



Pharming Group N.V.

1Q 2023 Financial Results

May 11, 2023

NASDAQ: PHAR | Euronext Amsterdam: PHARM

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 and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2022 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2022, 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.



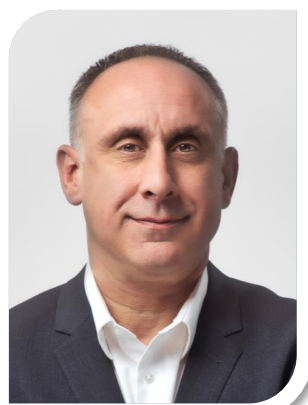
 **SPEAKERS**



Sijmen de Vries, MD
Chief Executive Officer



Anurag Relan, MD
Chief Medical Officer



Stephen Toor
Chief Commercial Officer



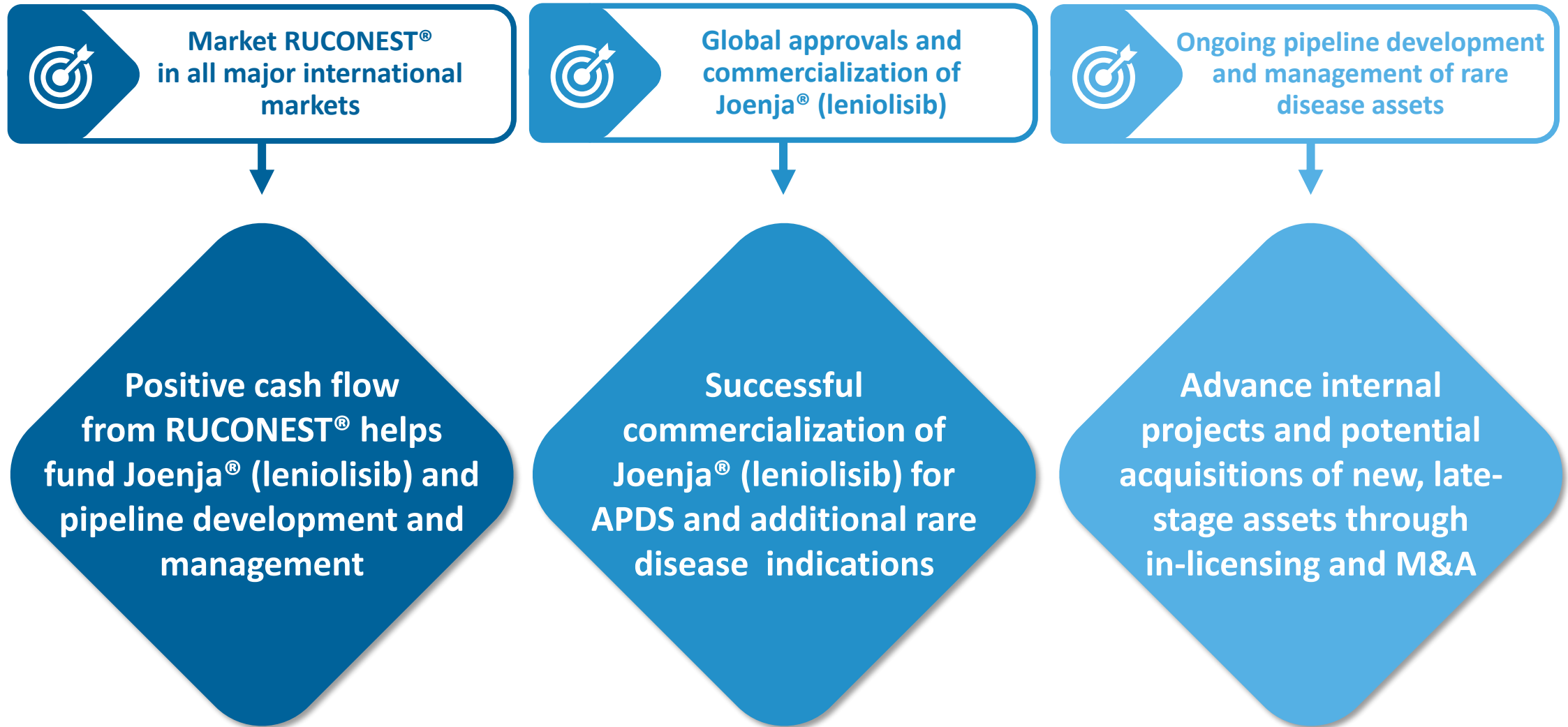
Jeroen Wakkerman
Chief Financial Officer



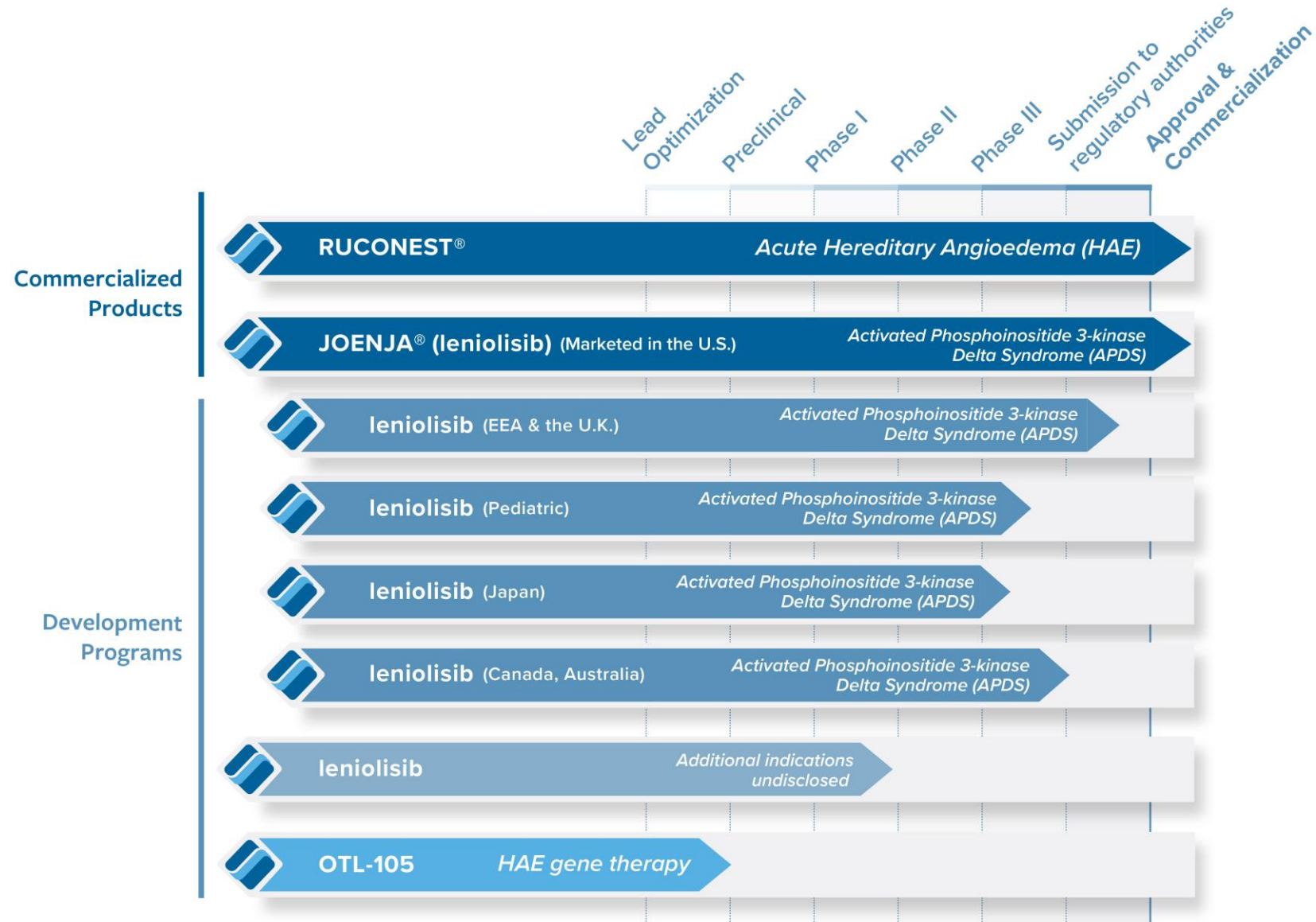
Sijmen de Vries, MD
Chief Executive Officer

