Pharming Group N.V.

Oppenheimer 36th Annual Healthcare Life Sciences Conference

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

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 2024 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2024, 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.

Combination of commercial and pipeline assets poised to deliver strong value creation

Pharming®

Commercial

Pipeline

HAE

RUCONEST®
(recombinant C1 esterase inhibitor)
Differentiated value proposition
Highly specific manufacturing process

PIDs
with immune dysregulation

Joenja® (leniolisib) for APDS
Significant near-term catalysts
Up to 100x current prevalence[1]

leniolisib for PIDs / CVID
Phase II trials

> $1B revenue potential

PMD

napazimone (KL1333) for mtDNA mitochondrial disease
Registrational Phase II trial
Positive interim analysis

> $1B revenue potential

*These product candidates are under investigation, and their safety and efficacy have not been established. There is no guarantee that these products will receive health authority approval or become commercially available for the uses being investigated

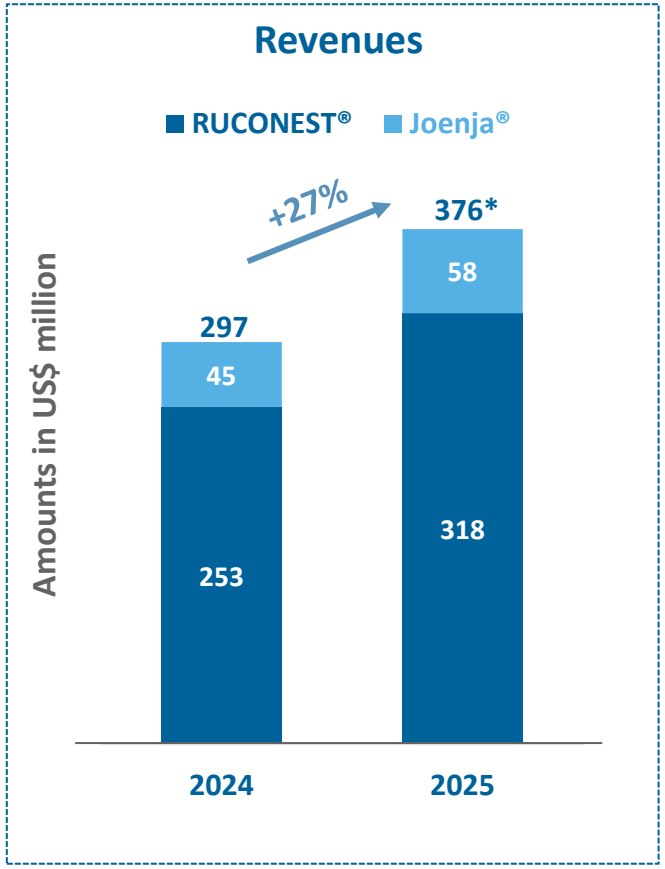
HAE: Hereditary Angioedema, **PIDs:** Primary Immunodeficiencies, **PMD**: Primary Mitochondrial Disease, **CVID:** Common Variable Immunodeficiency

1. Walsh et al., Scalable generation and functional classification of genetic variants in inborn errors of immunity to accelerate clinical diagnosis and treatment, Cell (2025), https://doi.org/10.1016/j.cell.2025.05.037

Vision for the future

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

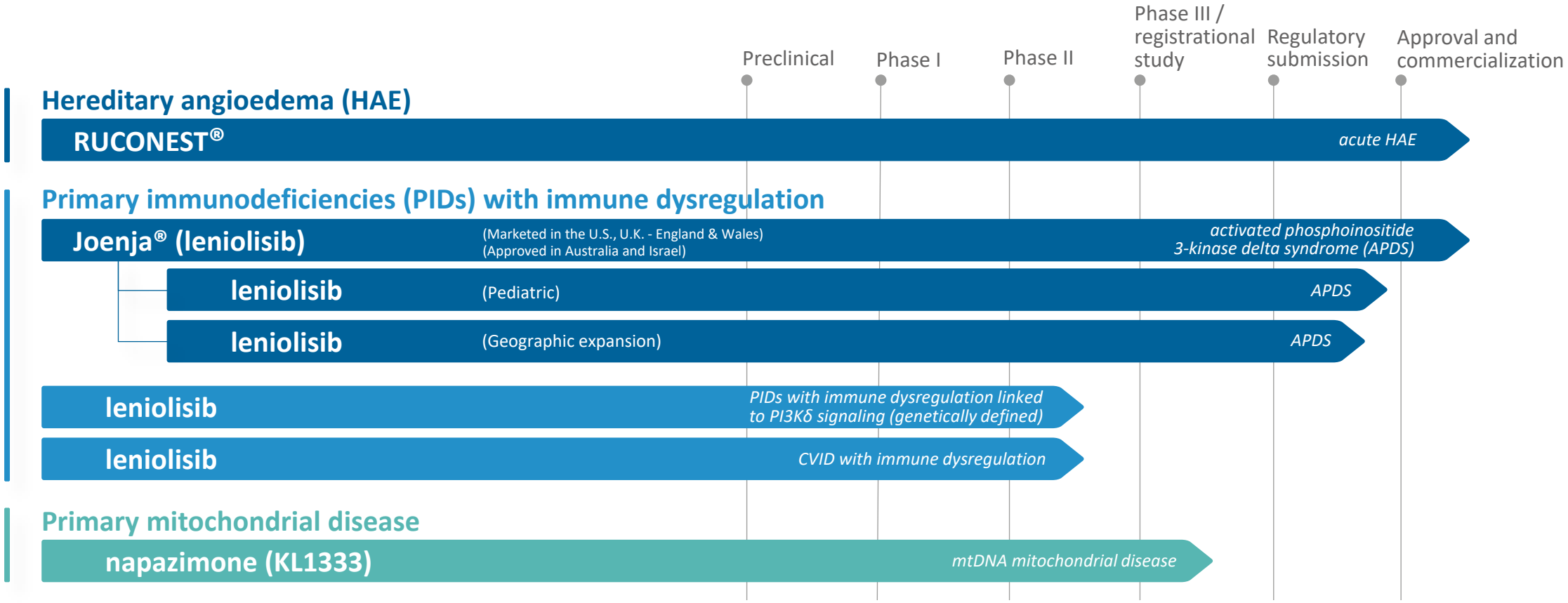
Strong commercial and financial momentum



- Announced preliminary 2025 revenues* of $376M (+ 27%) – above latest guidance
- Results reflects continued growth of RUCONEST® and acceleration in Joenja® APDS uptake
- Significant operating profit $30M and operating cash flow $44M in 9M 2025
- Reiterated $304-308M operating expense guidance for 2025 – committed to cost discipline and deploying capital to high growth initiatives
- Announced 2026 revenue guidance of $405 – 425M (8 – 13% growth)

* 2025 revenues are preliminary and unaudited. Final results may differ and will be reported in the financial results for the fourth quarter and full year 2025, to be published in March 2026.

