



**Pharming Group N.V.**  
2023 Cantor Global  
Healthcare Conference

**September 26-28, 2023**

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

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**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



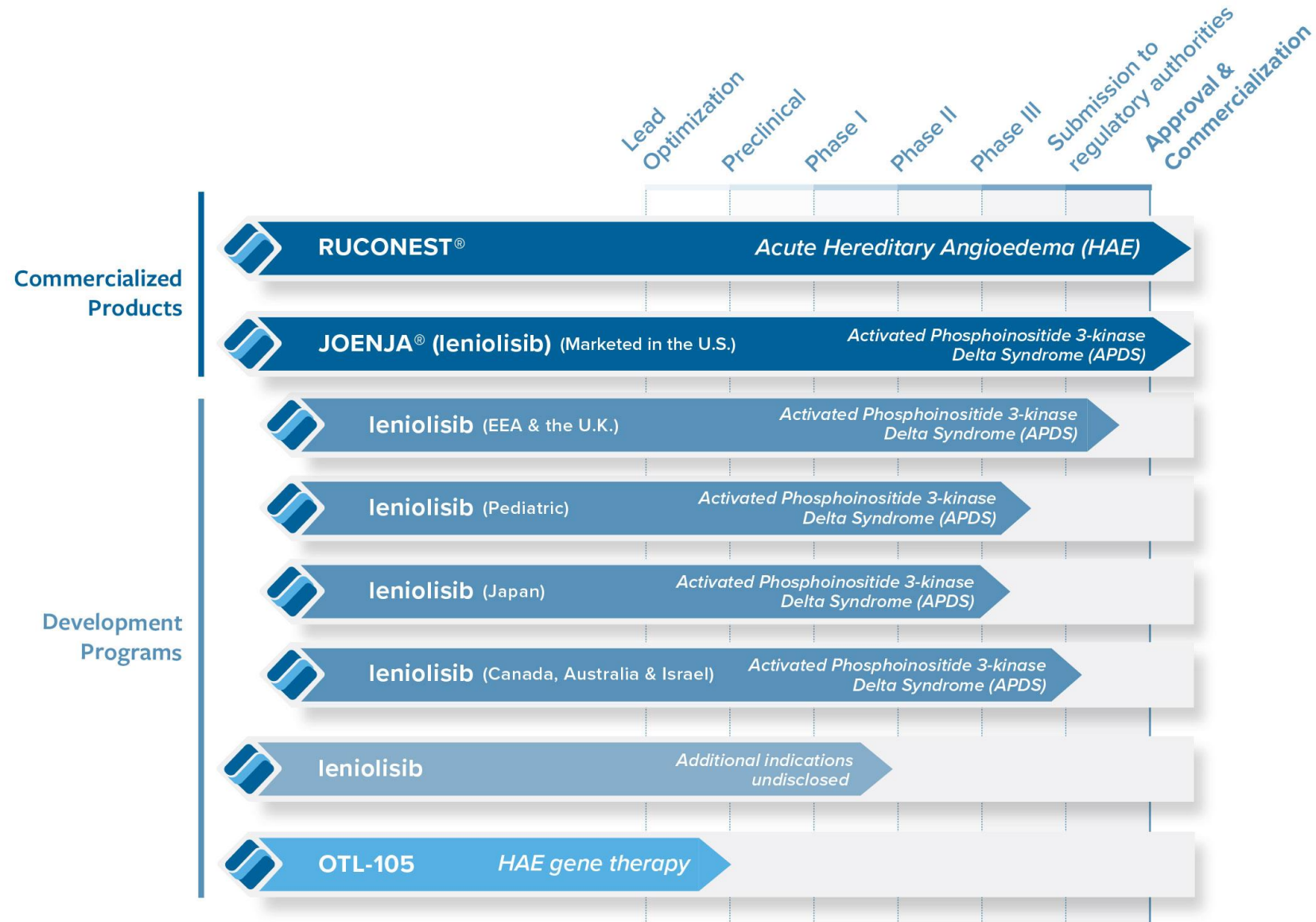
**Ongoing pipeline development and management of rare disease assets**



**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





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



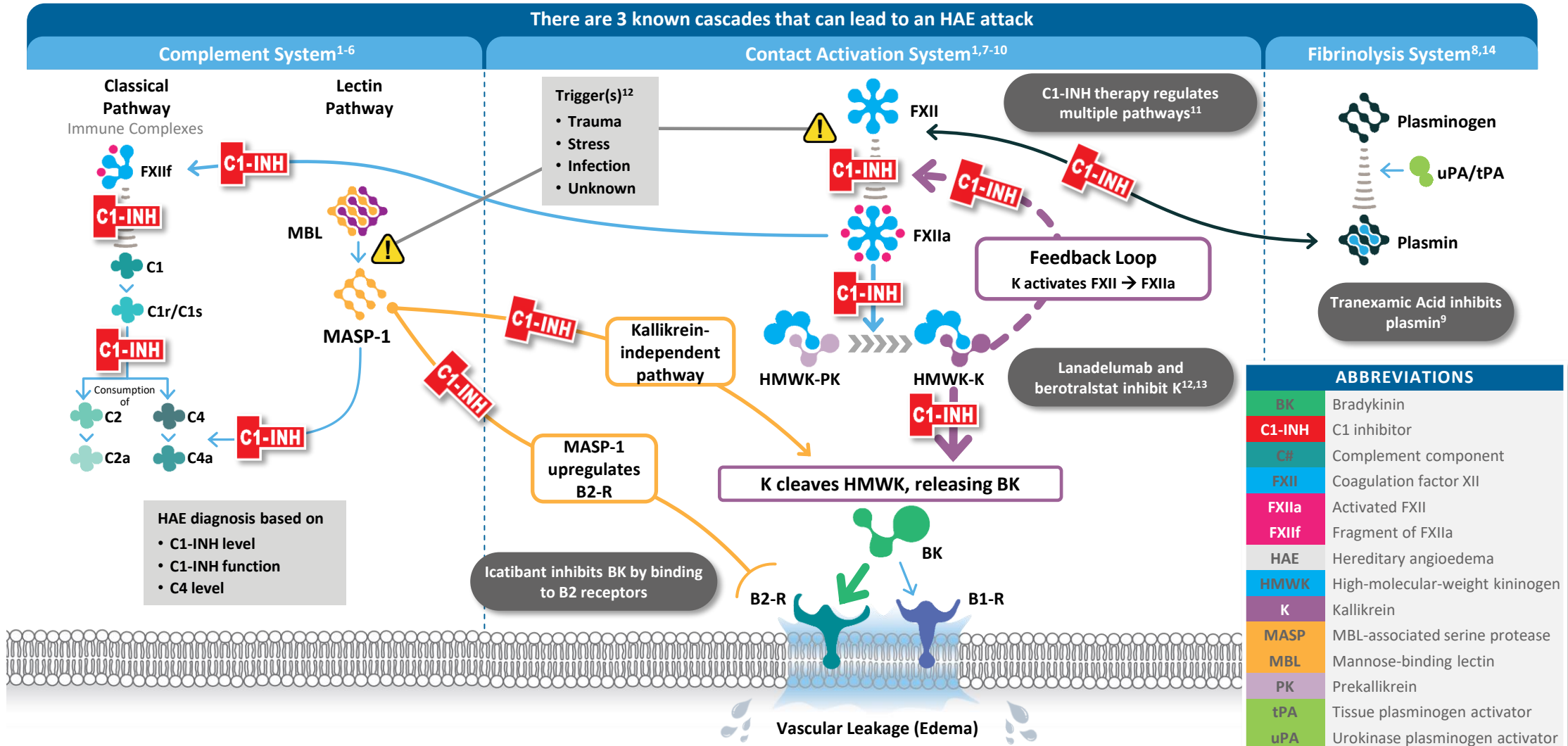
High conference penetration & Support for educational KOL speaker programs



**RUCONEST®**



# C1-INH targets the root cause of HAE



Adapted from a clinical cascade developed in partnership with Dr. Allen Kaplan. This is a current scientific understanding of the cascades. Clinical implications are unknown.