Introduction





Pipeline – multiple commercial stage rare disease products





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



Outlook of 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®¹



93% of attacks were stopped with
RUCONEST® for at least three days²



Patients are well managed and feel
confident to administer treatment
themselves³



Dedicated sales force and marketing in US, EU, and MENA



Market access teams



Patient support and reimbursement teams



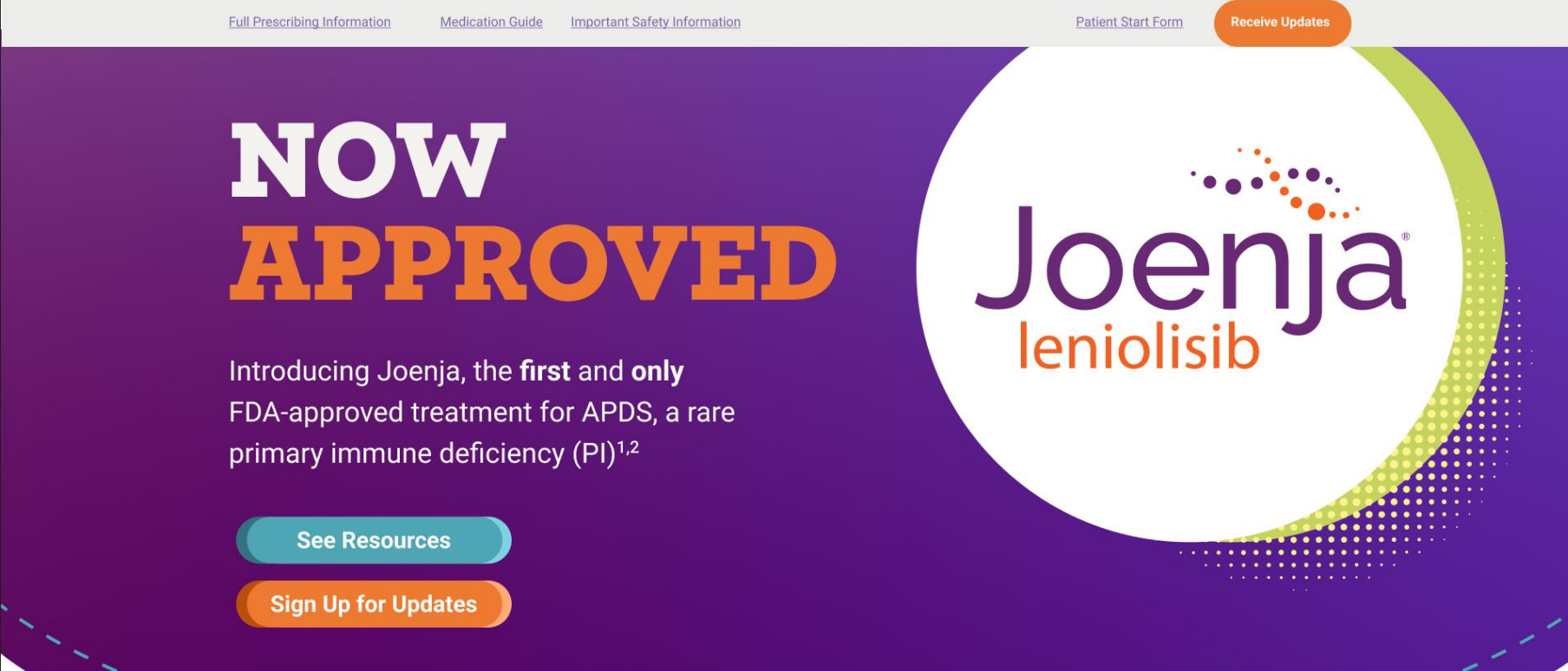
Disease educators and specialists for APDS and HAE



Medical Affairs teams



High conference penetration & Support for educational KOL speaker programs



Full Prescribing Information Medication Guide Important Safety Information Patient Start Form [Receive Updates](#)

NOW APPROVED

Introducing Joenja, the **first and only** FDA-approved treatment for APDS, a rare primary immune deficiency (PI)^{1,2}

[See Resources](#)

[Sign Up for Updates](#)

Joenja[®]
leniolisib



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*

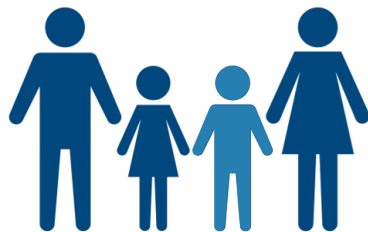
To date, Pharming has identified >500 of these patients

(as of December 2022 for US, Europe, UK, Japan, Canada, Australia)



