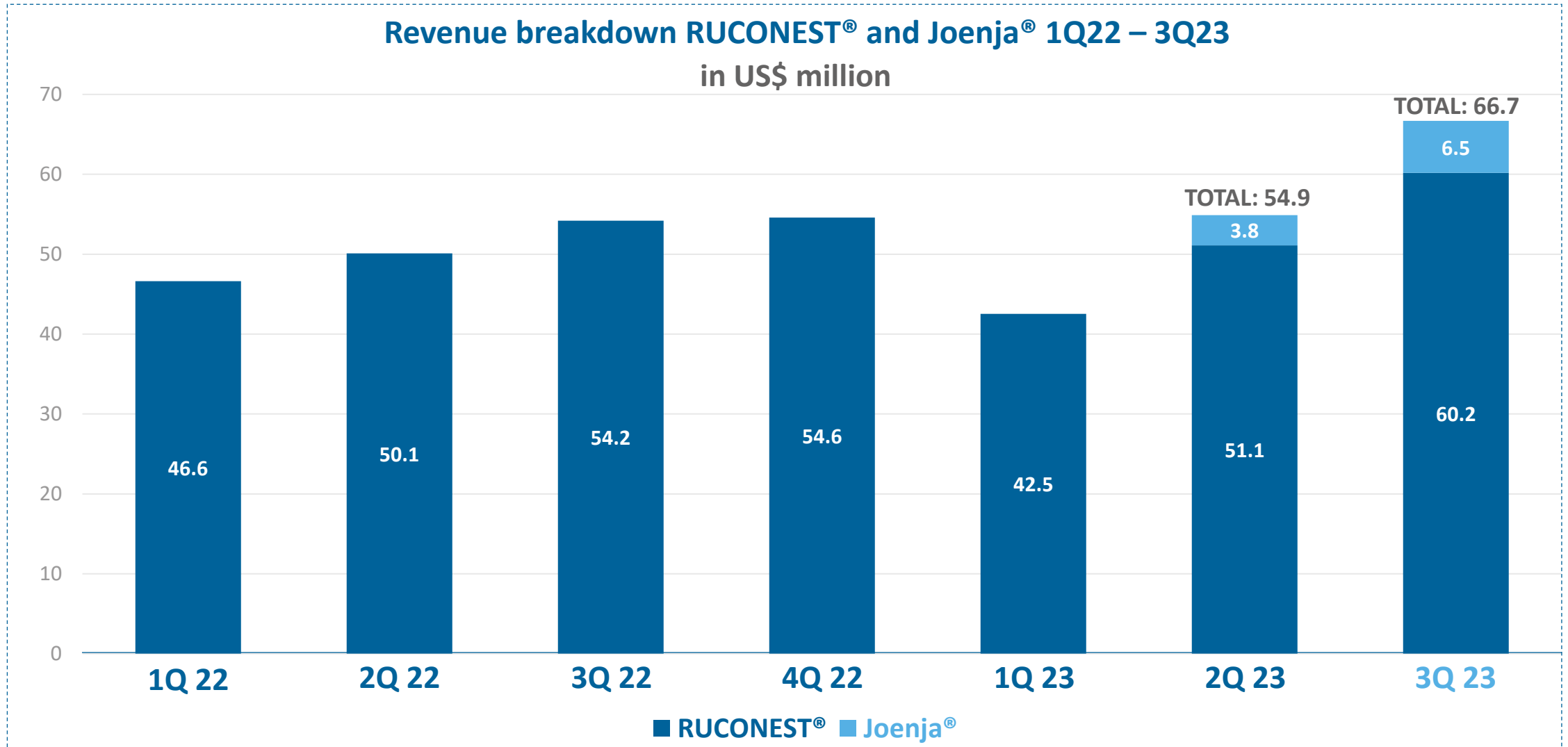


Cash and cash equivalents, together with restricted cash and marketable securities, increased from US\$194.1M at the end of 2Q23 to US\$199.2M at the end of 3Q23