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



Strong 2Q23 performance

Continued outlook of low single  
digit 2023 revenue growth



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%: needed just 1 dose of  
RUCONEST®<sup>1</sup>  
93%: acute attacks stopped with  
RUCONEST® for at least 3 days<sup>2</sup>



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



2Q23: Performed well in leading  
revenue indicators in the U.S.:  
active patients, vials shipped, #  
physicians prescribing



# Strong commitment to HAE community



Strong patient organization support since 2000

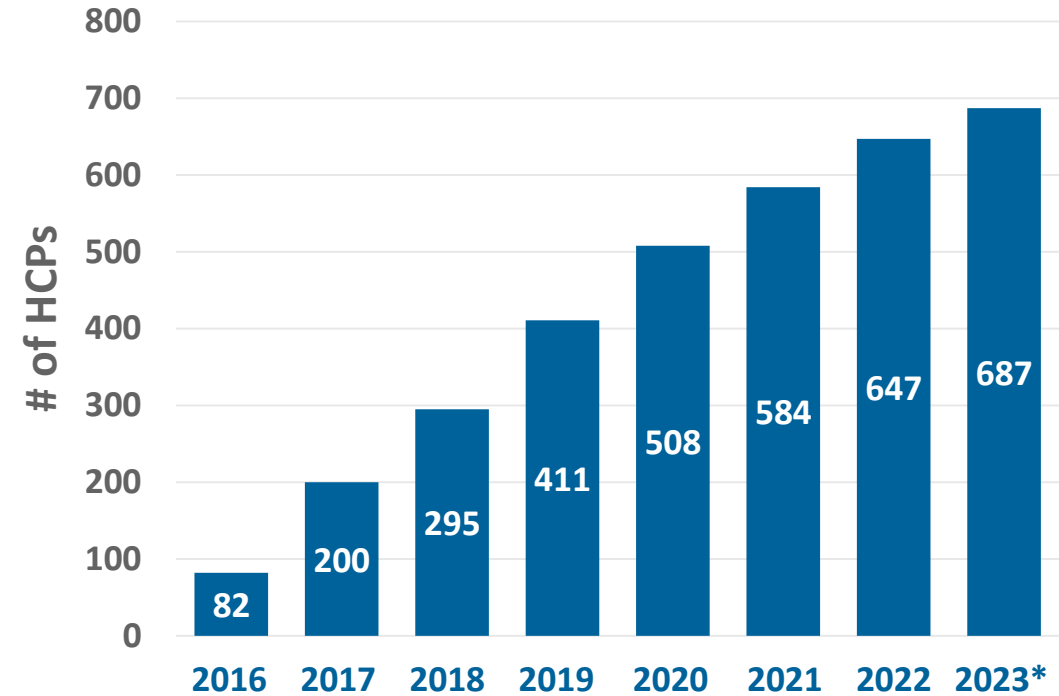


Almost 700 US physicians (and growing) prescribing RUCONEST®



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

# of unique U.S. physicians prescribing



\*Data thru June 30, 2023





# APDS Overview

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



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

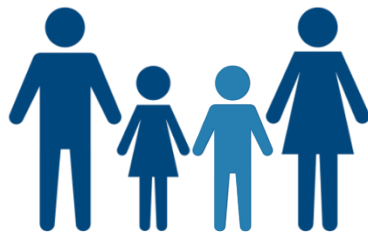
To date, Pharming has identified >640 of these patients in key global markets

(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



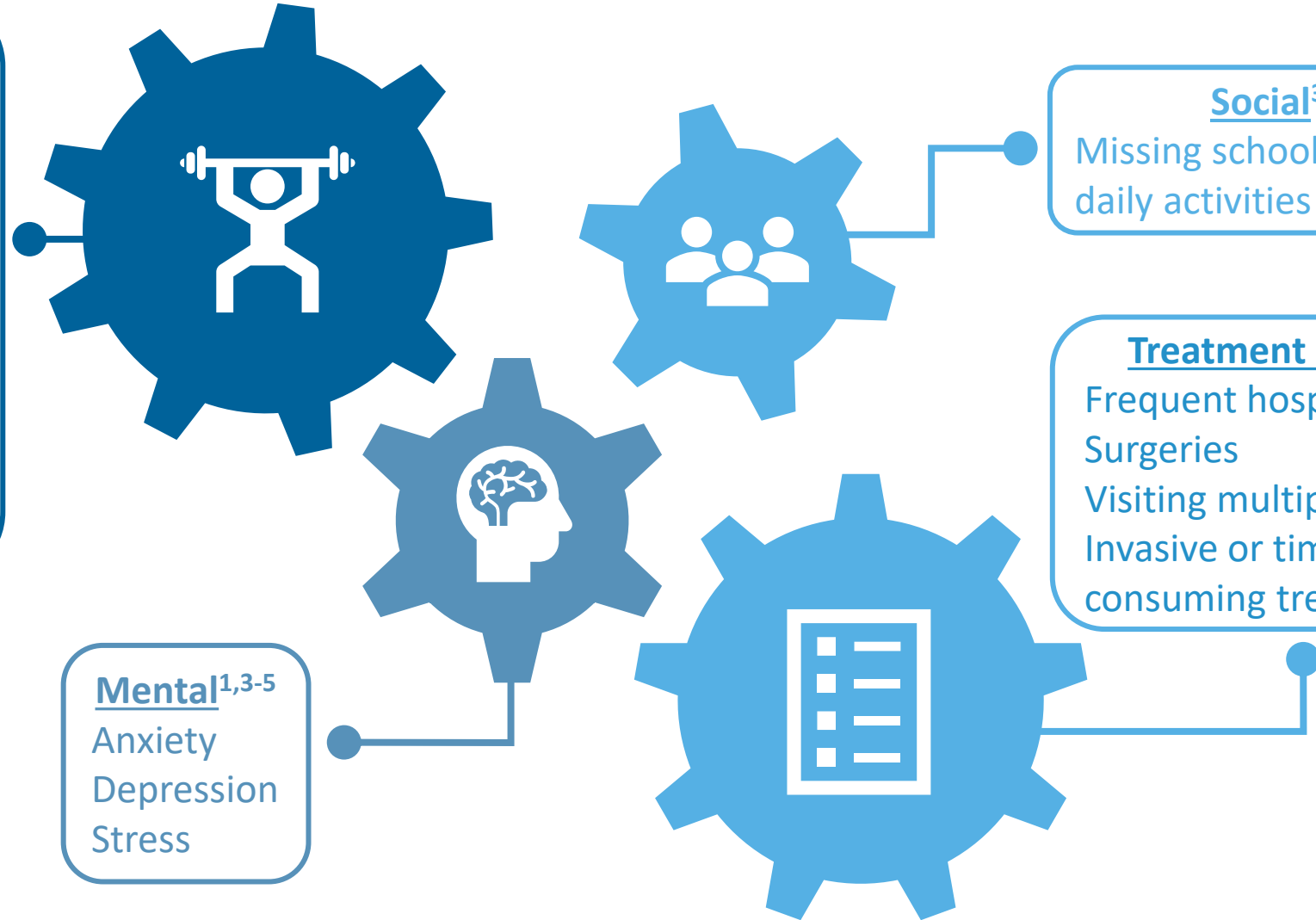
A genetic test can provide a definitive diagnosis of APDS