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



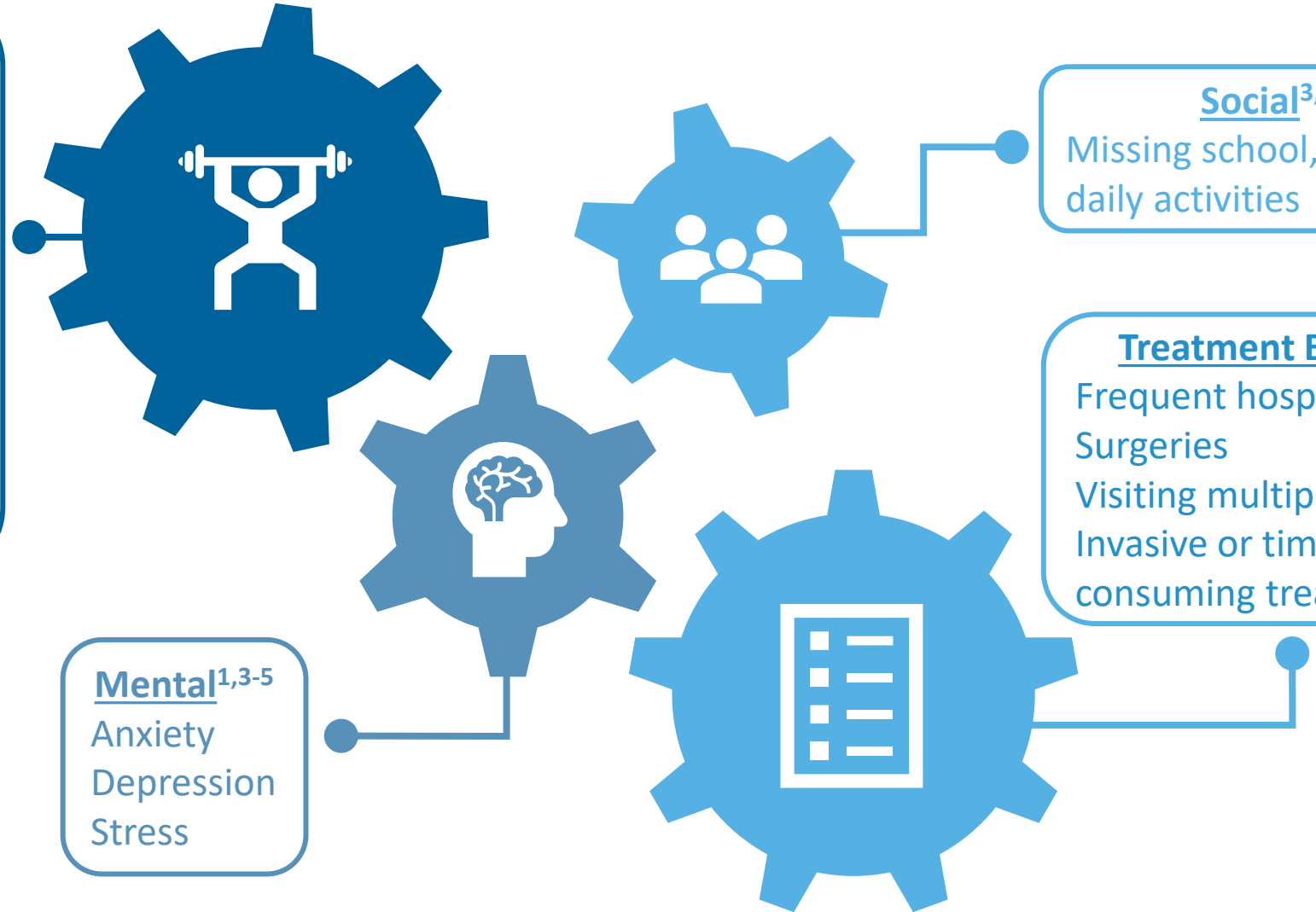
A genetic test can provide a definitive diagnosis of APDS

*Size based on estimate of 1.5 APDS patients per million (based on available literature) for US, Europe, UK, Japan, Canada and Australia

APDS can impact many facets of life

Physical^{1,2}

Frequent infections
Swollen glands
Shortness of breath
Coughing/wheezing
Chest or joint pain
Fatigue
Inability to exercise
Hearing loss
Diarrhea
Skin problems



Social^{3,4}

Missing school, work, or daily activities

Treatment Burden¹⁻⁴

Frequent hospitalizations
Surgeries
Visiting multiple doctors
Invasive or time-consuming treatments

Mental^{1,3-5}

Anxiety
Depression
Stress

APDS, activated phosphoinositide 3-kinase δ syndrome.

1. Coulter TI, et al. *J Allergy Clin Immunol.* 2017;139(2):597-606. 2. Elkaim E, et al. *J Allergy Clin Immunol.* 2016;138(1):210-218. 3. Rider NL, et al. *J Clin Immunol.* 2017;37(5):461-475.

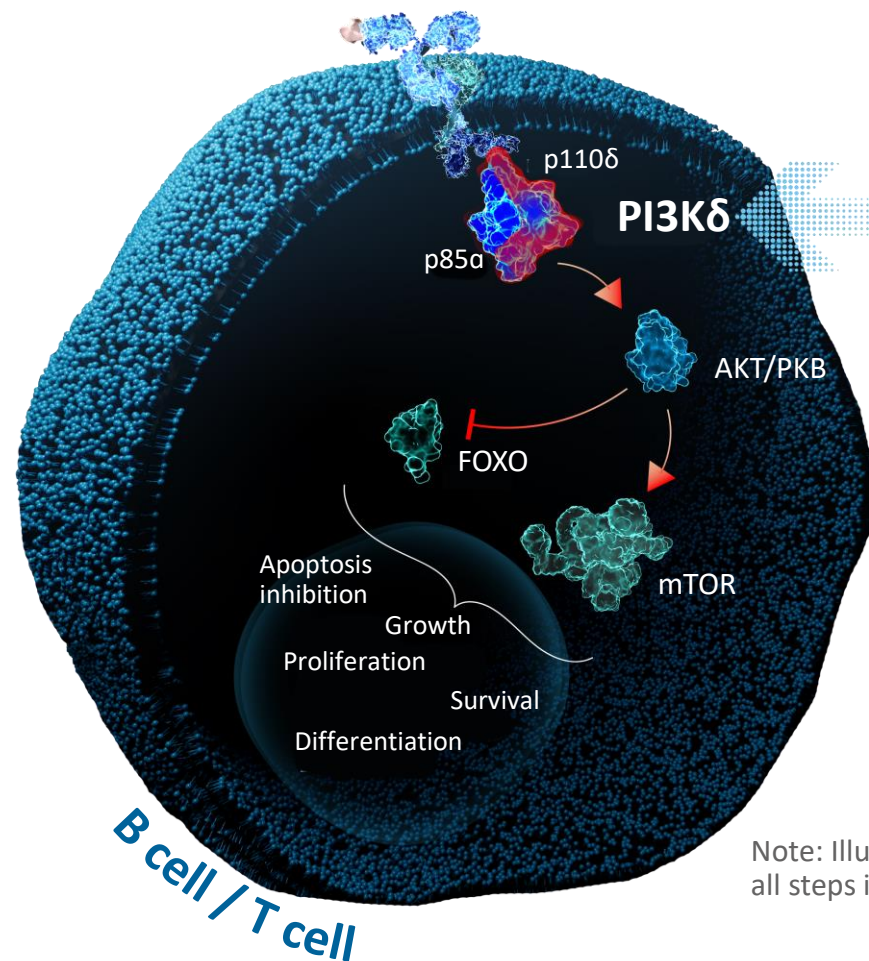
4. Jiang F, et al. *Allergy Asthma Clin Immunol.* 2015;11:27. 5. Kuburovic NB, et al. *Patient Prefer Adherence.* 2014;8:323-330.

Genetic defect leads to PI3K δ hyperactivity, disrupting immune cell balance

Hyperactive PI3K δ results in dysregulated B and T cell development¹⁻³

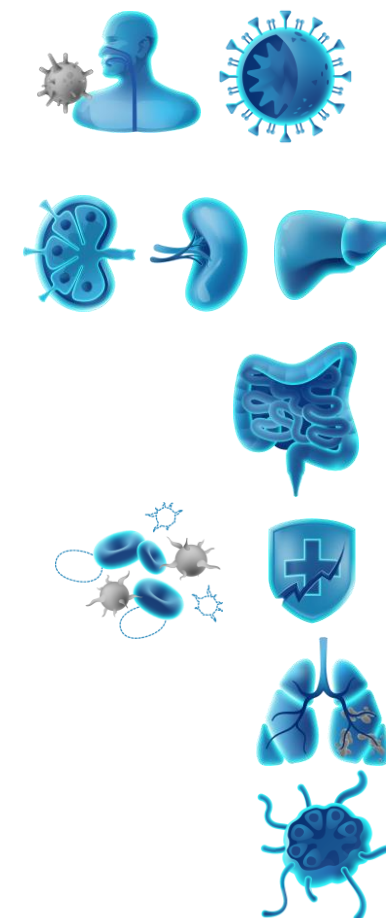


Immune imbalance leads to diverse signs and symptoms^{1,4-6}



The PI3K δ enzyme is at the beginning of a complex signaling pathway

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



Severe, recurrent, persistent infections

- Sinopulmonary
- Herpesvirus (especially EBV and CMV)

