



Pharming Group N.V.
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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



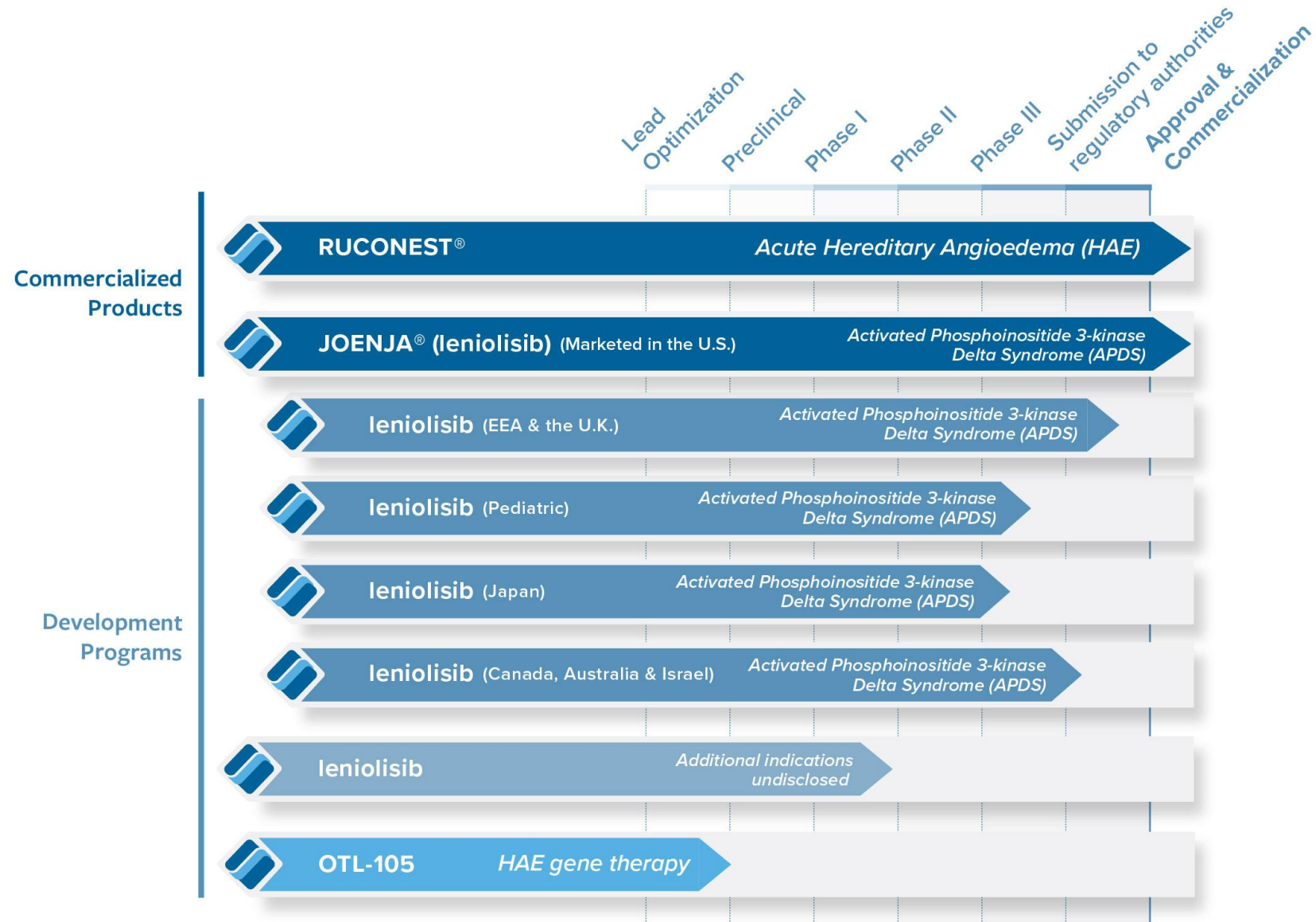
Ongoing pipeline development and management of rare disease assets