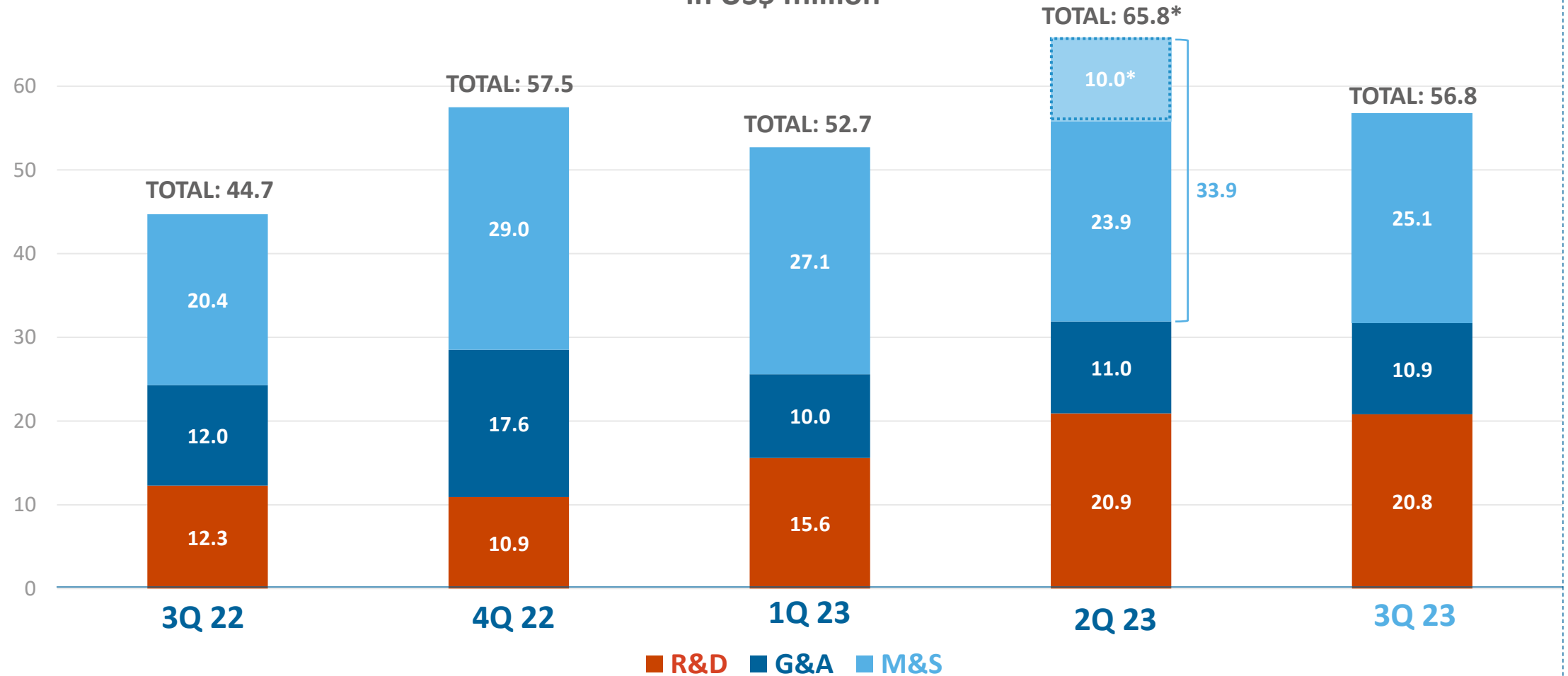
Financial highlights: 9M 2023 vs 9M 2022



RUCONEST® and Joenja® driving revenue growth



Cost category breakdown 3Q22 – 3Q23
in US\$ million



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



On track for 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*



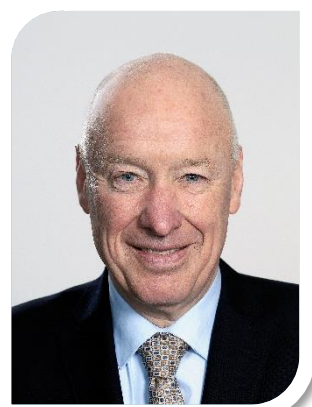
Continued operating cost investments to accelerate future growth



Further details on our plans to develop leniolisib in additional indications to be provided in 4Q 2023