Lymphoproliferation

- Lymphadenopathy
- Splenomegaly/hepatomegaly
- Nodular lymphoid hyperplasia

Enteropathy

Autoimmunity

- Cytopenias
- Autoimmune disorders
- Autoinflammatory disorders

Bronchiectasis

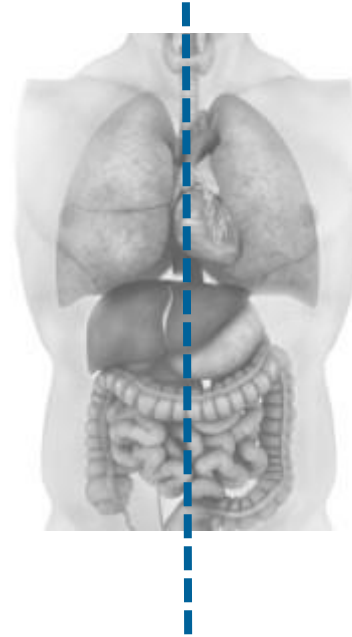
Lymphoma

FOXO, forkhead box O; mTOR, mammalian target of rapamycin; PI3K δ , phosphoinositide 3-kinase delta; PKB, protein kinase B.

1. Lucas CL, et al. *Nat Immunol.* 2014;15(1):88-97. 2. Fruman DA, et al. *Cell.* 2017;170(4):605-635. 3. Okkenhaug K, Vanhaesebroeck B. *Nat Rev Immunol.* 2003;3(4):317-330. 4. Coulter TI, et al. *J Allergy Clin Immunol.* 2017;139(2):597-606. 5. Elkaim E, et al. *J Allergy Clin Immunol.* 2016;138(1):210-218. 6. Jamee M, et al. *Clin Rev Allergy Immunol.* 2020;59(3):323-333.

Immune Deficiency

- Antimicrobial prophylaxis
- Immunoglobulin replacement therapy



Immune Dysregulation

- Corticosteroids
- Other immunosuppressants
- mTOR inhibitors

None of these therapies are FDA-approved for APDS treatment

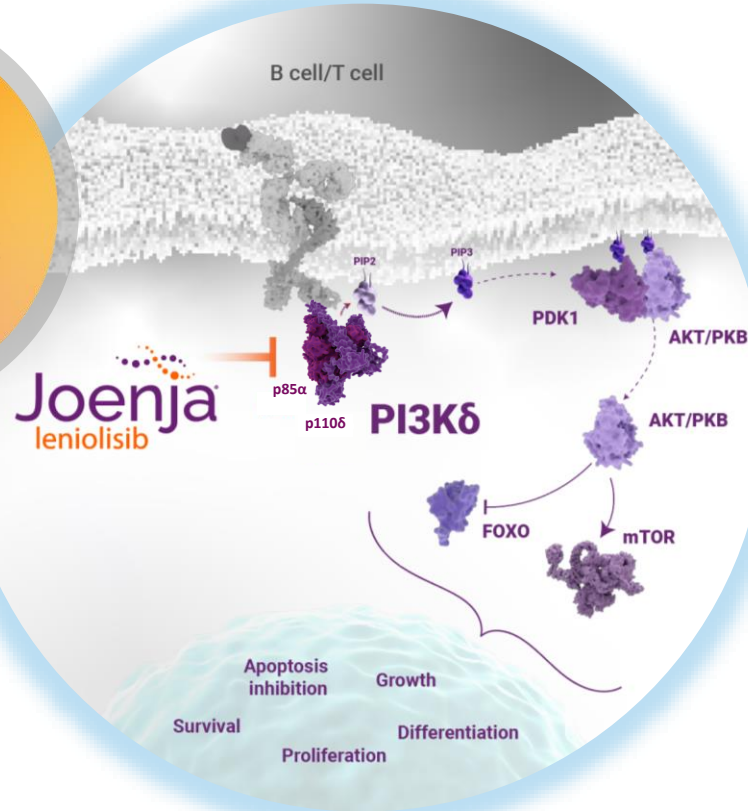
Hematopoietic stem cell transplant

APDS, activated phosphatidylinositol 3-kinase δ syndrome; IRT, immunoglobulin replacement therapy; mTOR, mammalian target of rapamycin; PI, primary immunodeficiency; PIRD, primary immune regulatory disorder.

1. Coulter TI, et al. *J Allergy Clin Immunol.* 2017;139(2):597-606. 2. Elkaim E, et al. *J Allergy Clin Immunol.* 2016;138(1):210-218. 3. Chan AY, et al. *Front Immunol.* 2020;11:239. 4. Chinn IK, et al. *J Allergy Clin Immunol.* 2020;145(1):46-69.

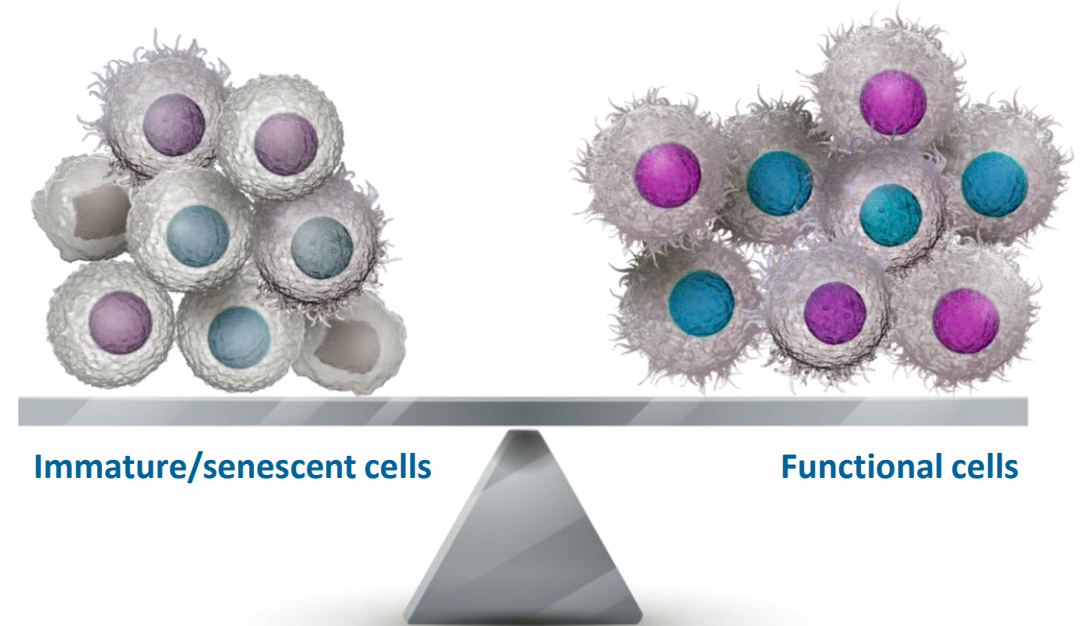
Joenja[®]: immune modulator that targets the root cause of APDS

JOENJA WAS DESIGNED TO TARGET THE ROOT CAUSE OF APDS TO HELP NORMALIZE THE HYPERACTIVE PI3K δ PATHWAY¹⁻⁵



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

Joenja[®] facilitates a balanced PI3K δ pathway to support proper immune function⁶



This is a graphical representation of a complex biological process.