\*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

# APDS can impact many facets of life

## Physical<sup>1,2</sup>

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



## Social<sup>3,4</sup>

Missing school, work, or daily activities

## Treatment Burden<sup>1-4</sup>

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

## Mental<sup>1,3-5</sup>

Anxiety  
Depression  
Stress

APDS, activated phosphoinositide 3-kinase  $\delta$  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.

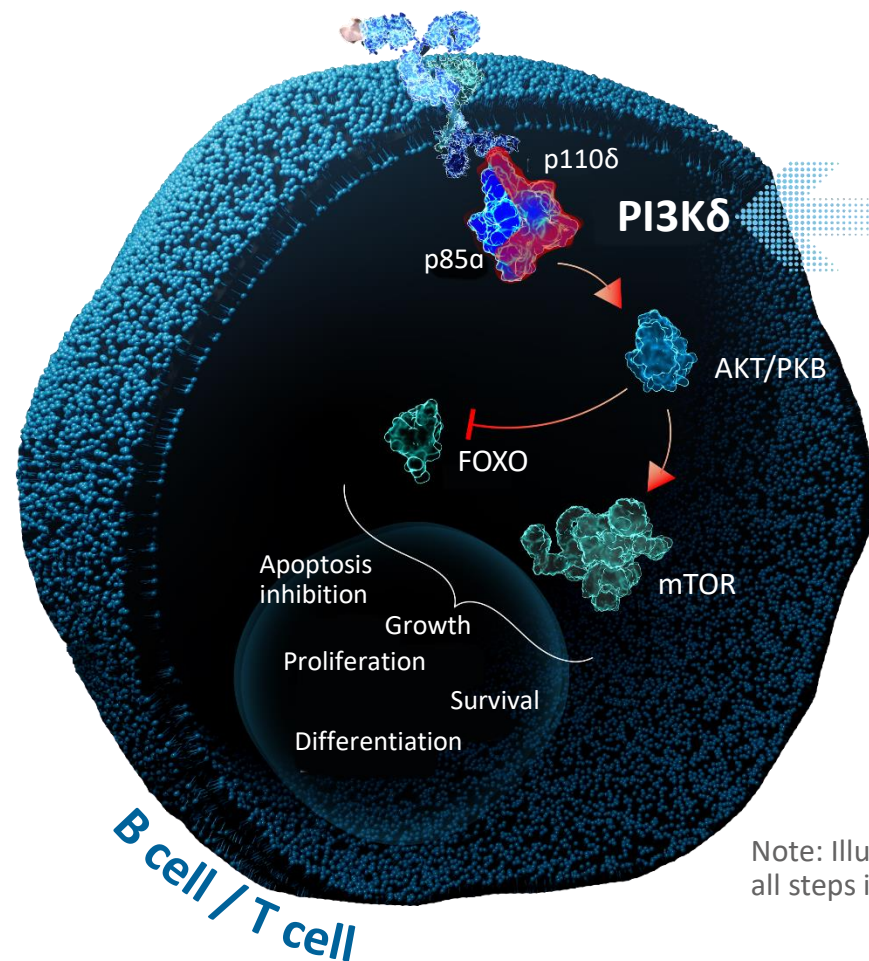
4. Jiang F, et al. *Allergy Asthma Clin Immunol.* 2015;11:27. 5. Kuburovic NB, et al. *Patient Prefer Adherence.* 2014;8:323-330.

# Genetic defect leads to PI3K $\delta$ hyperactivity, disrupting immune cell balance

Hyperactive PI3K $\delta$  results in dysregulated B and T cell development<sup>1-3</sup>

