





Fabrice Chouraqui
Chief Executive Officer



Stephen ToorChief Commercial Officer

(())) SPEAKERS



Anurag Relan, MD
Chief Medical Officer

Forward-looking statements

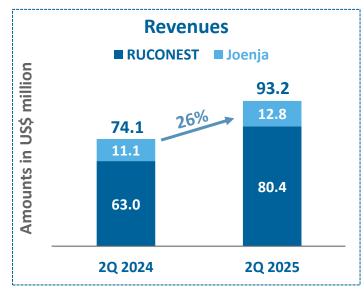


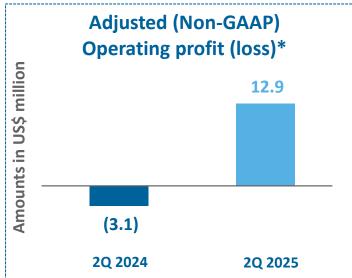
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Strong second quarter 2025 performance

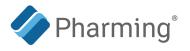


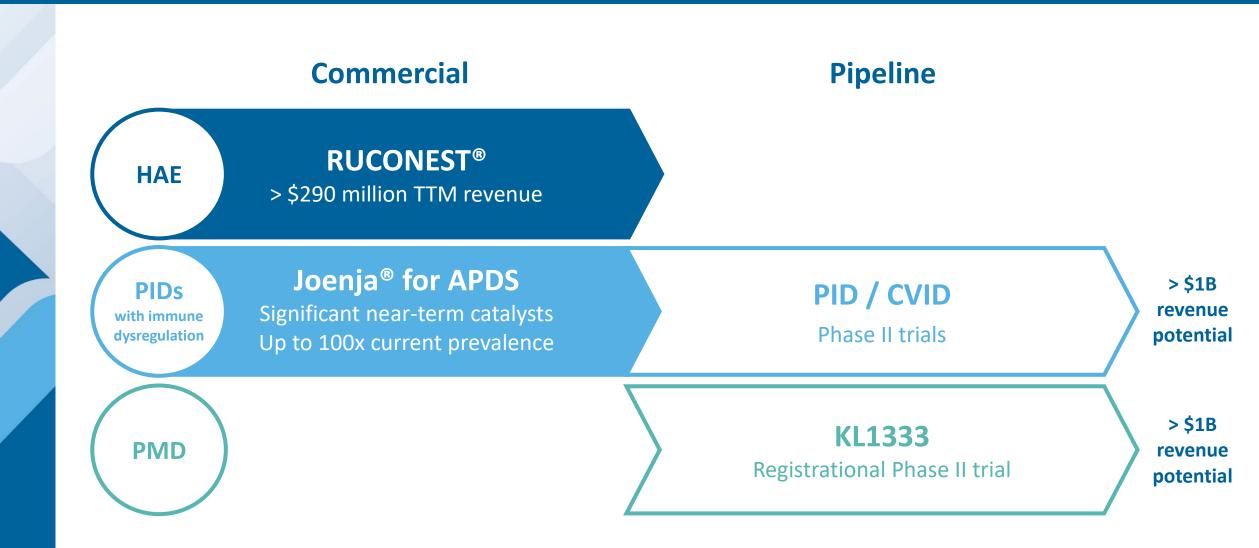




- Total revenues up 26%
- Meaningful operating profit and cash generated from operations
- Continued double-digit RUCONEST® revenue growth
- Double-digit Joenja® revenue growth with accelerating patient uptake
- Raising 2025 revenue guidance to US\$335-350 million

Unique combination of commercial and pipeline assets poised to deliver strong value creation





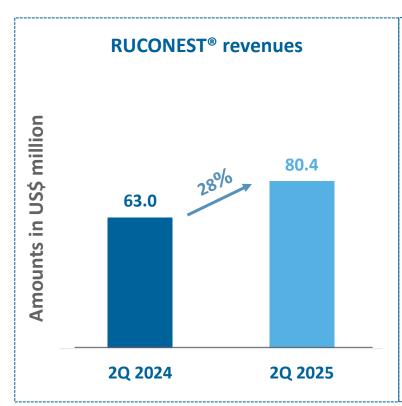


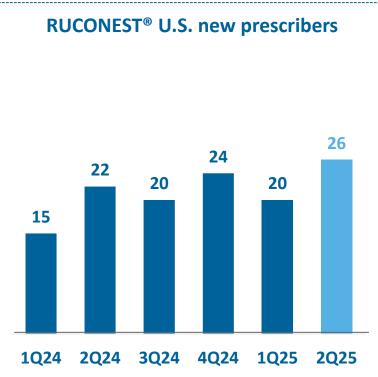
Develop a leading global rare disease company with a diverse portfolio and presence in large markets, leveraging proven and efficient clinical development, supply chain, and commercial infrastructure