Diverse rare disease portfolio and pipeline



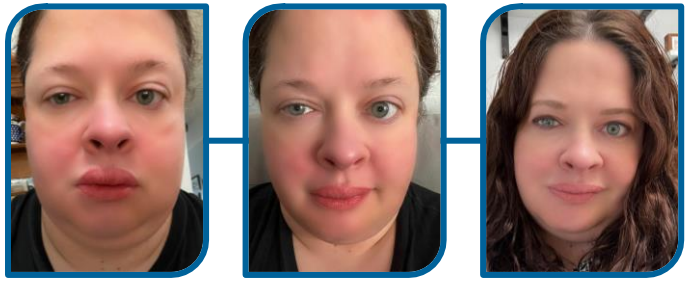
Pharming®

RUCONEST® for HAE

RUCONEST® poised to remain a cornerstone on-demand treatment for difficult to treat HAE patients

- Differentiated value proposition
  - Only recombinant C1-INH protein replacement therapy
  - Targets the root cause of HAE across all pathways
  - IV administration – rapid onset, high dose

- Unique patient population and positioning
  - Type 1, Type 2, and Normal C1-INH HAE patients
  - Mostly used by patients experiencing more severe / frequent attacks, who have failed other on-demand medications[3]



- Highly specific manufacturing process

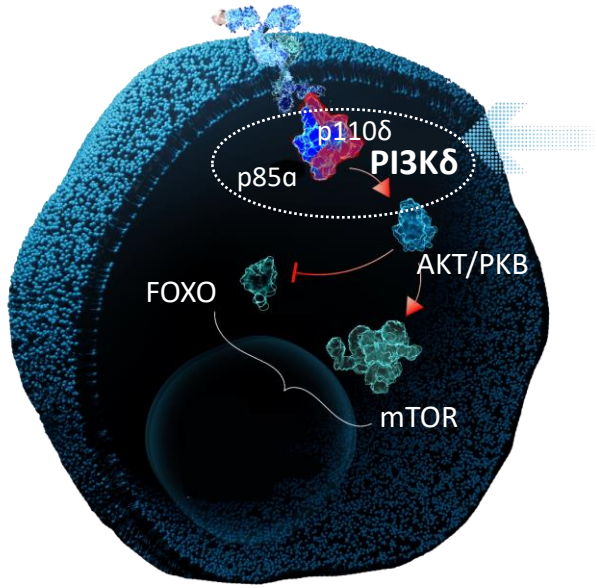
References: 1. RUCONEST®. Prescribing information. Pharming Healthcare Inc; 2020. 2. Bernstein JA, et al. *Ann Allergy Asthma Immunol.* 2017;118(4):452-453. 3. Data on file. Pharming Healthcare Inc; 2019
The most common adverse reactions (incidence ≥2%) were headache, nausea and diarrhea. The most serious adverse reaction reported in clinical trials was anaphylaxis.

Pharming®

Joenja® (leniolisib)
APDS & PID indications

PI3Kδ is a master regulator of the immune system and imbalance contributes to immune dysregulation

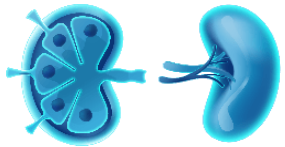
PI3Kδ is a master regulator of the immune system and influences



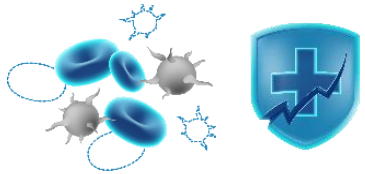
↑Cell trafficking ↑Cell Growth ↑Cell proliferation
↑Cell Differentiation ↑Apoptosis inhibition/survival

Immune dysregulation pathology

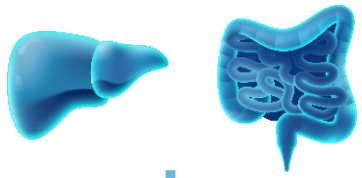
Lymphoproliferation



Autoimmunity



GI Disease



Pulmonary Disease

Shared pathology under the influence of PI3Kδ

- APDS
- Genetic PIDs linked to PI3Kδ
- CVID with immune dysregulation

Reports of Joenja® changing patients' lives

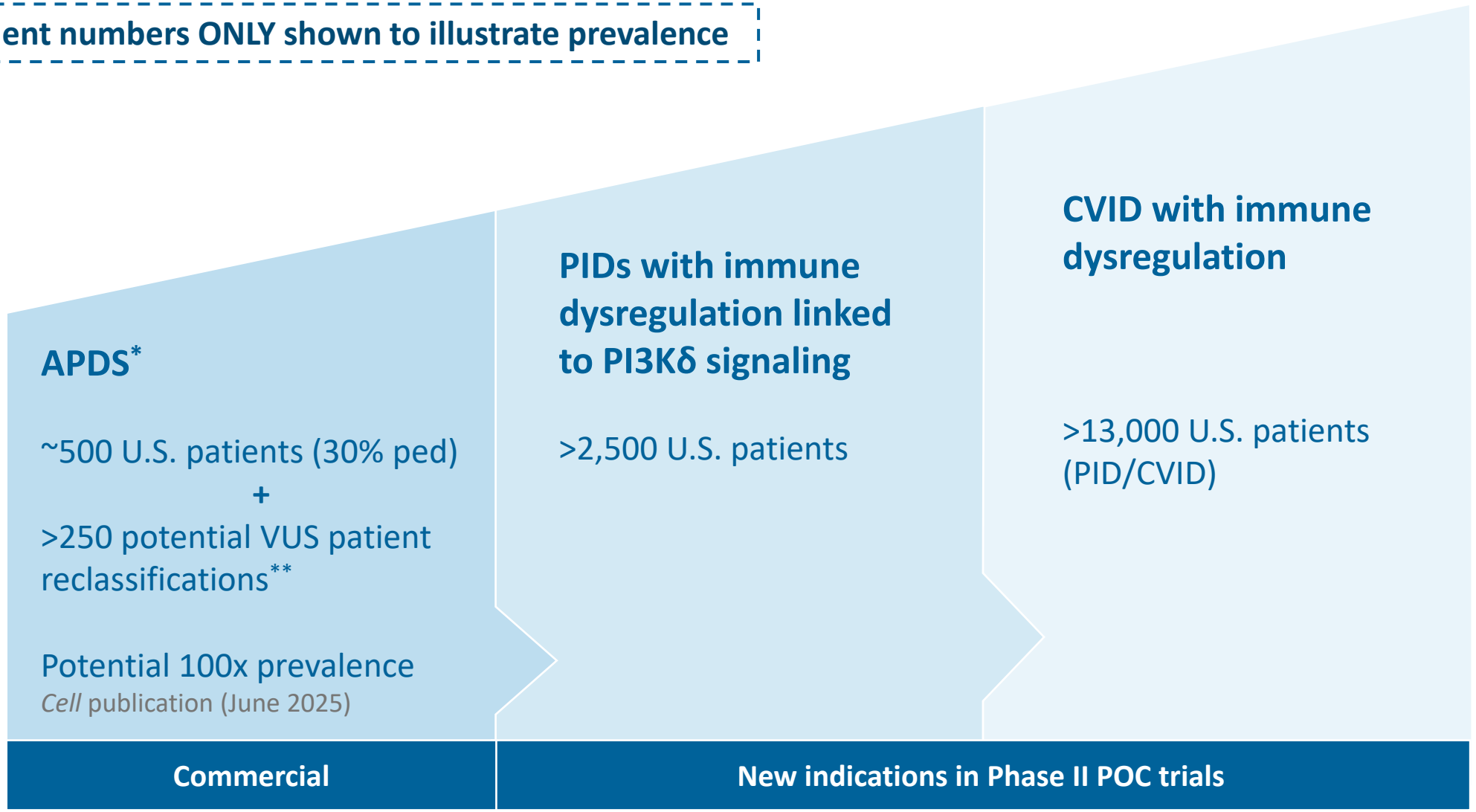
24-year-old male with APDS whose progress was followed in the Joenja® open-label extension study for 6 years