AKT/PKB, protein kinase B; FOXO, forkhead box O; mTOR, mammalian target of rapamycin; p85 α , the regulatory subunit of the PI3K δ enzyme; p110 δ , the catalytic subunit of the PI3K δ 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®: 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 (PI3Kδ) 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

Pharming is well-positioned to hit the ground running with Joenja®





Overview of Prescribing Information

Indication Statement	JOENJA is a kinase inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) in adult and pediatric patients 12 years of age and older.
Contraindications	None
Boxed Warning	None
Risk Evaluation and Mitigation Strategy	None
Dosing and Administration	Verify pregnancy status in females of reproductive potential prior to initiating treatment. Recommended dosage: 70 mg administered orally twice daily approximately 12 hours apart, with or without food, in adult and pediatric patients 12 years of age and older and weighing ≥ 45 kg
Warnings and Precautions	Embryo-Fetal Toxicity: JOENJA may cause fetal harm. Advise patients of the potential risk to a fetus and to use effective contraception. Vaccinations: Live, attenuated vaccinations may be less effective if administered during JOENJA treatment.
Adverse Reactions	Most common adverse reactions (incidence $>10\%$) were headache, sinusitis, and atopic dermatitis.

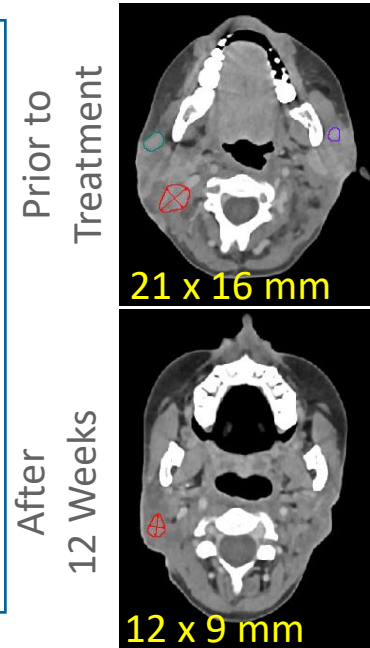
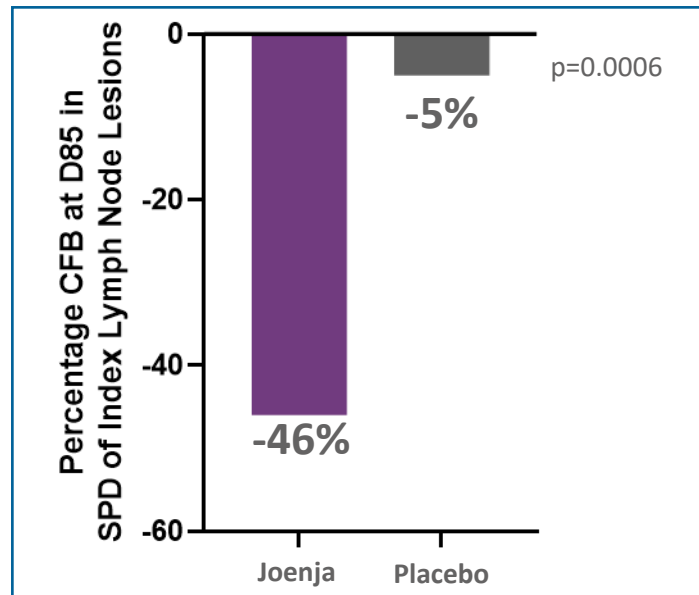
Joenja® addresses the underlying cause of APDS to help restore immune balance – Phase 3 co-primary endpoints

At 12 weeks Joenja® decreased lymphadenopathy and increased naïve B cells

Change from baseline in index nodes*

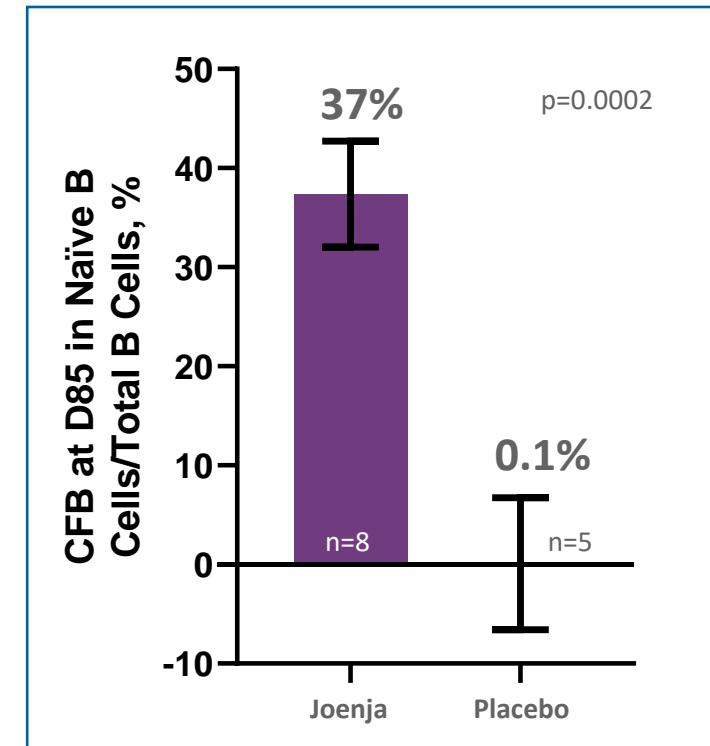
Log ₁₀ -transformed SPD of index lesions	Joenja (n=18)	Placebo (n=8)
Baseline mean (SD)	3.03 (0.42)	3.05 (0.39)
Change from baseline, LS mean (SE)	-0.27 (0.04)	-0.02 (0.05)
Difference vs placebo (95% CI)		-0.25 (-0.38, -0.12)

Immune Dysregulation



Change from baseline in naïve B cells†

Immune Deficiency



Data were analyzed using an ANCOVA model with treatment as a fixed effect and baseline as a covariate. Use of glucocorticoids and IRT at baseline were both included as categorical (Yes/No) covariates. Baseline is defined as the arithmetic mean of the baseline and D1 values when both are available, and if either baseline or the D1 value is missing, the existing value is used. P-value is 2-sided. Least square means are graphed. Error bars are standard error of the mean.