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



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Appendix

Statement of profit and loss

Amounts in US\$ '000	9M 2023	9M 2022
Revenues	164,099	151,001
Costs of sales	(18,094)	(11,288)
Gross profit	146,005	139,712
Other income	22,811	15,602
Research and development	(57,287)	(41,639)
General and administrative	(31,849)	(28,446)
Marketing and sales	(86,136)	(56,819)
Other Operating Costs	(175,272)	(126,904)
Operating profit (loss)	(6,456)	28,410
Other finance income	2,050	9,297
Other finance expenses	(4,621)	(3,978)
Finance cost net	(2,571)	5,319
Share of net profits in associates using the equity method	(954)	(660)
Profit (loss) before tax	(9,981)	33,069
Income tax credit (expense)	2,556	(4,765)
Profit (loss) for the period	(7,425)	28,304
Basic earnings per share (US\$)	(0.011)	0.043
Fully-diluted earnings per share (US\$)	(0.011)	0.040

Amounts in US\$ '000	September 30, 2023	December 31, 2022
Non-current assets		
Intangible assets	69,849	75,121
Property, plant and equipment	9,648	10,392
Right-of-use assets	27,834	28,753
Long-term prepayments	88	228
Deferred tax assets	26,608	22,973
Investments accounted for using the equity method	1,541	2,501
Investment in equity instruments designated as at FVTOCI	949	403
Investment in debt instruments designated as at FVTPL	6,749	6,827
Restricted cash	1,464	1,099
Total non-current assets	144,730	148,297
Current assets		
Inventories	53,439	42,326
Trade and other receivables	40,521	27,619
Restricted cash	212	213
Marketable securities	142,912	—
Cash and cash equivalents	54,653	207,342
Total current assets	291,737	277,500
Total assets	436,467	425,797

Amounts in US\$ '000	September 30, 2023	December 31, 2022
Equity		
Share capital	7,650	7,509
Share premium	475,983	462,297
Legal reserves	(10,915)	(8,737)
Accumulated deficit	(262,776)	(256,431)
Shareholders' equity	209,942	204,638
Non-current liabilities		
Convertible bonds	129,733	131,618
Lease liabilities	28,734	29,843
Total non-current liabilities	158,467	161,461
Current liabilities		
Convertible bonds	1,748	1,768
Trade and other payables	62,540	54,465
Lease liabilities	3,770	3,465
Total current liabilities	68,058	59,698
Total equity and liabilities	436,467	425,797

Amounts in \$'000	9M 2023	9M 2022
Profit before tax	(9,981)	33,069
<i>Adjustments to reconcile net profit (loss) to net cash used in operating activities:</i>		
Depreciation, amortization, impairment	8,370	6,216
Equity settled share based payments	5,935	4,522
Gain on disposal of investment in associate	—	(12,382)
Gain on disposal from PRV sale	(21,080)	—
Other finance income	(2,050)	(9,296)
Other finance expense	4,621	3,978
Share of net profits in associates using the equity method	954	660
Other	(1,130)	—
Operating cash flows before changes in working capital	(14,361)	26,767
<i>Changes in working capital:</i>		
Inventories	(11,113)	(6,196)
Trade and other receivables	(12,902)	1,155
Payables and other current liabilities	8,075	272
Restricted Cash	363	169
Total changes in working capital	(15,577)	(4,600)
Interest received (paid)	1,059	31
Income taxes paid (received)	—	(4,975)

Amounts in \$'000	9M 2023	9M 2022
Net cash flows generated from (used in) operating activities	(28,879)	17,223
Capital expenditure for property, plant and equipment	(1,133)	(1,071)
Proceeds on PRV sale	21,080	—
Investment intangible assets	23	(591)
Investment in associate	—	7,384
Purchases of marketable securities	(231,901)	—
Proceeds from sale of marketable securities	86,451	—
Net cash flows used in investing activities	(125,480)	5,722
Payment of lease liabilities	(3,847)	(2,385)
Interests on loans and leases	(4,052)	(3,999)
Settlement of share based compensation awards	7,880	1,124
Net cash flows generated from (used in) financing activities	(19)	(5,260)
Increase (decrease) of cash	(154,378)	17,685
Exchange rate effects	1,689	(20,906)
Cash and cash equivalents at the start of the period	207,342	191,924
Total cash and cash equivalents at the end of the period	54,653	188,703