	Before study enrollment	Since starting Joenja treatment
Infections and treatment burden	• Experienced fatigue from IRT infusions, anxiety, and difficulty coping with treatment burden • Hospitalized yearly for infections • Frequently prescribed antibiotics	• Stopped IRT infusions and fatigue got better • No hospitalizations • He had 7 infections, none of which returned • Only doctor he visits regularly is his immunologist
Clinical manifestations	• Low blood platelet counts • Damaged lung airways • Gastrointestinal issues and migraines	• Blood platelet count increased • Damaged lung airways did not get worse

Rao VK, et al. Blood Adv. 2024;8(12):3092-3108. doi:10.1182/bloodadvances.2023011000.

Unlocking Joenja® (leniolisib) growth to realize $1Bn+ potential
Expanding addressable patient population and indications

U.S. patient numbers ONLY shown to illustrate prevalence

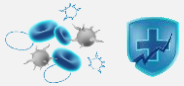


*Initial APDS prevalence estimate ~1.5 patients / million. 270 patients currently identified in the U.S. (73 pediatric), 990 identified globally. (Data as of September 30, 2025)
**Estimate: 20% of >1,400 U.S. patients with a variant of uncertain significance, or VUS, in the PIK3CD and PIK3R1 genes implicated in APDS could ultimately be diagnosed with APDS.

Leniolisib compassionate use experience in PID/CVID with immune dysregulation

- 6 patients treated in Expanded Access Program
- Leniolisib has been generally well-tolerated with signs of improvements:
  - Biomarkers (immunophenotype)
  - Lymphoproliferation
  - End-organ disease
  - Fatigue/well-being
- Median duration of treatment 1.4 years (range 0.5-2.5 years)

Three primary immunodeficiency with immune dysregulation indications driven by dysfunctional B and T cells under the influence of the PI3Kδ pathway

	APDS	Genetic PIDs linked to PI3Kδ	CVID w/immune dysregulation
Prevalence per million population	1.5	7.5	39
Genetic Diagnosis	Yes. (*PIK3CD, PIK3R1*)	Yes. (7 different genes in study)	No. Clinical Diagnosis (80%[1] no genetic drivers**)
Link to PI3Kδ pathway	PI3Kδ Lock & KEY	Mutation linked to PI3Kδ hyperactivity	Cluster of clinical manifestations driven by B & T Cell dysfunction
 Recurrent viral and bacterial infections	**Joenja®** controls B and T cell dysregulation via PI3Kδ pathway, correcting the abnormal immunophenotype	Generally well controlled with immunoglobulin replacement therapy and antibiotics	
 Autoantibodies: Autoimmune cytopenias			
 Lymphoproliferation: lymphadenopathy splenomegaly		**Current SoC Poor disease control** (steroids, immunosuppressants, and immunomodulators)	**Current SoC Poor disease control** (steroids, immunosuppressants, and immunomodulators)
 Lymphocytes infiltrate end-organs: lung, GI tract, liver,			
 Malignancy: Lymphomas			
Development status	Approved	Phase II POC trial (2H26 readout)	Phase II POC trial (2H26 readout)

1-Data on file –Pharming systematic literature review

**NFKB1 CTLA4, PTEN, FAS, SOCS1, NRAS/KRAS (total prevalence 7.5/million)*

*** (20% genetically driven including NFKB1, CTLA4 and PTEN variants which have a combined prevalence of 4.5/million and have been included in the total prevalence of CVID with APDS like endotype of 39/million)*

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Napazimone (KL1333)
mtDNA Mitochondrial Disease

Napazimone (KL1333) for mtDNA-driven primary mitochondrial disease
Aiming for the first disease-modifying treatment

Napazimone (KL1333) targets underlying pathology

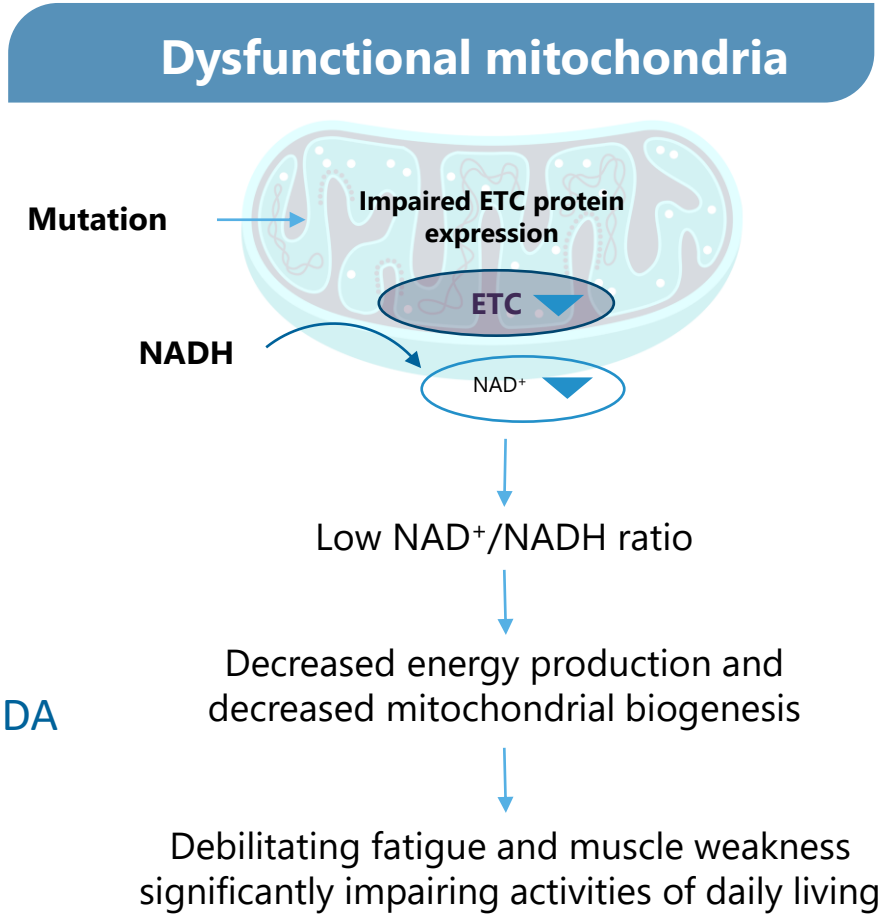
- Normalizes NAD+/NADH ratio and mitochondrial function, with evidence from in vitro data, animal models, and in patients treated with KL1333