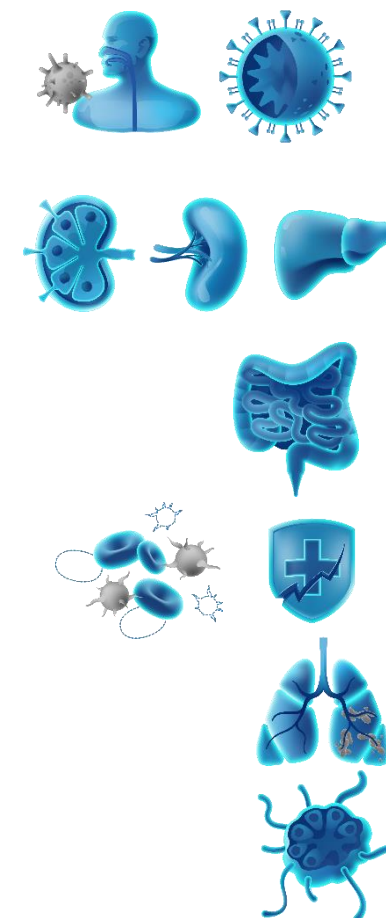


Immune imbalance leads to diverse signs and symptoms<sup>1,4-6</sup>



The PI3K $\delta$  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

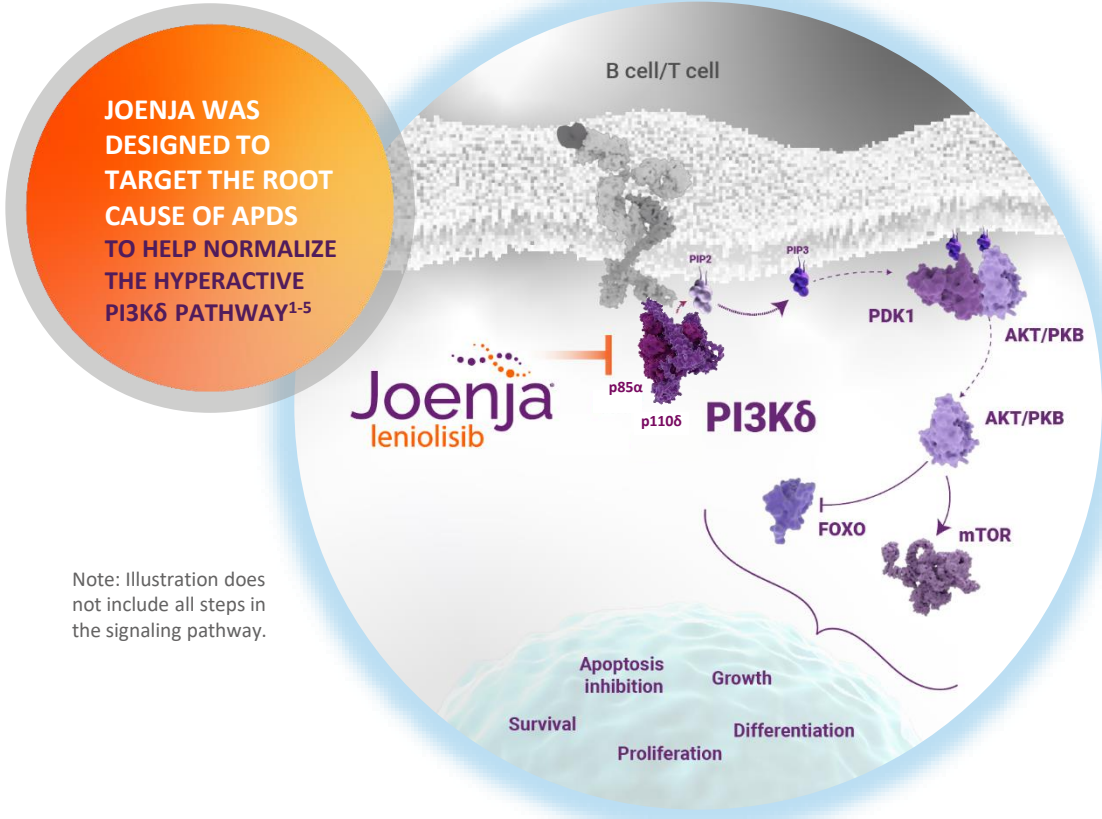
## Bronchiectasis

## Lymphoma

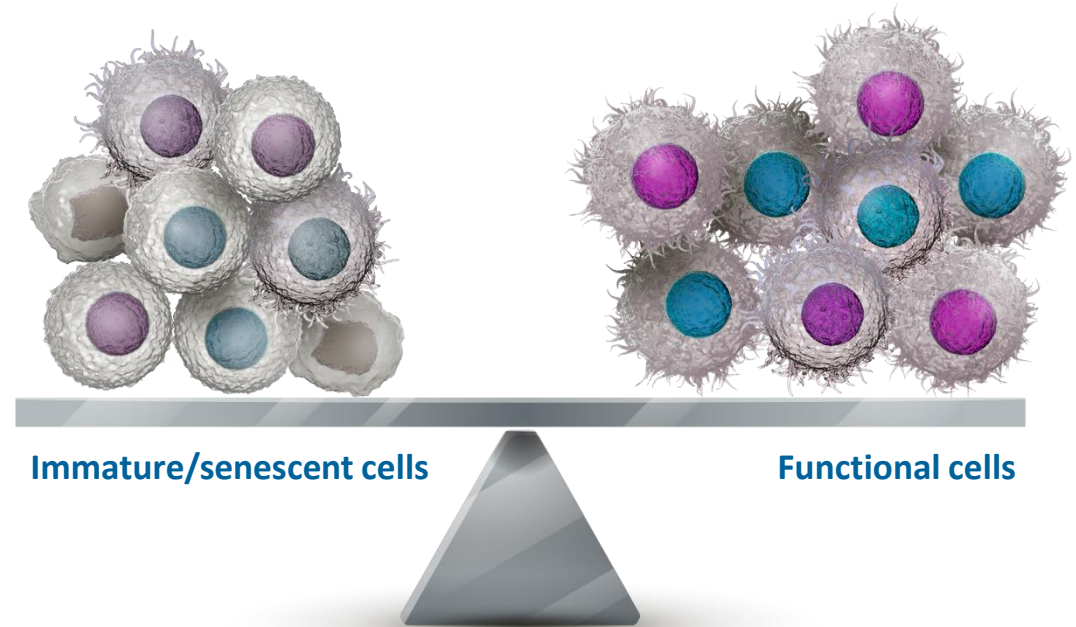
FOXO, forkhead box O; mTOR, mammalian target of rapamycin; PI3K $\delta$ , phosphoinositide 3-kinase delta; PKB, protein kinase B.

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.





Joenja® 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.