RUCONEST® strong growth continues in acute HAE market







Strong U.S. in-market demand in 2Q 2025

- Continuing to add prescribers and patients
- New patient enrollments remain high (~90)
- Continued robust U.S. volume growth
 - +27% in 2Q25
 - +31% in 1H25

RUCONEST® unique value proposition and positioning



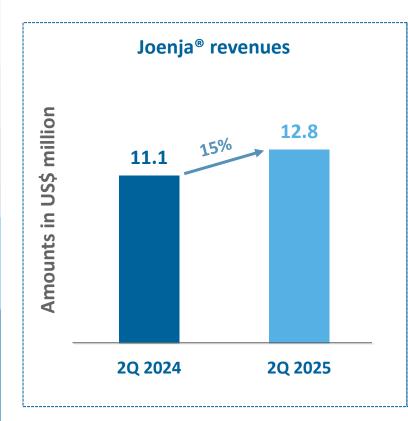
- ◆ Type 1, Type 2, and Normal C1-INH HAE patients rely on RUCONEST
- ◆ 97% attacks treated with just 1 dose¹
- 93% acute attacks stopped for at least 3 days²
- RUCONEST® mostly used by patients experiencing more severe/frequent attacks

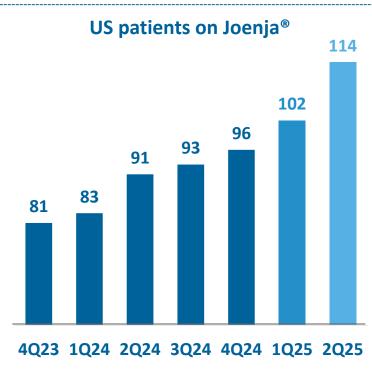




Joenja® acceleration in patient growth in 12y+ APDS







- Acceleration in APDS patients on therapy
 - 114 U.S. patients(+25% vs 2Q24)
 - 1H25 increase exceeded total for 2024
- Launched in the U.K. in April
- Additional 185 APDS patients globally in access programs and clinical studies

Joenja® near-term growth catalysts – APDS



Reclassification of VUS* patients 2H25

- >1400 patients in the US with VUS results
- June 2025 publication in *Cell* supports functional classification of >100 variants
- 20% patients could ultimately be diagnosed with APDS

Pediatric label expansion 1H26

- 4-11 years pediatric filing in the US (3Q25)
- Approval expected in 1H26
- > 50 US pediatric patients, many already on drug

Geographic Expansion *ongoing*

- Launched and reimbursed in the UK
- Japan NDA filing for ages 4+ in June 2025
- Japan, EU and Canada approvals expected in 2026
- 150 APDS patients in access programs and clinical studies

Joenja® (leniolisib) lifecycle to realize \$1Bn+ sales potential



U.S. patient numbers used to illustrate estimated prevalence

APDS*

~500 U.S. patient prevalence

VUS patient reclassifications

Up to 100x prevalence expansion

PIDs with immune dysregulation linked to PI3Kδ signaling

>2,500 U.S. patients

CVID with immune dysregulation

>13,000 U.S. patients (PID/CVID)

Commercial

New indications in Phase II POC trials







Anurag Relan, MD
Chief Medical Officer

R&D update

Newly published study expands characterization of APDS

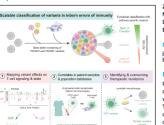




Article

Scalable generation and functional classification of genetic variants in inborn errors of immunity to accelerate clinical diagnosis and treatment

Graphical abstrac



Authors Zachary H. Walsh, Chris J. Frangiel Neeharika Kothapalli, ..., Dusan Bogunovic, Joshua D. Milne

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. . . .

In lieu of traditional genetic variant testing approaches, an approach using scalable variant classification in primary human T cells with a clinically relevant readout can inform rapid diagnosis and treatment of inborn errors of immunity.