Significant patient population

- >30,000 diagnosed patients with mtDNA disorders[1]
- Majority of patients treated in centers of excellence[2]

Registrational clinical study underway

- Clinically-relevant Fatigue, Sit-to-Stand endpoints supported by FDA
- Positive interim analysis – both endpoints cleared futility
- Expect readout in 2027 and FDA approval end of 2028

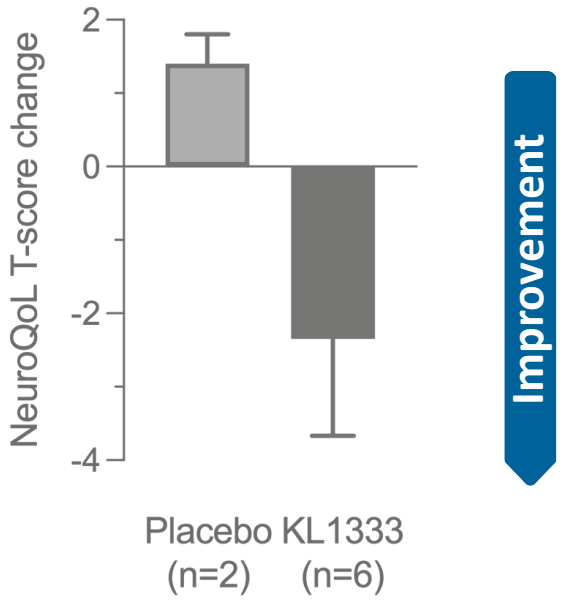


1. In US, EU4 and UK. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome.
2. UNITED MITOCHONDRIAL DISEASE FOUNDATION, Voice of the Patient Report, 2019.

In a Phase Ia/b study, napazimone (KL1333) demonstrated positive changes in outcome measures

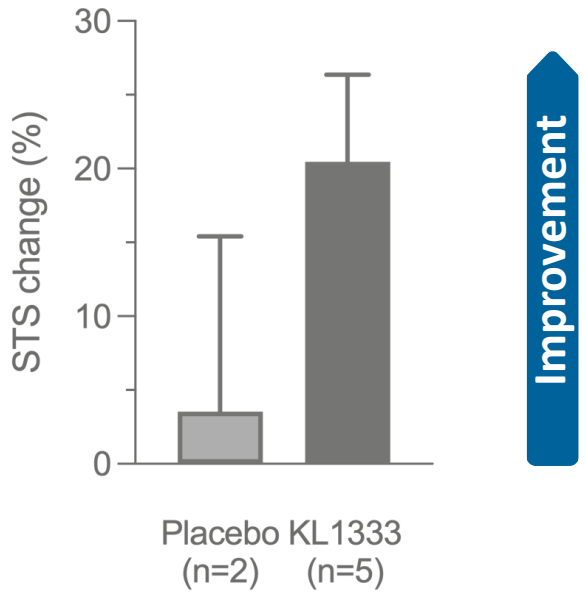
Reduction of fatigue

Changes from baseline to day 10[1]



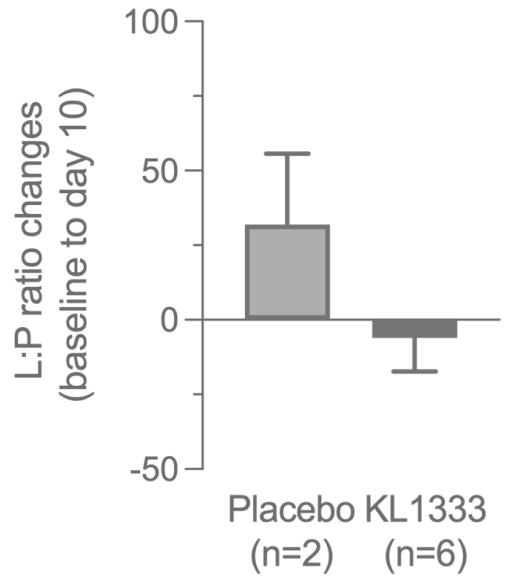
Improvement in muscle function

Changes from baseline to day 10[1]



Biomarker changes

Lactate: Pyruvate ratio[1]



1. Pizzamiglio C et al., Optimizing rare disorder trials: a phase 1a/1b randomized study of KL1333 in adults with mitochondrial disease, Brain 2025;148(1):39-46 https://pmc.ncbi.nlm.nih.gov/articles/pmid/39657714/

Napazimone (KL1333) – FALCON study positive interim analysis

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Pivotal FALCON Study

WAVE 1 – Fully enrolled

- 40 patients recruited across six countries (U.S., UK, France, Spain, Belgium, Denmark)
- Interim analysis at 24 weeks

WAVE 2 – Enrolling

- 180 total patients treated for 48 weeks
- All Wave 1 sites + three new sites active (n=20)
- Planning 40+ total sites, with significant expansion in US
- Readout anticipated 2027

Interim Futility Analysis

Positive outcome achieved, with both primary endpoints passing futility

- Promising differences favoring the active arm vs. placebo for both primary efficacy endpoints; if trends continue consistently, we expect a successful result at the completion of this trial
- Data monitoring committee (DMC) concluded:
  - Safety and tolerability profile acceptable
  - No changes to study design
  - 180 total patients confirmed in the study

Executing on high value rare disease pipeline

APDS

Leniolisib sNDA for 4-11 yo APDS patients – requested Type A meeting
Responded to CHMP (EMA) questions – expect March opinion with potential 1H 2026 approval
Japan and other regulatory reviews on track for 2026 approvals

PIDs with immune dysregulation

Genetic PID and CVID phase II POC trials
on track for 2H 2026 read-outs

PMD

Napazimone (KL1333) pivotal trial
Over 20 sites actively recruiting – site footprint expanding during 1H 2026
on track for late 2027 read-out