**Joenja<sup>®</sup> (leniolisib)**

# U.S. launch 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

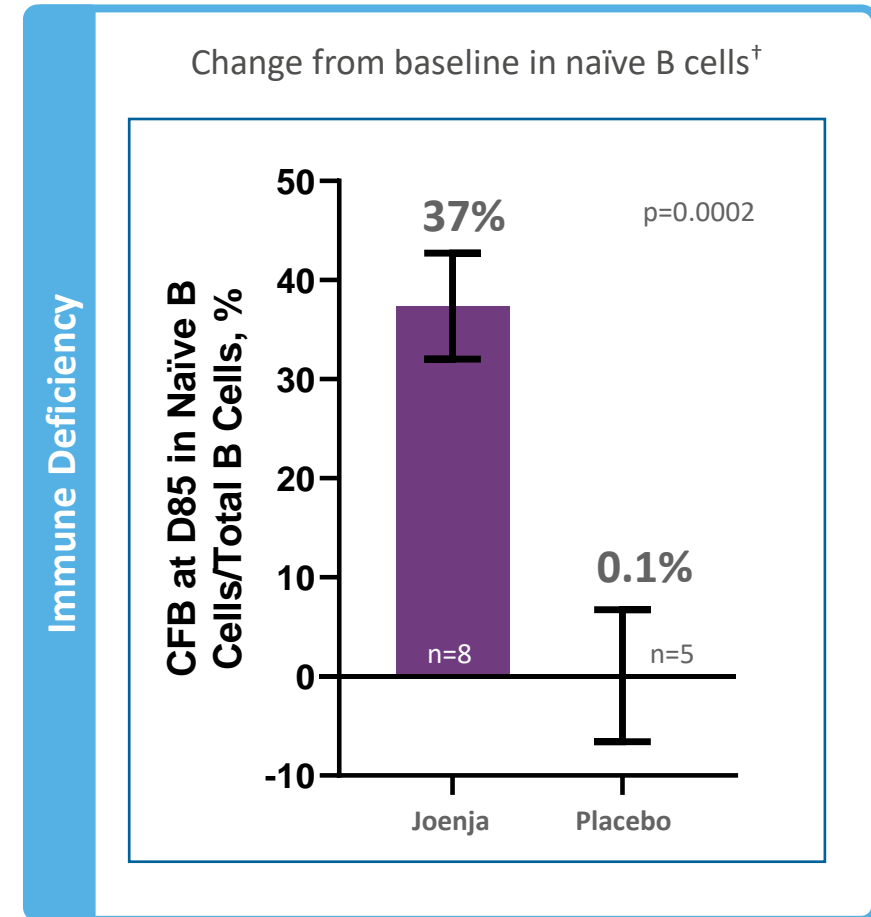
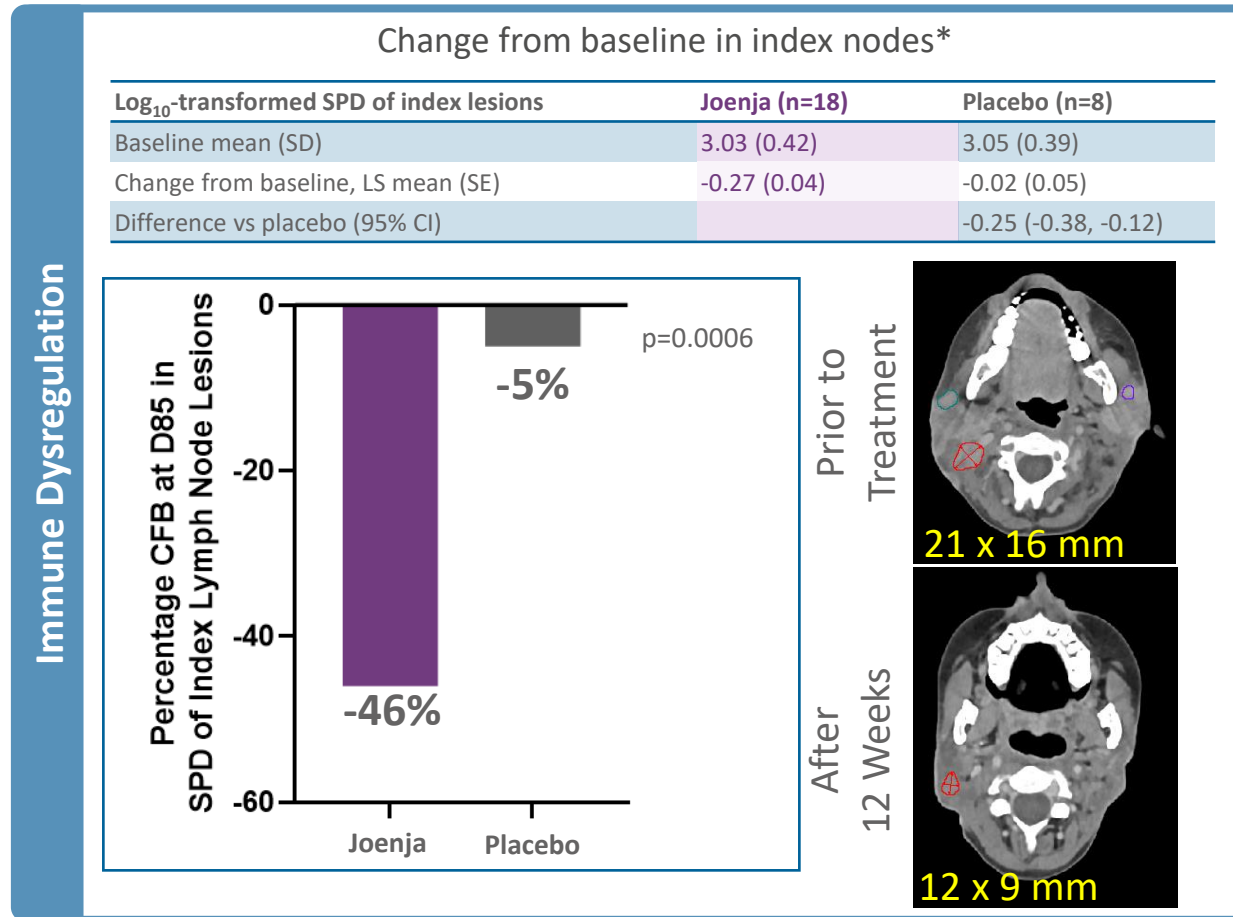
Extension study interim analysis demonstrated safety consistent with the randomized, controlled trial. We continue to collect observational long-term data on lymphadenopathy, naive B cells and IgM

Strong start to Joenja<sup>®</sup> launch with 60 enrollments & 43 patients on paid therapy as of June 30, 2023



# 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



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.

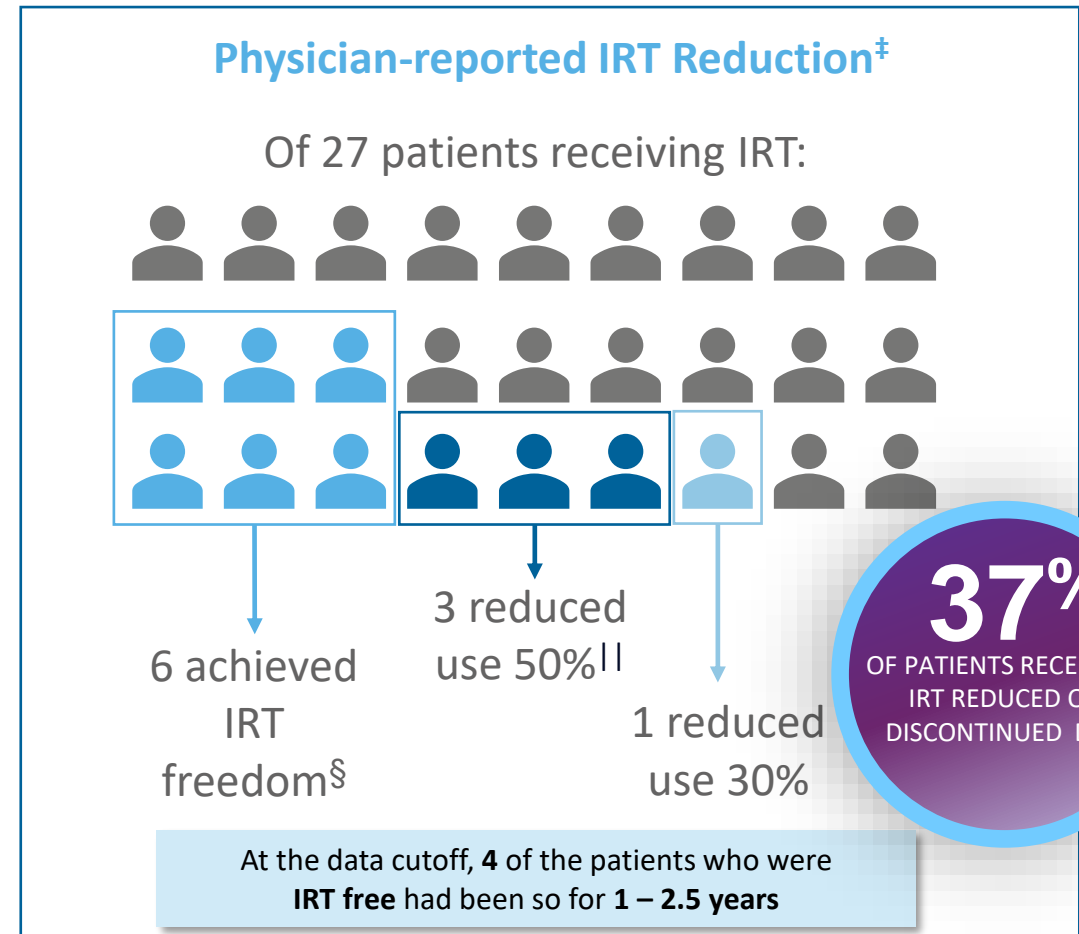
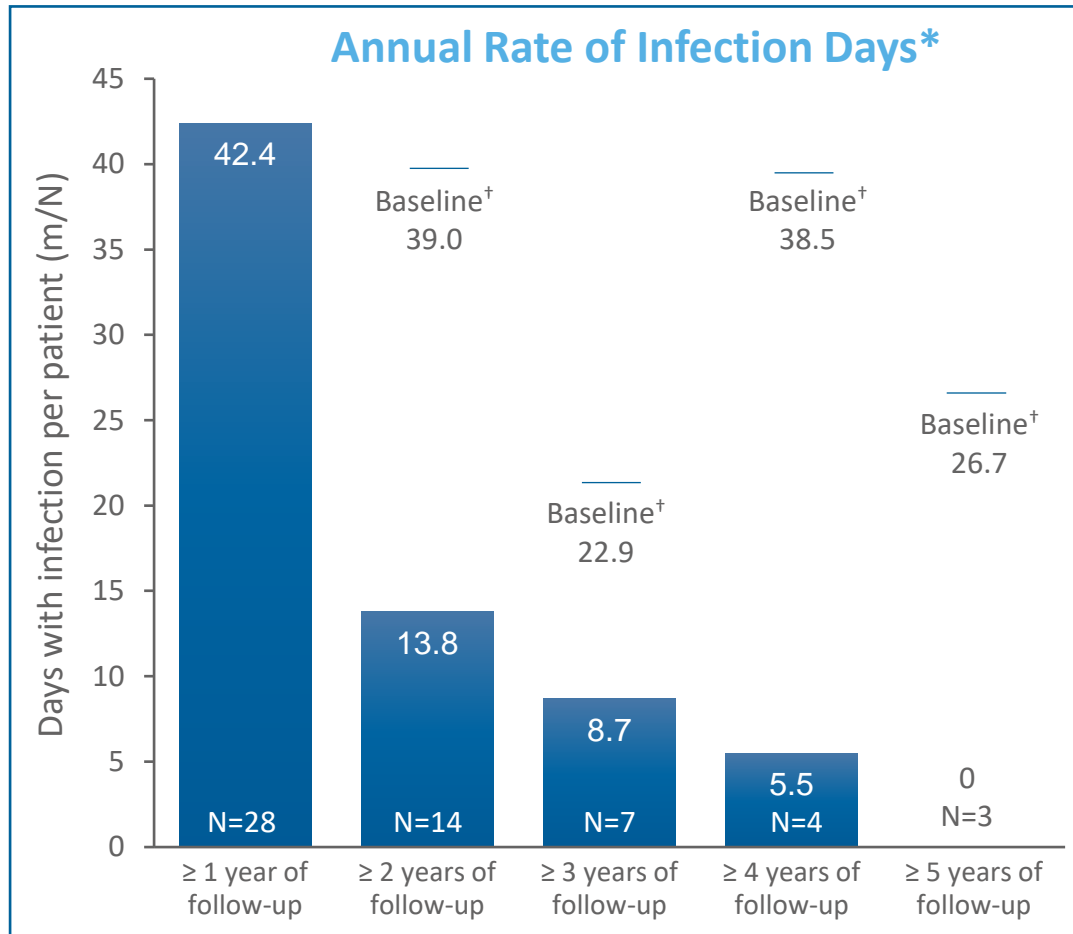
\*The analysis excluded 2 patients from each treatment group due to protocol deviations and 1 Joenja patient having complete resolution of the index lesion identified at baseline.