Study uncovered >100 new variants leading to PI3K δ hyperactivity (GOF variants)

Leniolisib restored / improved PI3Kδ signaling defects and immune abnormalities caused by GOF variants





Data suggests that VUS patients with these GOF variants should be reclassified as APDS

Genetic test labs will utilize data to independently re-assess VUSs and reclassify patients to APDS*

Expand studies to functionally evaluate the remainder of all possible variants in APDS genes

Frequency of new GOF variants in population databases is significantly higher than current understanding of APDS

Study concludes that APDS may be up to 100x more prevalent than previously estimated with broader clinical features

Population-based studies planned to refine the genetic prevalence and clinical manifestations of APDS using biobanks

^{*} Over 1,400 known U.S. patients with a variant of uncertain significance, or VUS, in the PIK3CD and PIK3R1 genes implicated in APDS

Ongoing regulatory progress and pipeline execution





Japan NDA filed for adult and pediatric patients 4 years of age and older (June) FDA filing for pediatric label expansion for children aged 4 to 11 years (3Q)

PIDS with immune dysregulation

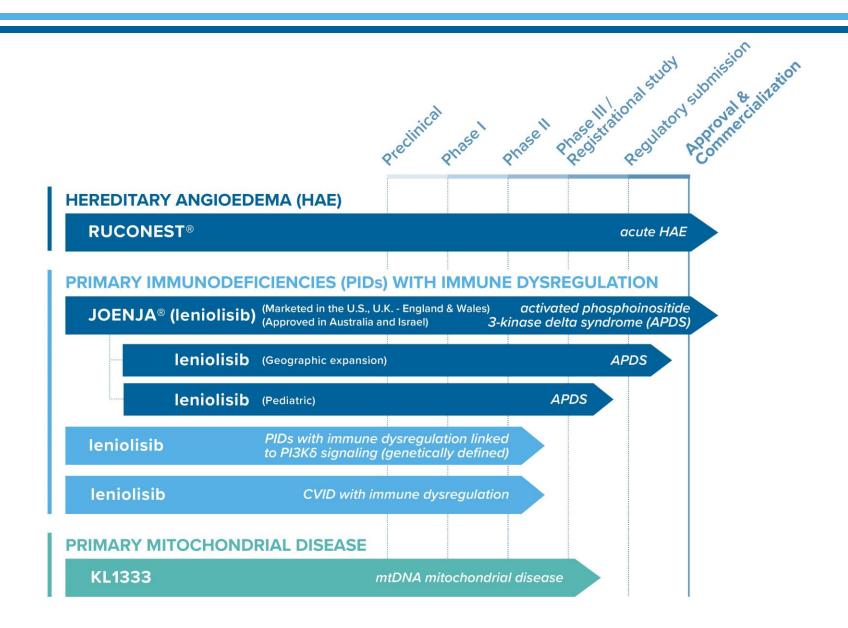
Genetic PID/CVID Phase II POC trials on track for 2026 read-outs

PMD

KL1333 pivotal trial – new sites activated, first patients dosed, on track for 2027 read-out

Diverse rare disease portfolio and pipeline









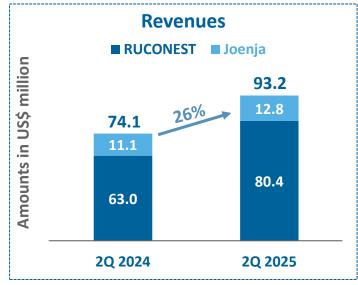


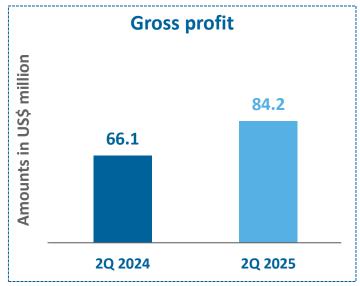
Fabrice Chouraqui Chief Executive Officer

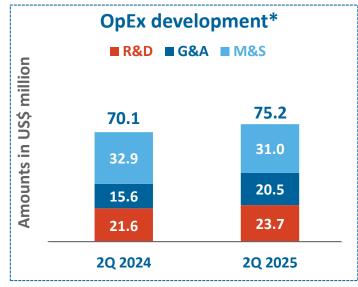
Financials and outlook

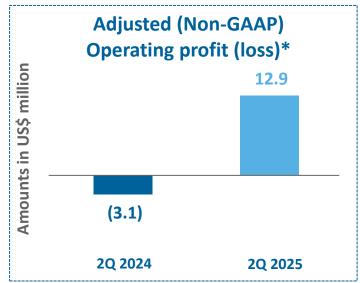
Financial highlights: 2Q 2025 vs 2Q 2024