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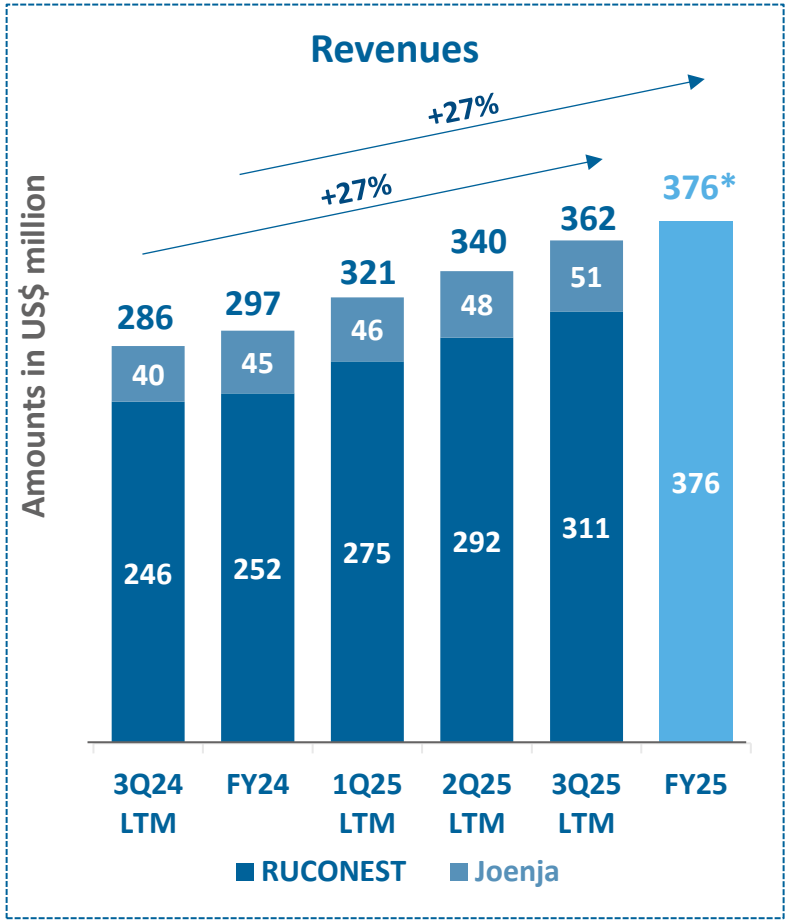
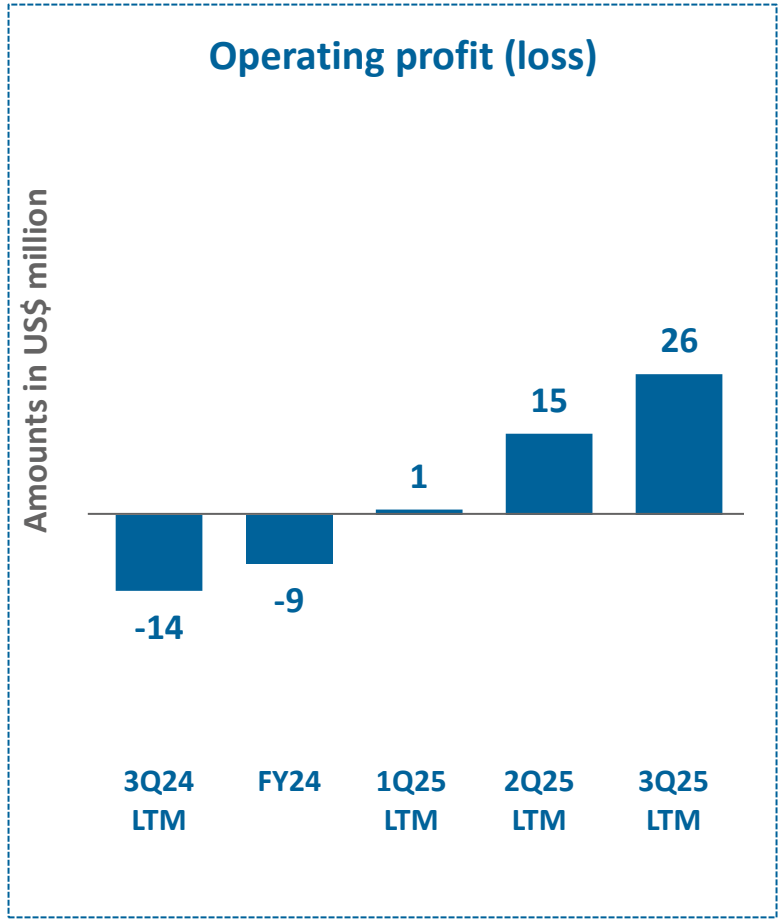
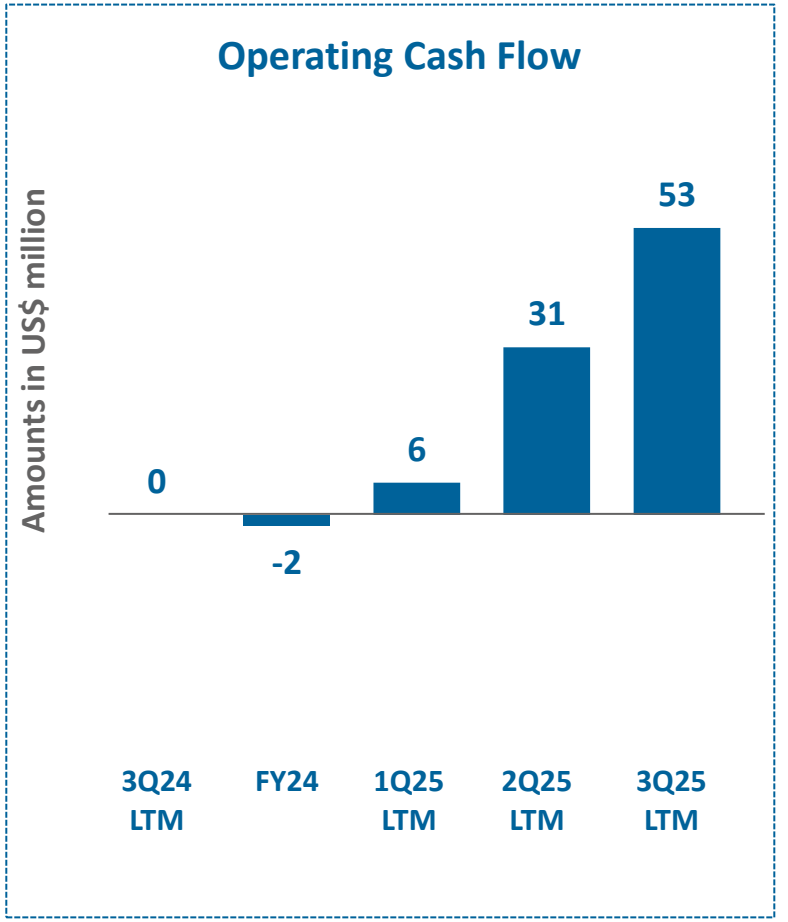
Financials and Outlook

Positive rolling 12-month financial trends







* 2025 revenues are preliminary and unaudited. Final results may differ and will be reported in the financial results for the fourth quarter and full year 2025, to be published in March 2026.