†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](http://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.  
 Rao VK, et al. Poster presented at: 64<sup>th</sup> 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](http://joenja.com)



## 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

- ◆ 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 (based on Annual Cost (WAC) – US\$547,500)
- ◆ Productive ongoing engagement with both national and regional payers
- ◆ The sales team continue to drive new patient enrollments





## 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 – first patient enrolled in August 2023



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 2H23



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

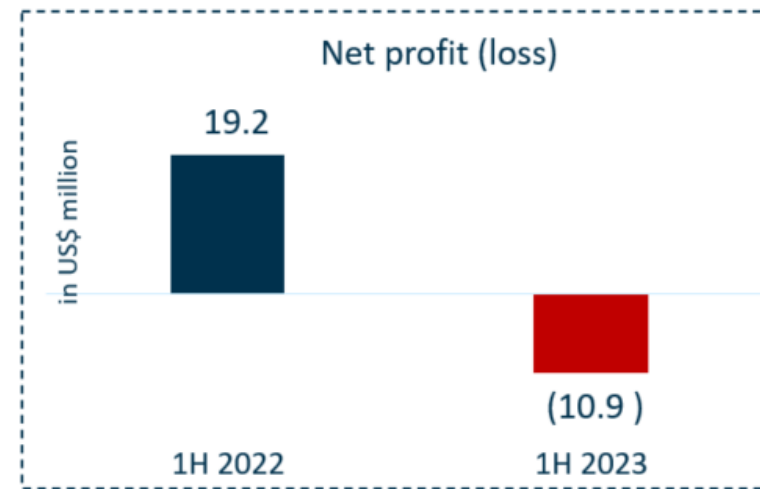
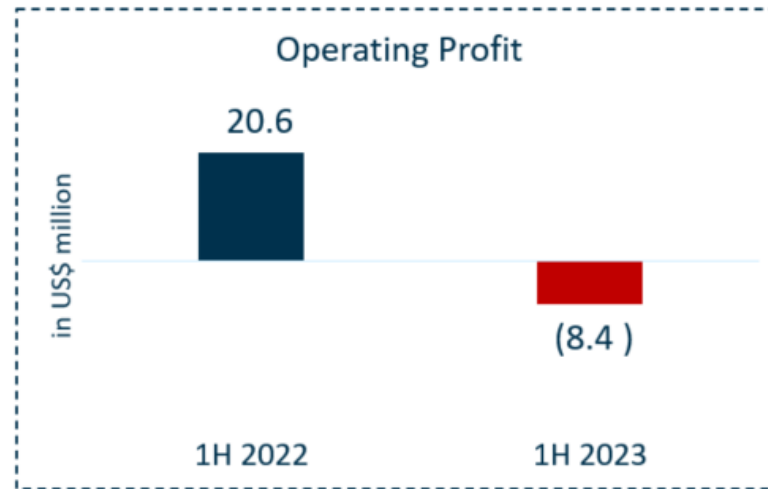
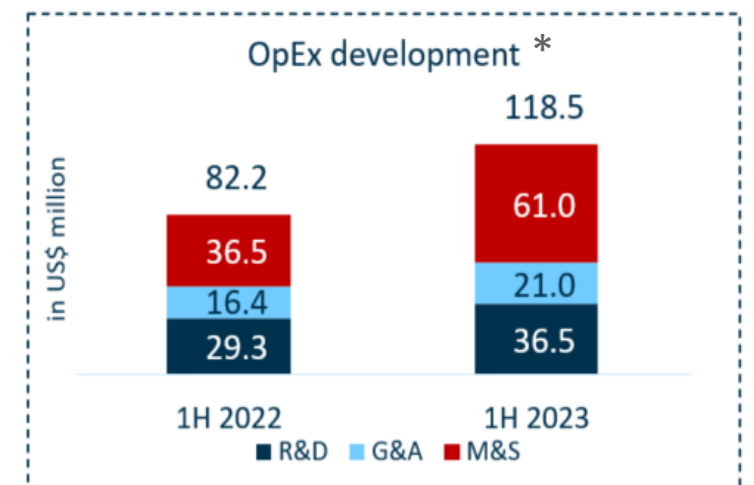
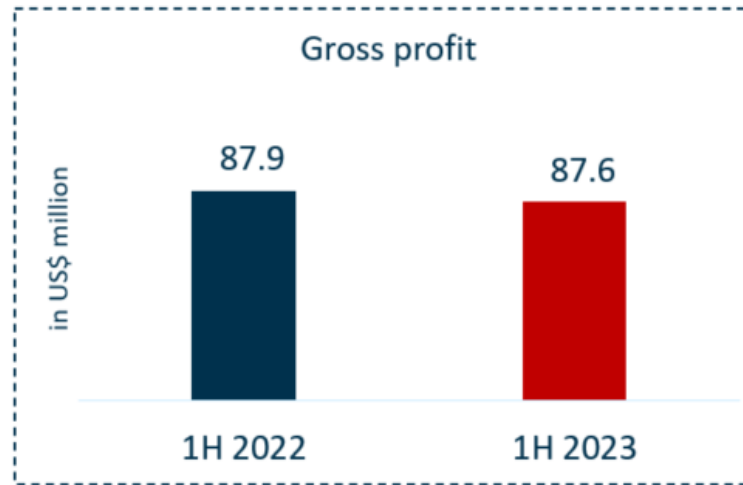
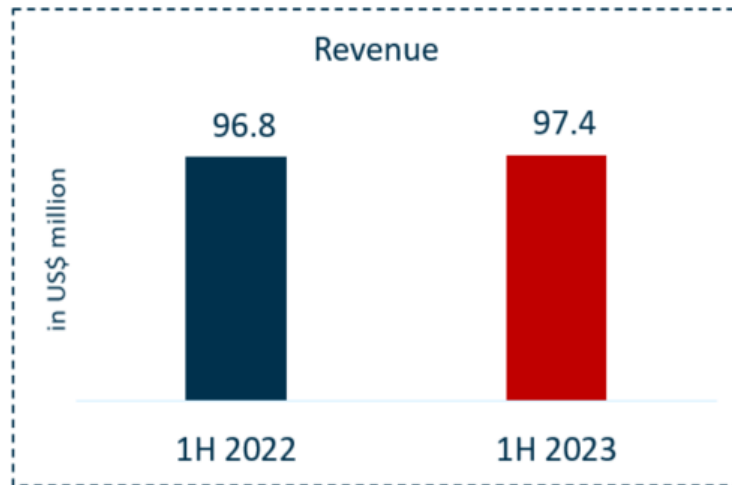
\*Received CHMP Day 180 list of outstanding issues in July. CHMP will consult an Ad-hoc Expert Group (AEG) given the rarity of the disease and the unmet medical need for the treatment of APDS patients. Approval is subject to positive outcomes of the EMA CHMP review.