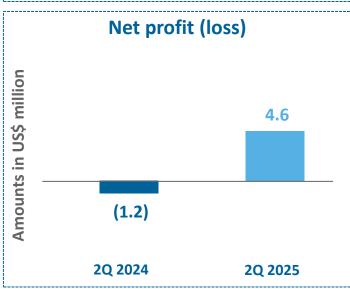










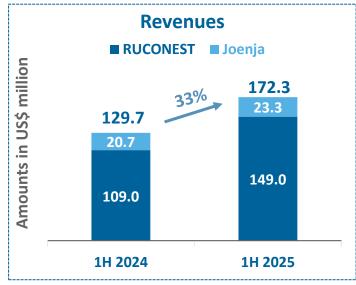




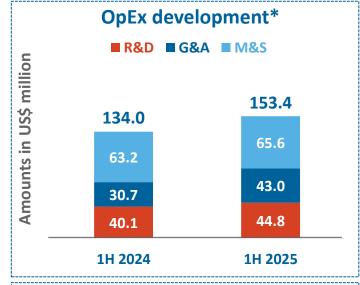
^{*} Adjusted operating profit for 2Q 2025 excludes US\$2.1 million of non-recurring Abliva acquisition-related expenses (US\$1.9 million in G&A, \$0.2 million in R&D).

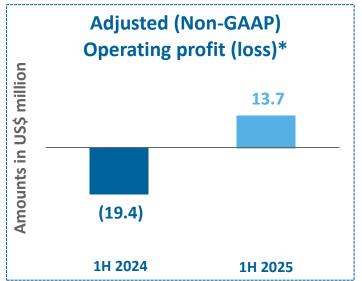
Financial highlights: 1H 2025 vs 1H 2024

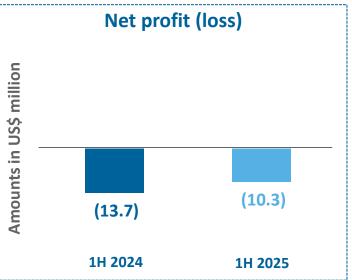


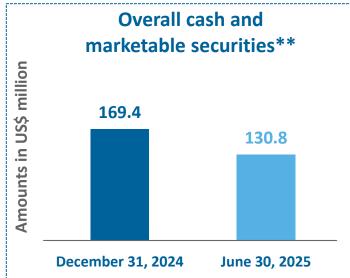








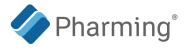




^{*} Adjusted operating profit for 1H 2025 excludes US\$9.9 million of non-recurring Abliva acquisition-related expenses (US\$7.6 million in G&A, \$2.3 million in R&D).

^{**} Decrease in cash primarily driven by purchases of Abliva shares totaling US\$66.1 million and non-recurring Abliva acquisition-related expenses totaling US\$9.9 million.

2025 financial guidance and long-term capital outlook



Revenue and operating expenses:

	FY 2025 Guidance	Notes
Total Revenues	US\$335 - 350 million	13 - 18% growth
Operating Expenses	US\$304 - 308 million	Assumes constant currency, Includes \$10.2 million non-recurring Abliva- related transaction and integration expenses

- **♦ RUCONEST®** well positioned to provide continued strong cash flows
- Available cash and future cash flows expected to cover current pipeline and pre-launch costs

Building a leading global rare disease biopharma company



Strong start to 2025

Strong RUCONEST® growth and acceleration of Joenja® patient uptake.

2Q25 revenues +26% 1H25 revenues +33%

Achieved operating profit

Updated 2025 guidance

Raising revenue guidance to US\$335 - 350M

High value pipeline

Joenja® (leniolisib) for PIDs/CVID with immune dysregulation

KL1333 for mtDNA mitochondrial disease

Significant catalysts

Joenja® for APDS: VUSs, pediatric label, geo expansion (2025-26)

Higher APDS prevalence

Leniolisib PIDs/CVID PhII readouts (2026)

KL1333 pivotal study readout (2027)