2026 financial guidance and long-term capital outlook

- Revenue and operating expenses (in constant currency):

	FY 2026 Guidance	Notes
Total Revenues	**US$405 - 425 million**	• 8 - 13% growth
Operating Expenses	**US$330 - 335 million**	• US$60 million incremental R&D investments to advance pipeline • US$9 million structural G&A cost reductions (as announced in October 2025)

- Continued RUCONEST® growth, and significant and accelerating Joenja® growth
- Strong financial discipline, and prioritized investments to drive value creation
- Available cash and future cash flows expected to cover current pipeline and pre-launch costs

Building momentum across commercial, financial and pipeline

Strong growth momentum

- 2025 revenue ~$376M:
  - High dbl-digit growth for RUCONEST® and Joenja®
- Significant operating profit and operating cash flow (9M 2025)
- 2026 revenue guidance: $405-425M:
  - Continued RUCONEST® growth, significant and accelerating Joenja® growth

Strategic growth priorities

- Sustained growth of commercial portfolio
- Significant Joenja® APDS growth catalysts:
  - Pediatric label, VUSs, targeted geo expansion, prevalence expansion
- Enhanced capital allocation driving growth

High value pipeline

- Joenja® (leniolisib) for PIDs/CVID with immune dysregulation
  - Phase II readouts (2026)
- Napazimone KL1333 for mtDNA mitochondrial disease
  - Pivotal study readout (2027)

Building a leading rare disease company

- Growth-oriented leadership team
- Proven commercial and development capabilities
- Scalable organization

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Appendix

Joenja: Targeting the root cause of APDS to help restore immune balance

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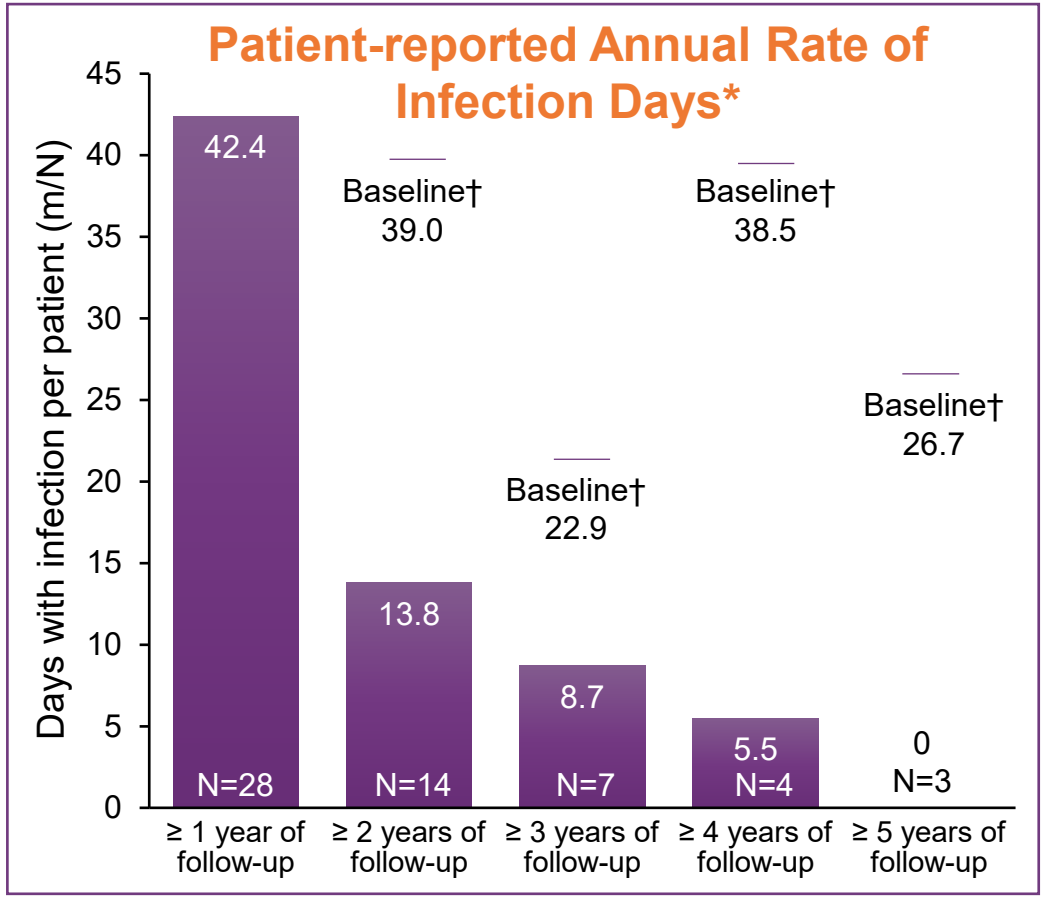
- Treatment with Joenja in a randomized, controlled-trial led to:
  - Significant improvements in immune dysregulation (e.g, lymph node and splenomegaly reductions)
  - Significant improvements in immunophenotype
- Favorable Safety Profile
  - No serious AEs were related to Joenja treatment
  - No patients withdrew from the clinical trials due to an adverse drug reaction
  - The most common adverse reactions (incidence >10%) in the phase 3 trial were headache, sinusitis, and atopic dermatitis
- Long-term open-label study
  - Median duration of Joenja exposure was ~2 years
  - Reduction in infections (see right)



Joenja® clinical trial designs

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Pivotal Trial - Part 1: Dose-finding[1,2]

- Nonrandomized, open-label, dose-escalating
- 6 patients with APDS
- 12 weeks
- 10 mg, 30 mg, 70 mg bid (4 weeks each dose)
- 70 mg bid selected for Part 2

Pivotal Trial - Part 2: Efficacy & Safety Evaluation[3]

- Randomized, triple-blinded, placebo-controlled
- 31 patients with APDS (21 Joenja®, 10 placebo)
- 12 weeks
- 70 mg bid
- Co-primary efficacy end points
  - Change from baseline in log^{10}-transformed SPD of index lesions
    - Also assessed as % change
  - Change from baseline in percentage of naïve B cells out of total B cells
- Secondary and exploratory end points
- Safety

Open-label extension study[4,5]

- Nonrandomized, open-label, long-term study
- 35 patients with APDS from Parts 1 and 2
- 2 patients with APDS previously treated with investigational PI3Kδ inhibitors
- Ongoing
- 70 mg bid
- Long-term safety, tolerability, efficacy, and pharmacokinetics

bid, twice a day; PI3Kδ, phosphoinositide 3-kinase delta; SPD, sum of product diameters

1. Rao VK, et al. *Blood*. 2017;130(21):2307-2316. 2. NCT02435173. ClinicalTrials.gov. https://clinicaltrials.gov/ct2/show/NCT02435173. Updated May 6, 2015. Accessed March 13, 2023. 3. Rao VK, et al. *Blood*. 2023;141(9):971-983.
4. NCT02859727. ClinicalTrials.gov. https://clinicaltrials.gov/ct2/show/NCT02859727. Updated October 31, 2022. Accessed March 3, 2023. 5. Data on file. Pharming Healthcare Inc; 2022.