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



# Financials and Outlook

# Financial highlights: 1H 2023 vs 1H 2022



- Cash and cash equivalents, including restricted cash, increased from \$186.2M in 1Q23 to \$194.1M in 2Q23

\*1H2023 marketing and sales expenses includes US\$10.5 million milestone payments

# Financial highlights: 2Q 2023 vs 2Q 2022



Cash and cash equivalents, including restricted cash, increased from \$186.2M in 1Q23 to \$194.1M in 2Q23

\*includes US\$10 million paid milestone payment



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





[www.pharming.com](http://www.pharming.com)

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**Pharming Group N.V.**

# Appendix

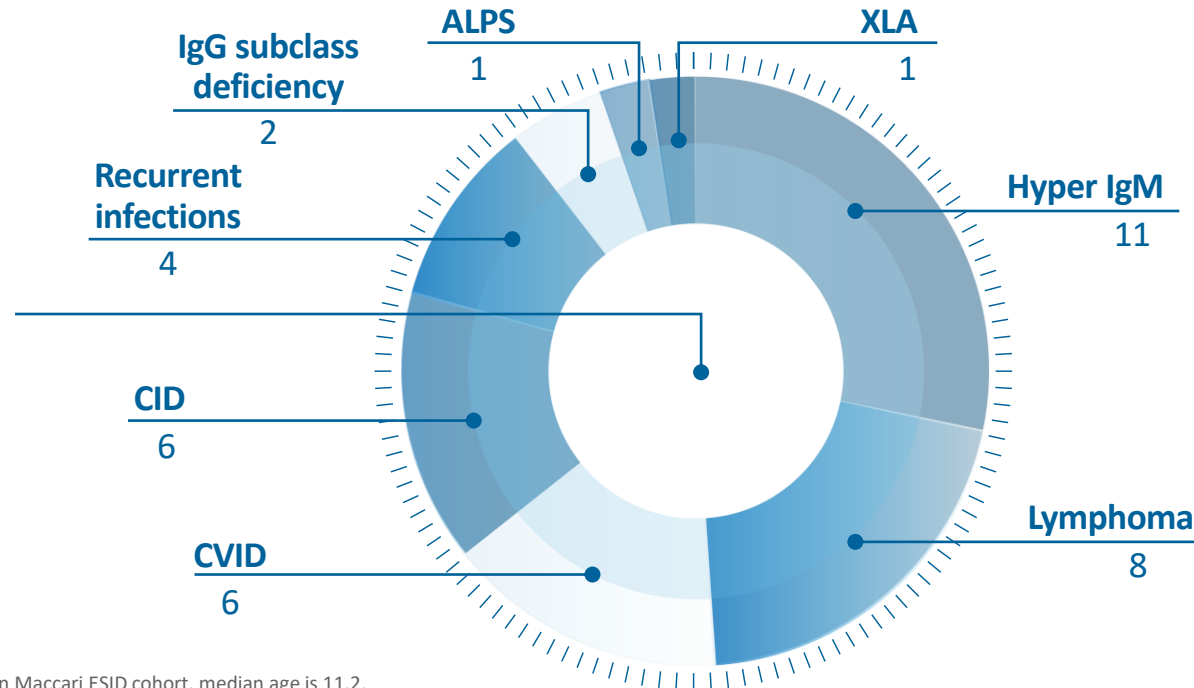
# Heterogeneous, evolving symptomology can often lead to missed diagnoses

## Timeline of the most common pathologies\* seen in APDS<sup>1-4</sup>

Median age at diagnosis:  
12 years (7-year median diagnosis delay)



APDS has often been diagnosed as another PI or condition, causing delays in diagnosis<sup>1</sup>



Improved identification of symptoms, increased genetic testing, and earlier diagnosis are needed

\*Pathologies can occur at any time.

<sup>†</sup>In Elkaim APDS2 cohort, median age of bronchiectasis is 13; in Maccari ESID cohort, median age is 11.2.

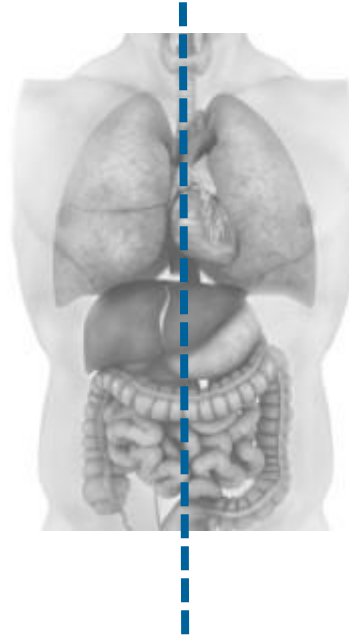
<sup>‡</sup>No median ages are available for these manifestations.