Fabrice Chouraqui
Chief Executive Officer



Stephen ToorChief Commercial Officer



Q&A



Anurag Relan, MD
Chief Medical Officer





Statement of profit and loss



Amounts in US\$ '000	notes	1H 2025	1H 2024
Revenues	8	172,315	129,679
Costs of sales	10	(17,295)	(16,367)
Gross profit	8	155,020	113,312
Other income	9	2,232	1,257
Research and development		(44,837)	(40,118)
General and administrative		(42,991)	(30,707)
Marketing and sales		(65,619)	(63,177)
Other Operating Costs	10	(153,447)	(134,002)
Operating profit (loss)		3,805	(19,433)
Fair value gain (loss) on revaluation	13	_	5,138
Other finance income	11	1,263	2,935
Other finance expenses	11	(9,785)	(4,490)
Finance result, net		(8,522)	3,583
Share of net profits (loss) in associates using the equity method	13	8	(834)
Profit (loss) before tax		(4,709)	(16,684)
Income tax credit (expense)	12	(5,629)	3,018
Profit (loss) for the period		(10,338)	(13,666)
Attributable to:			
Equity holders of the parent		(10,025)	(13,666)
Non-controlling interests	7	(313)	_
Earnings per share			
Basic, attributable to equity holders of the parent (US\$)	20	(0.015)	(0.019)
Diluted, attributable to equity holders of the parent (US\$)	20	(0.015)	(0.019)

Balance sheet – assets



Amounts in US\$ '000	notes	June 30, 2025	December 31, 2024
Non-current assets			
Intangible assets	7	135,901	61,039
Property, plant and equipment		7,965	7,752
Right-of-use assets		16,601	16,382
Long-term prepayments		95	90
Deferred tax assets	14	31,200	30,544
Investment accounted for using the equity method	13	1,006	466
Investment in equity instruments designated as at FVTOCI	13	1,392	_
Investment in debt instruments designated as at FVTPL	13	4,300	3,767
Restricted cash	17	2,023	1,505
Total non-current assets		200,483	121,545
Current assets			
Inventories	15	63,715	55,724
Trade and other receivables		53,327	54,823
Restricted cash	17	2,726	_
Marketable securities	16	33,917	112,949
Cash and cash equivalents	17	92,091	54,944
Total current assets		245,776	278,440
Total assets		446,259	399,985

Balance sheet – liabilities



Equity			
Share capital		7,821	7,769
Share premium		491,853	488,990
Other reserves		25,908	(209)
Accumulated deficit		(286,031)	(275,489)
Shareholders' equity	17	239,551	221,061
Non-current liabilities			
Convertible bonds	19	91,268	78,154
Lease liabilities		27,498	26,968
Total non-current liabilities		118,766	105,122
Current liabilities			
Convertible bonds	19	5,105	4,245
Trade and other payables		78,382	66,611
Lease liabilities		4,455	2,946
Total current liabilities		87,942	73,802
Total equity and liabilities		446,259	399,985

Cash flow (1/2)



Amounts in \$'000	1H 2025	1H 2024
Profit (loss) before tax	(4,709)	(16,684)
Adjustments to reconcile net profit (loss) to net cash used in operating activities:		
Depreciation, amortization, impairment of non-current assets	5,284	5,628
Equity settled share based payments	6,052	5,687
Fair value loss (gain) on revaluation	_	(5,138)
Loss (gain) on disposal of leases	(10)	_
Other finance income	(1,263)	(2,935)
Other finance expenses	9,650	4,450
Share of net losses (profits) in associates using the equity method	(8)	834
Operating cash flows before changes in working capital	14,996	(8,158)
Changes in working capital:		
Inventories	(309)	(3,115)
Trade and other receivables	2,359	(4,963)
Payables and other current liabilities	1,031	(2,255)
Restricted cash	(3,052)	_
Total changes in working capital	29	(10,333)
Interest received	1,273	2,370
Income taxes received (paid)	(4,323)	(4,747)
Net cash flows generated from (used in) operating activities	11,975	(20,868)

Cash flow (2/2)



Capital expenditure for property, plant and equipment	(410)	(294)
Investment intangible assets	(6)	_
Disposal of investment designated as at FVOCI	_	1,964
Investment in associates using the equity method	(429)	_
Purchases of marketable securities	_	(112,453)
Proceeds from sale of marketable securities	84,967	147,841
Acquisition of a subsidiary, net of cash acquired	(57,476)	_
Net cash flows generated from (used in) investing activities	26,646	37,058
Payment of lease liabilities	(1,781)	(1,513)
Interests on lease liabilities	(562)	(580)
Net proceeds of issued convertible bonds		104,802
Repurchase of convertible bonds		(134,922)
Interests on convertible bonds	(2,450)	(2,024)
Settlement of share based compensation awards	1,287	3,462
Acquisition of non-controlling interests	(5,970)	_
Net cash flows generated from (used in) financing activities	(9,476)	(30,775)
Increase (decrease) of cash	29,145	(14,585)
Exchange rate effects	8,002	(14)
Cash and cash equivalents at January 1	54,944	61,741
Total cash and cash equivalents at June 30	92,091	47,142