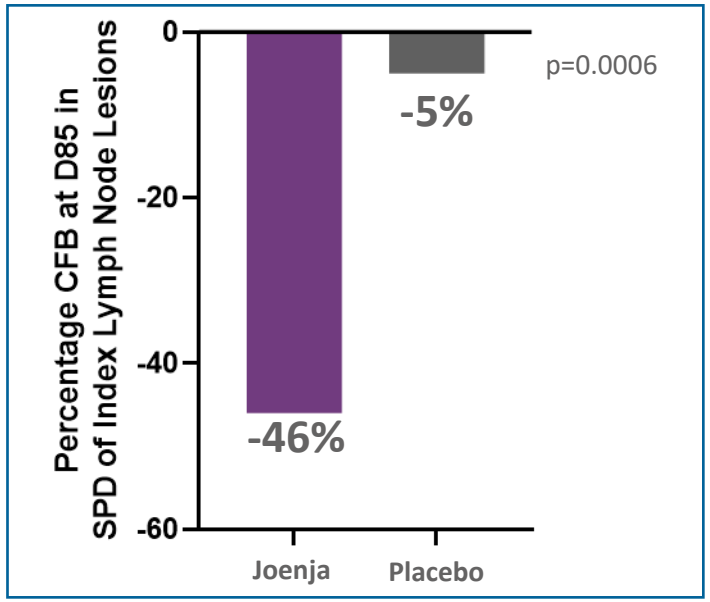
Joenja® addresses the underlying cause of APDS to help restore immune balance – Phase 3 co-primary endpoints

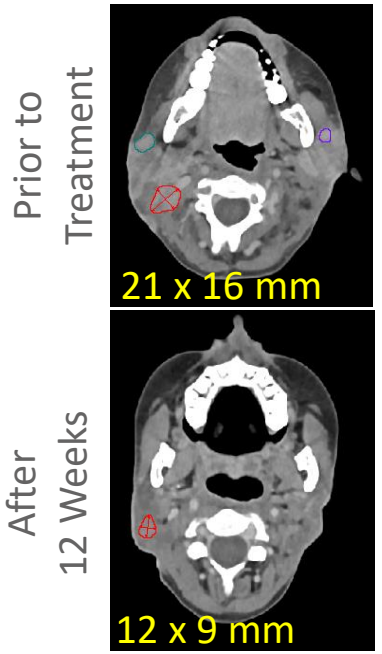
At 12 weeks Joenja® decreased lymphadenopathy and increased naïve B cells

Immune Dysregulation

Change from baseline in index nodes*

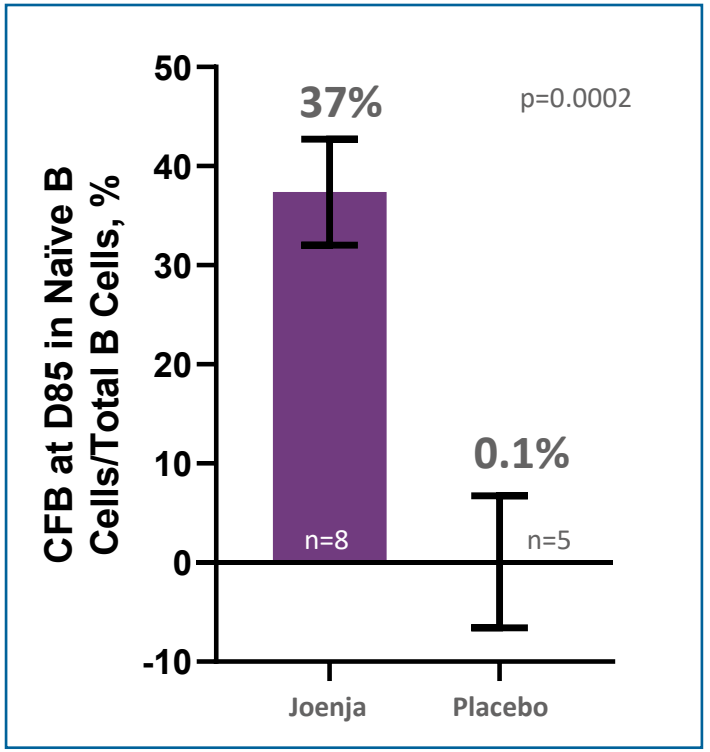
Log_{10}-transformed SPD of index lesions	Joenja (n=18)	Placebo (n=8)
Baseline mean (SD)	3.03 (0.42)	3.05 (0.39)
Change from baseline, LS mean (SE)	-0.27 (0.04)	-0.02 (0.05)
Difference vs placebo (95% CI)		-0.25 (-0.38, -0.12)





Immune Deficiency

Change from baseline in naïve B cells†



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

Joenja® significantly reduced splenomegaly

Secondary endpoint: Significant reductions in spleen size by 2D and 3D analysis compared to placebo

- The adjusted mean difference in bidimensional spleen size between Joenja® (n=19) and placebo (n=9) was -13.5 cm^2 (95% CI: -24.1, -2.91), *P*=0.0148
- The adjusted mean difference in 3D spleen volume between Joenja® (n=19) and placebo (n=9) was -186 cm^3 (95% CI: -297, -76.2), *P*=0.0020

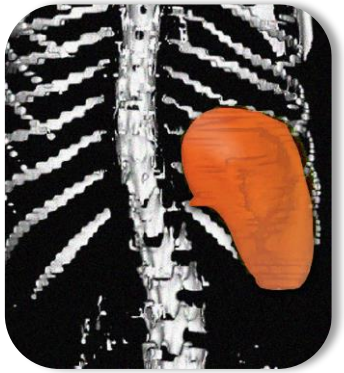
at week 12

27%

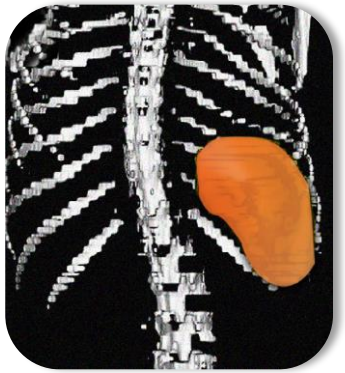
reduction in 3D spleen volume*

Secondary measure: spleen volume scan results of actual patient illustrate average improvement documented for patients taking Joenja®

Prior to treatment:
491 mL



At week 12:
314 mL



Actual patient images of a 17-year-old male. As individual results vary, images may not be representative of all patients.

Rao VK, et al. Blood. 2023;141(9):971-983.

*In the PD analysis set, the mean (SD) percentage change from baseline to week 12 in 3D spleen volume (mm^3) was -26.68% (12.137) with Joenja® (n=19) and -1.37% (24.238) with placebo (n=9). The ANCOVA model was used with treatment as a fixed effect and log_{10}-transformed baseline as a covariate for index and non-index lesions. The use of both glucocorticoids and IV Ig at baseline was included as categorical (yes/no) covariates.
This analysis excluded 2 patients in each treatment group. In the Joenja® group, 1 patient with a complete index lesion response was excluded, and 3 patients were excluded for no non-index lesion at baseline.
PD, pharmacodynamics.