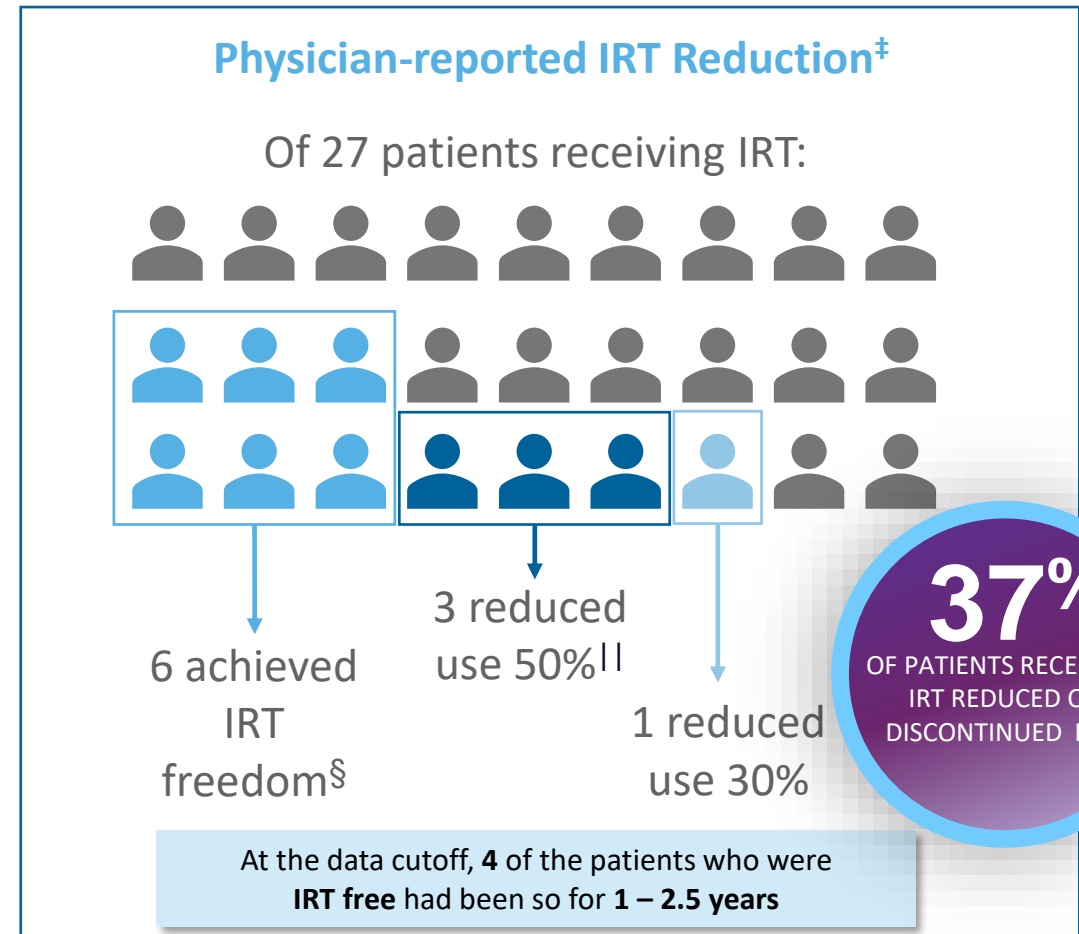
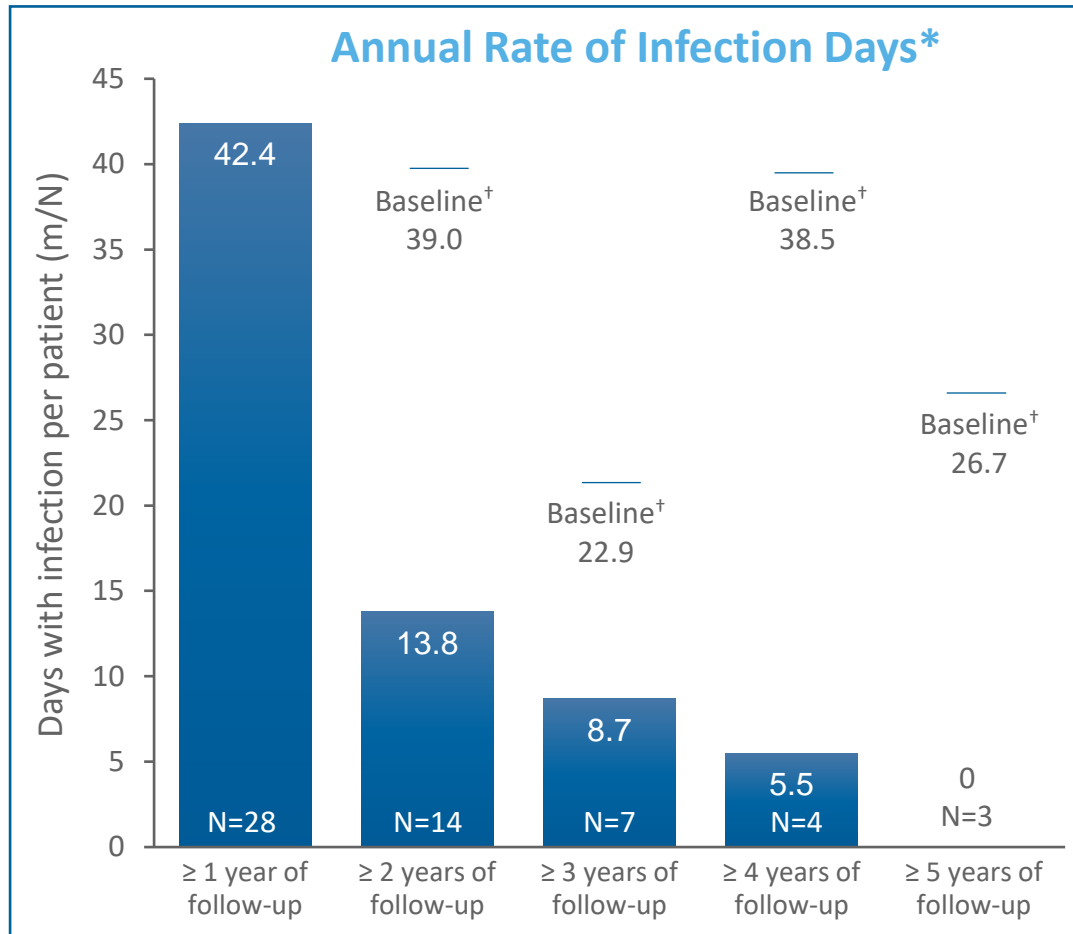
*The analysis excluded 2 patients from each treatment group due to protocol deviations and 1 Joenja patient having complete resolution of the index lesion identified at baseline.

†Out of 27 patients in the PD analysis set, 13 patients met the analysis requirements, including having a percentage of <48% of naïve B cells at baseline, to form the B-PD analysis set.

Joenja [package insert]. Leiden, The Netherlands: Pharming Technologies B.V.; 2023.

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

Open-label extension interim analysis of days spent with infections and IRT reduction



Although safety was the primary objective of the open-label study, this post hoc analysis from the open-label study was not powered to provide any statistical significance of efficacy and therefore no conclusions should be drawn.

*Infections that developed during the study were reported as adverse events. Investigators were requested to inquire about signs and symptoms of infections at each visit, with a particular focus on bacterial enterocolitis. Patients were not provided an infection diary to document infections occurring between visits. One patient was excluded from the analysis due to an incorrect year that was recorded for an infection.
 †Baseline infections are each group's year 1 annual rate of infections. N values changed because patients were in the OLE for different lengths of time. ‡Data on concomitant medication usage was reported at each patient visit. §One patient had a subsequent one-time dose. ||One patient achieved IRT freedom for 3 months but subsequently restarted IRT.
 IRT, immunoglobulin replacement therapy; m, number of infection days; N, number of patients in follow-up category.
 Rao VK, et al. Poster presented at: 64th Annual American Society of Hematology Annual Meeting; December 10-13, 2022; New Orleans, LA.
 Please see Important Safety Information and full Prescribing Information available at joenja.com



Joenja[®] launched & reimbursed commercial shipments to patients commenced early April



Europe – CHMP opinion on MAA expected 2H23 (approval ~ 2 months later)



UK – MHRA filing expected 2H23 (approval ~2 months later)