ALPS, autoimmune lymphoproliferative syndrome; CID, combined immunodeficiency; CVID, common variable immune deficiency; ESID, European Society for Immunodeficiencies; HIGM, hyper immunoglobulin M syndrome; IgG, immunoglobulin G; PI3Kδ, phosphoinositide 3-kinase delta; XLA, X-linked agammaglobulinemia.

1. Jamee M, et al. *Clin Rev Allergy Immunol.* 2020;59(3):323-333. 2. Maccari ME, et al. *Front Immunol.* 2018;9:543. 3. Elkaim E, et al. *J Allergy Clin Immunol.* 2016;138(1):210-218.e9. 4. Coulter TI, et al. *J Allergy Clin Immunol.* 2017;139(2):597-606.

## 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  $\delta$  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.

## Pivotal Trial - Part 1: Dose-finding<sup>1,2</sup>



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<sup>3</sup>



Randomized, triple-blinded, placebo-controlled



31 patients with APDS (21 Joenja<sup>®</sup>, 10 placebo)



12 weeks



70 mg bid



Co-primary efficacy end points

- Change from baseline in log<sup>10</sup>-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<sup>4,5</sup>



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.

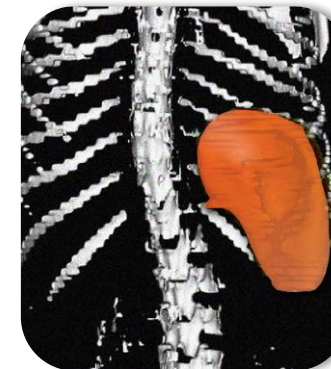
## Secondary endpoint: Significant reductions in spleen size by 2D and 3D analysis compared to placebo

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

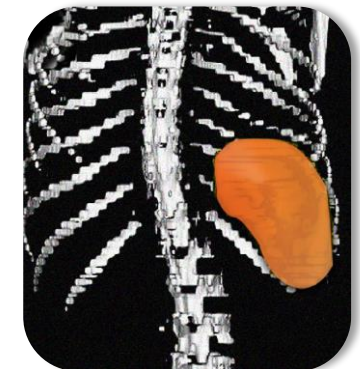
at week 12  
**27%**  
reduction in 3D spleen volume\*

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

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.

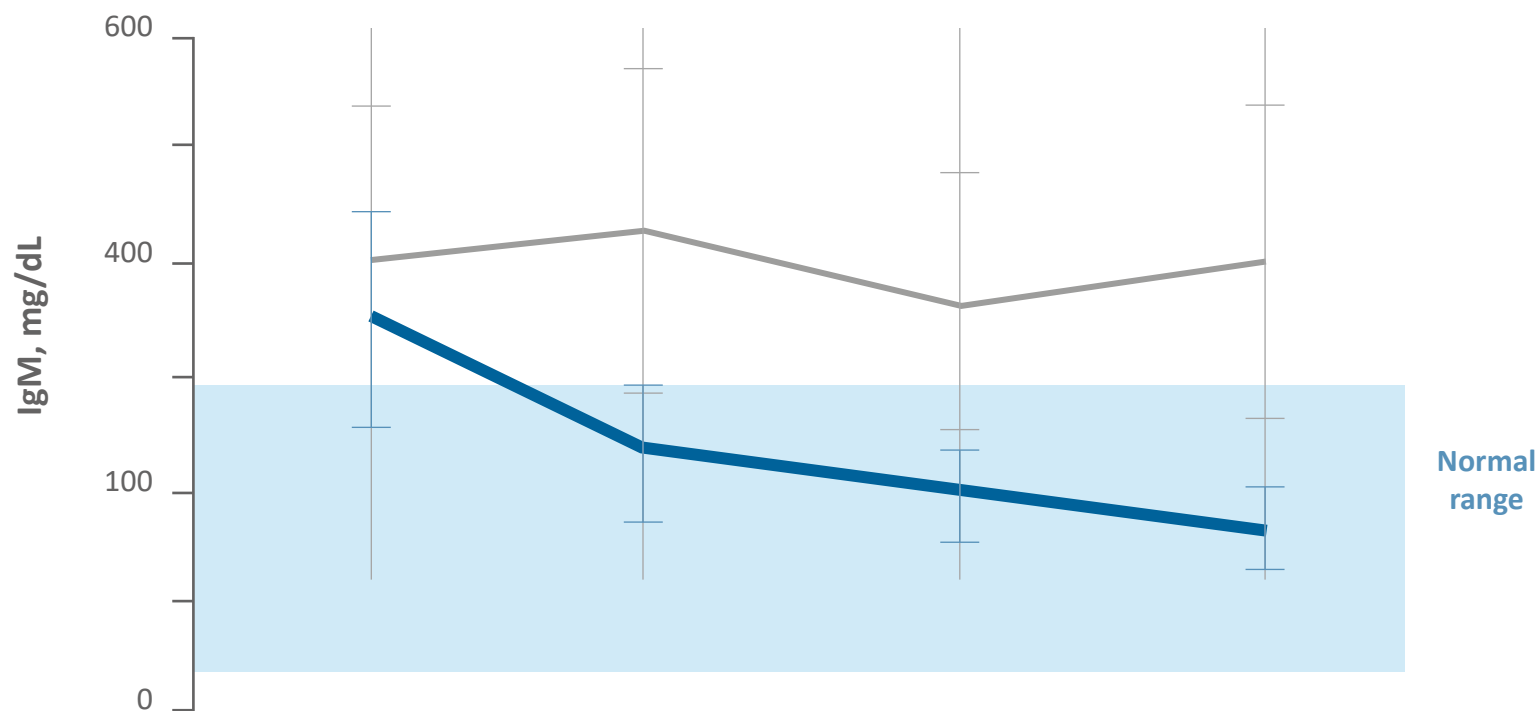
\*In the PD analysis set, the mean (SD) percentage change from baseline to week 12 in 3D spleen volume ( $\text{mm}^3$ ) was  $-26.68\%$  (12.137) with Joenja<sup>®</sup> (n=19) and  $-1.37\%$  (24.238) with placebo (n=9). The ANCOVA model was used with treatment as a fixed effect and  $\log_{10}$ -transformed 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<sup>®</sup> 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.



# An exploratory end point showed Joenja<sup>®</sup> reduced IgM levels

Mean serum IgM rapidly reduced to within normal limits



- In the Joenja<sup>®</sup> arm, IgM was elevated above normal limits in 6 patients at baseline, and by week 12 was reduced in all, with 50% returning to within normal limits
- In contrast, IgM was elevated above normal limits at baseline in 4 patients in the placebo arm, and by week 12 levels remained stable or elevated, with 0% returning to within normal limits

		Baseline	Week 4	Week 8	Week 12
Joenja <sup>®</sup>	n	21	20	21	21
Placebo	n	10	10	10	10

Error bars are standard error of the mean. Safety analysis set (N=31) shown. Blue box indicates IgM normal range.

Soluble biomarkers, including IgM, were prespecified exploratory endpoints in the protocol. Although an observational decrease in IgM was noted in some patients, no statistical significance can be made from this analysis, and no conclusions should be drawn.

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

## Phase 3 Trial<sup>1,2</sup>

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 <sup>†</sup>	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

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. <sup>†</sup>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 Study<sup>3</sup>

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