Joenja® safety profile

Phase 3 Trial[1,2]

Adverse reactions reported by ≥2 patients treated with Joenja and more frequently than placebo

	Joenja (n=21) n (%)	Placebo (n=10) n (%)
Headache	5 (24)	2 (20)
Sinusitis	4 (19)	0
Dermatitis atopic*	3 (14)	0
Tachycardia†	2 (10)	0
Diarrhea	2 (10)	0
Fatigue	2 (10)	1 (10)
Pyrexia	2 (10)	0
Back pain	2 (10)	0
Neck pain	2 (10)	0
Alopecia	2 (10)	0

- Study drug-related AEs occurred in 8 patients; the incidence was lower in the Joenja arm (23.8%) than in the placebo arm (30.0%)
- No AEs led to discontinuation of study treatment

Open-label Extension Study[3]

Data cutoff for interim analysis: December 13, 2021

- 32/37 patients reported ≥1 AE
- 78.4% of AEs were grade 1, 48.6% grade 2, 27.0% grade 3, 0% grade 4
- No SAEs related to Joenja

Most common AEs	n
Upper respiratory tract infection	8
Headache	6
Pyrexia	6
Otitis externa	5
Weight increase	5
COVID-19, positive/negative	5/14

One patient with significant baseline cardiovascular comorbidities suffered cardiac arrest resulting in death at extension Day 879; determined by investigator not to be related to study drug

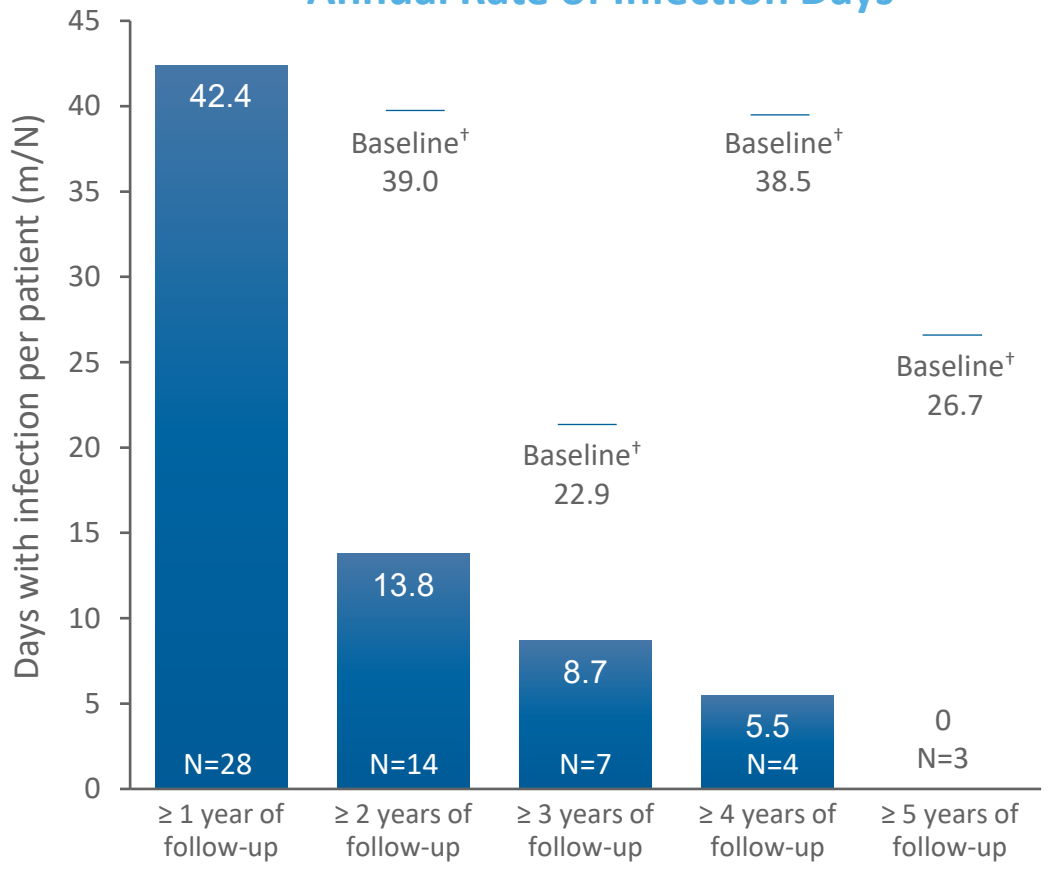
Across all trials[2]

- 38 patients had a **median exposure of ~2 years**
- 4 patients had **>5 years of exposure**

A patient with multiple occurrences of an AE is counted only once in the AE category. Only AEs occurring at or after first drug intake are included.
*Includes dermatitis atopic and eczema. †Includes tachycardia and sinus tachycardia.
AEs, adverse events; **ALT**, alanine aminotransferase; **AST**, aspartate aminotransferase; **SAE**, serious adverse event.
1. Rao VK, et al. Blood. 2023;141(9):971-983. **2.** Joenja [package insert]. Leiden, The Netherlands: Pharming Technologies B.V.; 2023. **3.** Data on file. Pharming Healthcare Inc; 2022.
Please see Important Safety Information and full Prescribing Information available at joenja.com

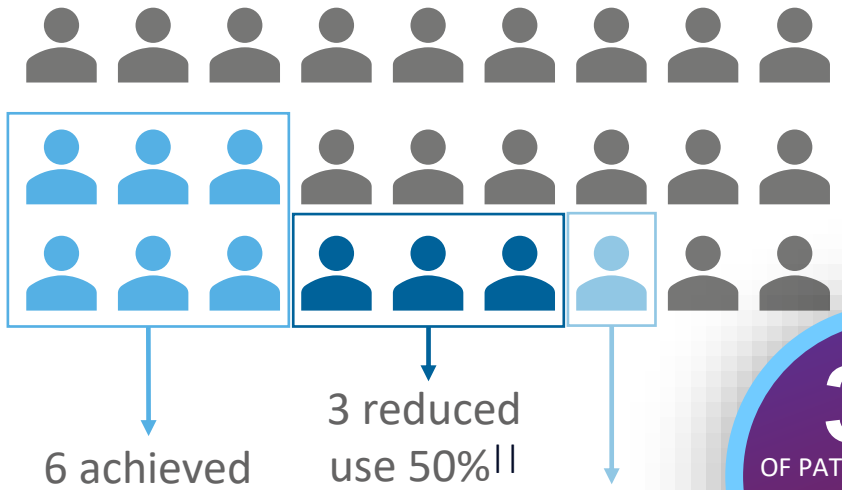
Open-label extension interim analysis of days spent with infections and IRT reduction

Annual Rate of Infection Days*



Physician-reported IRT Reduction‡

Of 27 patients receiving IRT:



6 achieved IRT freedom§

3 reduced use 50%‖

1 reduced use 30%

37% OF PATIENTS RECEIVING IRT REDUCED OR DISCONTINUED DOSE

At the data cutoff, **4** of the patients who were **IRT free** had been so for **1 – 2.5 years**

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