Initiation of Japan clinical study in 1H23



Development ongoing for pediatric patients 4 to 11 years old



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



Partnership with Orchard Therapeutics to develop *ex vivo* autologous hematopoietic stem cell gene therapy for HAE



Progress continues in preclinical study



Good progress on developing the lentiviral vector to enhance C1-inhibitor expression, now testing in preclinical HAE disease models



Anticipate providing further updates as we move towards preparing an Investigational New Drug (IND) filing



Stephen Toor
Chief Commercial Officer
Commercial update





Strong underlying in-market demand for RUCONEST® including high, double digit, new patient enrollments in 1Q23



Disruptions have since resolved, but impacted February sales



Disruptions in reimbursement for some patients on U.S. government insurance programs impacted the entire HAE market in 1Q23



Pharming has since seen a recovery in sales



These market-wide factors caused a delay in shipments to patients



We are maintaining our outlook for low single digit RUCONEST® revenue growth in 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[®] 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

- ◆ Strong start to our early April product launch
- ◆ Pharming continues to engage with both national and regional payers
- ◆ First commercial shipment of Joenja[®], with full reimbursement, ~two weeks after FDA approval
- ◆ To date, we have 23 U.S. patients on paid therapy with Joenja[®]
- ◆ The sales team continue to drive new patient enrollments
- ◆ Good progress moving EAP and OLE patients to commercial drug
- ◆ First revenues will be seen in the second quarter



Pharming® | 35 years



CFO



Jeroen Wakkerman
Chief Financial Officer

Financials

Financial highlights: 1Q 2023 vs 1Q 2022

TOTAL REVENUES
1Q 2022

US\$46.6 million



TOTAL REVENUES
1Q 2023

US\$42.5 million



GROSS PROFIT
1Q 2022

US\$41.7 million



GROSS PROFIT
1Q 2023

US\$38.5 million



OPERATING COSTS
1Q 2022

US\$39.8 million



OPERATING COSTS
1Q 2023

US\$52.7 million



OPERATING PROFIT
1Q 2022

US\$2.8 million



OPERATING PROFIT
1Q 2023

US\$(13.7) million



NET PROFIT
1Q 2022

US\$3.5 million



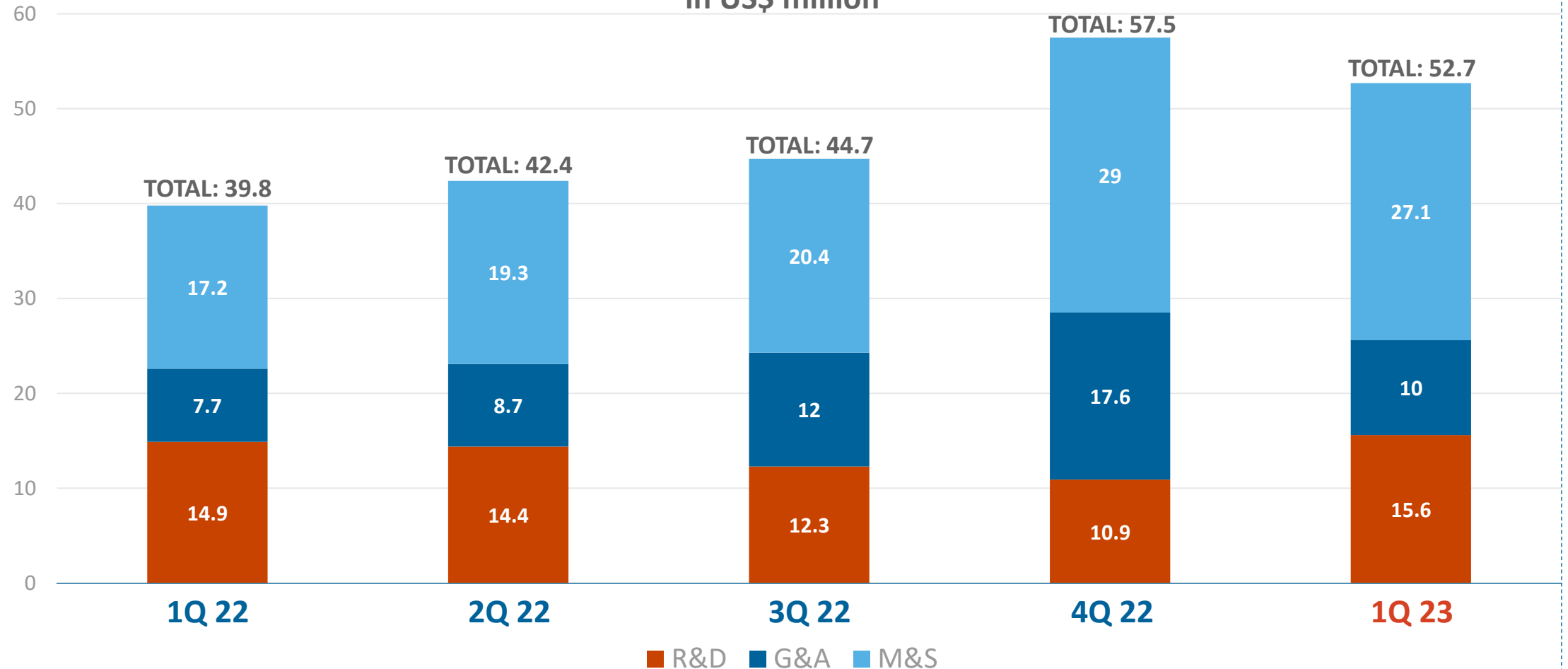
NET PROFIT
1Q 2023

US\$(12.2) million

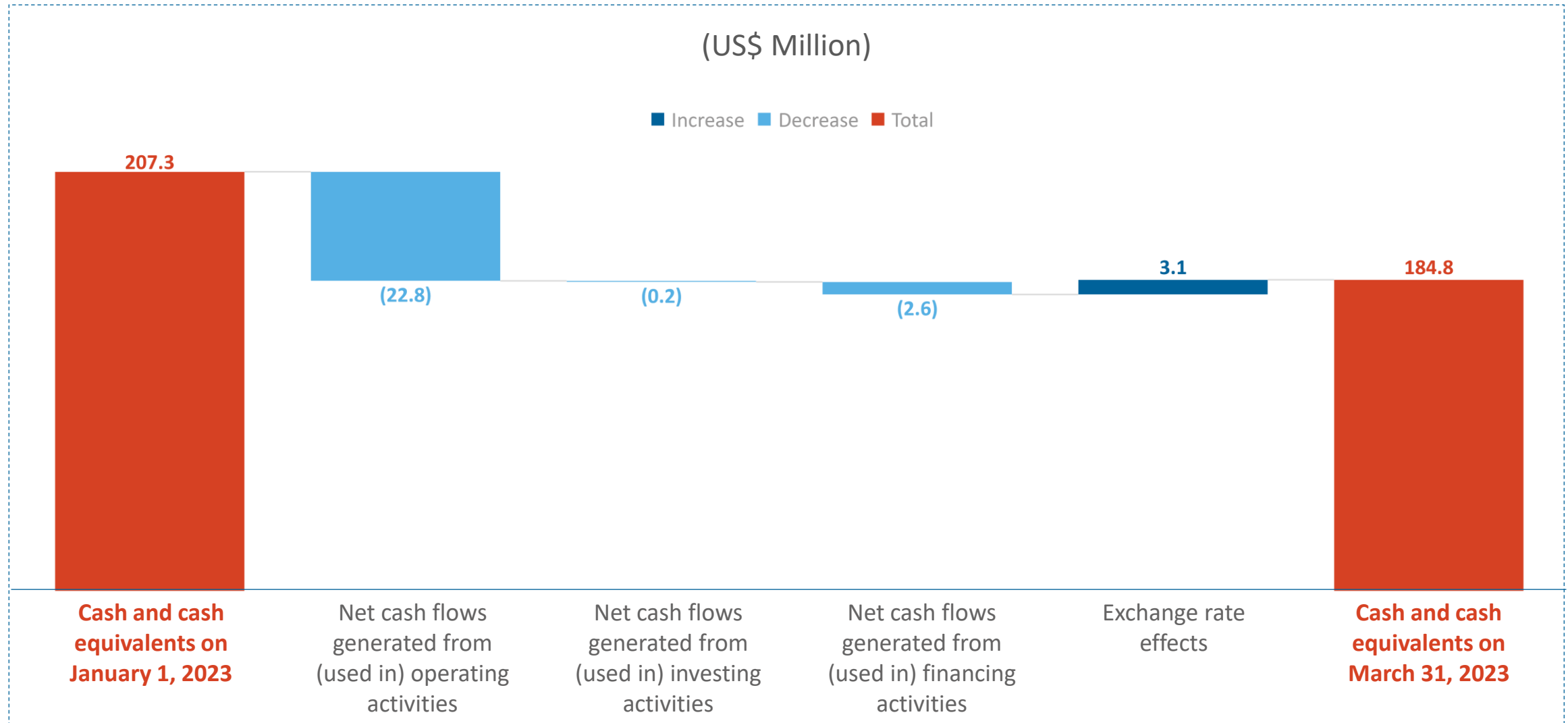


Continued investment in the launch of Joenja

Cost category breakdown 1Q22 – 1Q23
in US\$ million



1Q 2023: Cashflow January 1, 2023 – March 31, 2023





Continued low single-digit growth in RUCONEST® revenues



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



Positive CHMP opinion in 2H 2023, marketing authorization in Europe ~2 months later*



File leniolisib with UK's MHRA following ECDRP route*



Continued operating cost investments to accelerate future growth



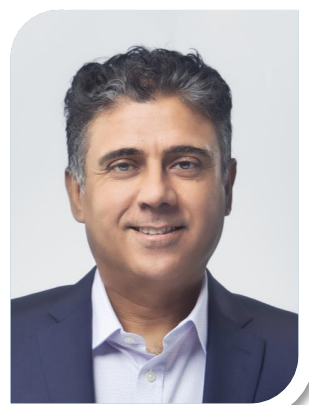
Further details on our plans to develop leniolisib in additional indications to be provided in 2H 2023



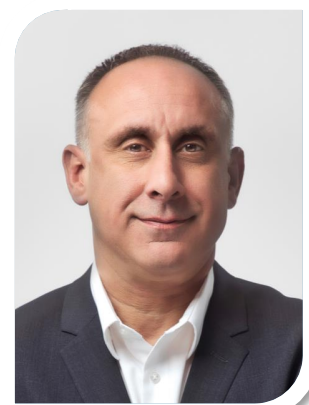
Investment and continued focus on potential acquisitions and in-licensing of late-stage opportunities in rare diseases



Sijmen de Vries, MD
Chief Executive Officer



Anurag Relan, MD
Chief Medical Officer



Stephen Toor
Chief Commercial Officer



Jeroen Wakkerman
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 | investor@pharming.com

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

Bloomberg: **PHAR.AS**



Pharming Group N.V.

Appendix



Statement of profit and loss

Amounts in US\$ '000	YTD 2023	YTD 2022
Revenues	42,541	46,617
Costs of sales	(4,075)	(4,877)
Gross profit	38,466	41,740
Other income	579	873
Research and development	(15,620)	(14,863)
General and administrative	(9,981)	(7,728)
Marketing and sales	(27,107)	(17,197)
Other Operating Costs	(52,708)	(39,788)
Operating profit (loss)	(13,663)	2,825
Other finance income	123	3,228
Other finance expenses	(2,795)	(1,379)
Finance result, net	(2,672)	1,849
Share of net profits in associates using the equity method	(339)	(441)
Profit (loss) before tax	(16,674)	4,233
Income tax expense	4,466	(772)
Profit (loss) for the year	(12,208)	3,461
Basic earnings per share (US\$)	(0.019)	0.005
Diluted earnings per share (US\$)	(0.017)	0.005

Amounts in US\$ '000	March 31, 2023	December 31, 2022
Non-current assets		
Intangible assets	75,606	75,121
Property, plant and equipment	10,403	10,392
Right-of-use assets	30,369	28,753
Long-term prepayments	233	228
Deferred tax assets	29,350	22,973
Investment accounted for using the equity method	2,210	2,501
Investments in equity instruments designated as at FVTOCI	585	403
Investment in debt instruments designated as at FVTPL	6,972	6,827
Restricted cash	1,212	1,099
Total non-current assets	156,940	148,297
Current assets		
Inventories	48,127	42,326
Trade and other receivables	32,931	27,619
Restricted cash	218	213
Cash and cash equivalents	184,780	207,342
Total current assets	266,056	277,500
Total assets	422,996	425,797

Equity		
Share capital	7,518	7,509
Share premium	463,222	462,297
Other reserves	(4,906)	(8,737)
Accumulated deficit	(266,491)	(256,431)
Shareholders' equity	199,343	204,638
Non-current liabilities		
Convertible bonds	133,576	131,618
Lease liabilities	31,074	29,843
Total non-current liabilities	164,650	161,461
Current liabilities		
Convertible bonds	1,805	1,768
Trade and other payables	53,254	54,465
Lease liabilities	3,944	3,465
Total current liabilities	59,003	59,698
Total equity and liabilities	422,996	425,797

Amounts in US\$'000	YTD 2023	YTD 2022
Profit (loss) before tax	(16,674)	4,233
<i>Non-cash adjustments:</i>		
Depreciation, amortization, impairment of non-current assets	2,306	2,190
Equity settled share based payments	1,558	1,070
Other finance income	(123)	(3,228)
Other finance expenses	2,795	1,379
Share of net profits in associates using the equity method	339	441
Other	(455)	—
Operating cash flows before changes in working capital	(10,254)	6,085
<i>Changes in working capital:</i>		
Inventories	(5,801)	(2,297)
Trade and other receivables	(5,313)	(1,462)
Payables and other current liabilities	(1,211)	(1,645)
Restricted cash	117	(20)
Total changes in working capital	(12,208)	(5,424)

Interest received (paid)	117	(52)
Income taxes paid	(440)	—
Net cash flows generated from (used in) operating activities	(22,785)	609
Capital expenditure for property, plant and equipment	(215)	(208)
Investment intangible assets	—	(167)
Net cash flows used in investing activities	(215)	(375)
Payment of lease liabilities	(1,312)	(807)
Interests on loans	(2,013)	(2,100)
Proceeds of equity and warrants	695	18
Net cash flows generated from (used in) financing activities	(2,630)	(2,889)
Increase (decrease) of cash	(25,630)	(2,655)
Exchange rate effects	3,068	405
Cash and cash equivalents at 1 January	207,342	191,924
Total Cash and cash equivalents at 31 March	184,780	189,674