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

- ◆ Advanced 2nd 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

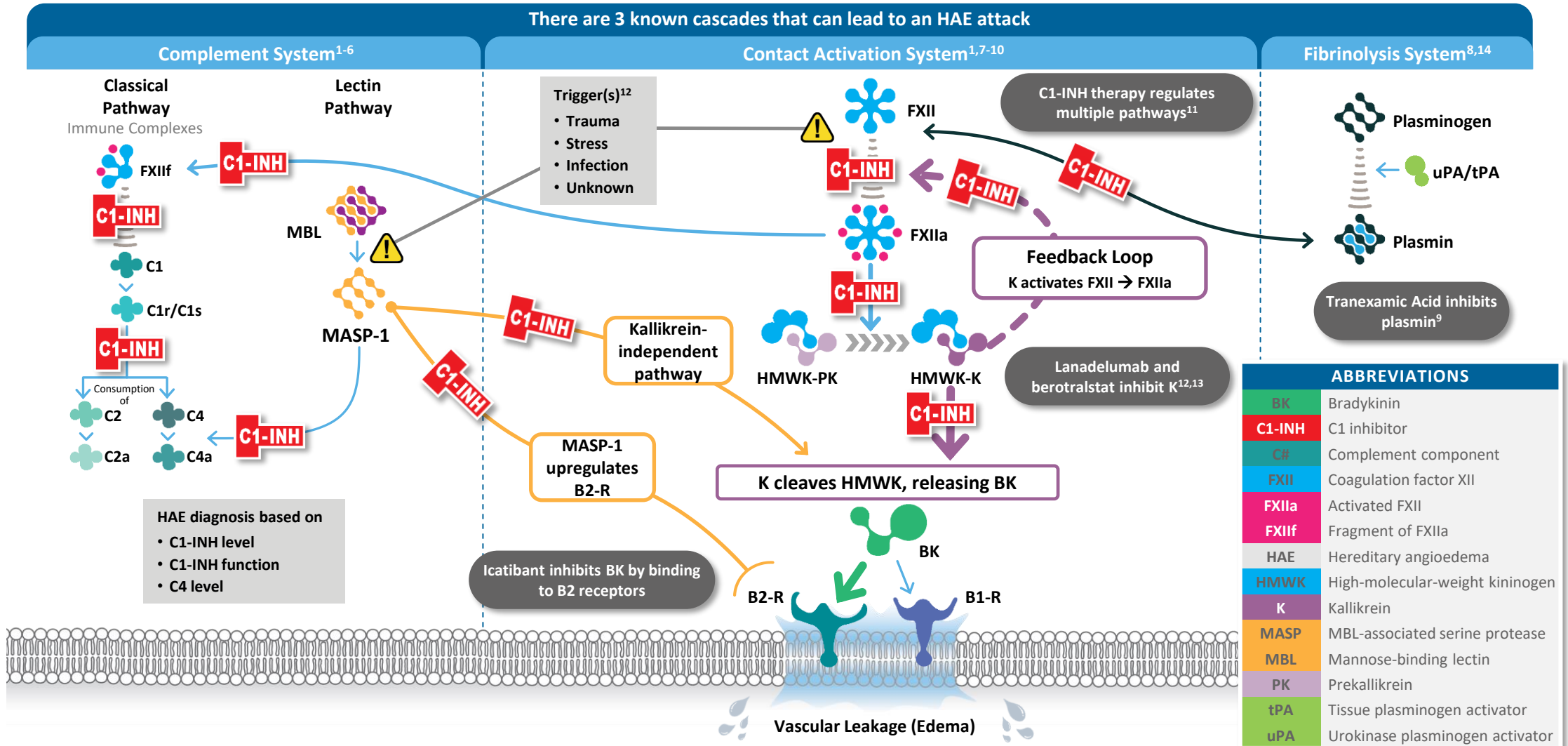


High conference penetration & Support for educational KOL speaker programs

 Pharming® | **35** years

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)**



**2Q23: RUCONEST® returned to
growth
Outlook of low 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%: needed just 1 dose of
RUCONEST®¹
93%: acute attacks stopped with
RUCONEST® for at least 3 days²**



**Performed well in leading
revenue indicators in the U.S.:
active patients, vials shipped, &
physicians prescribing**



**Patients are well managed and feel
confident to administer treatment
themselves³**

Strong commitment to HAE community



Strong patient organization support since 2000

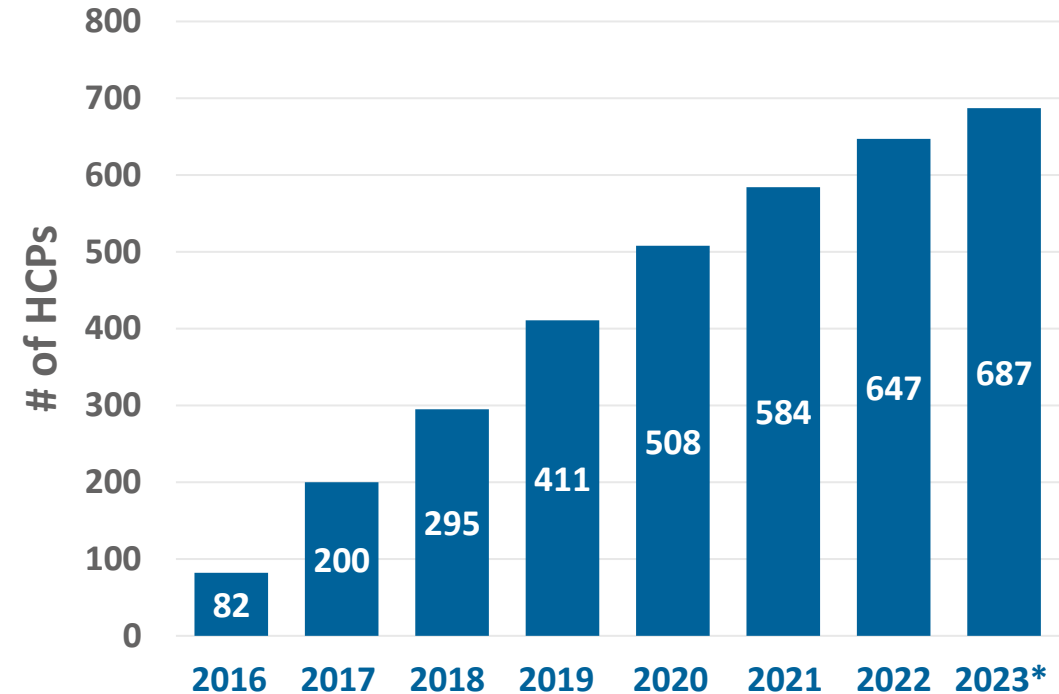


Almost 700 US physicians (and growing) prescribing RUCONEST®



>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 δ) syndrome (APDS) affects >1500 patients*

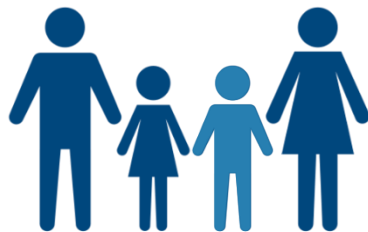
To date, Pharming has identified >640 of these patients in key global markets

(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



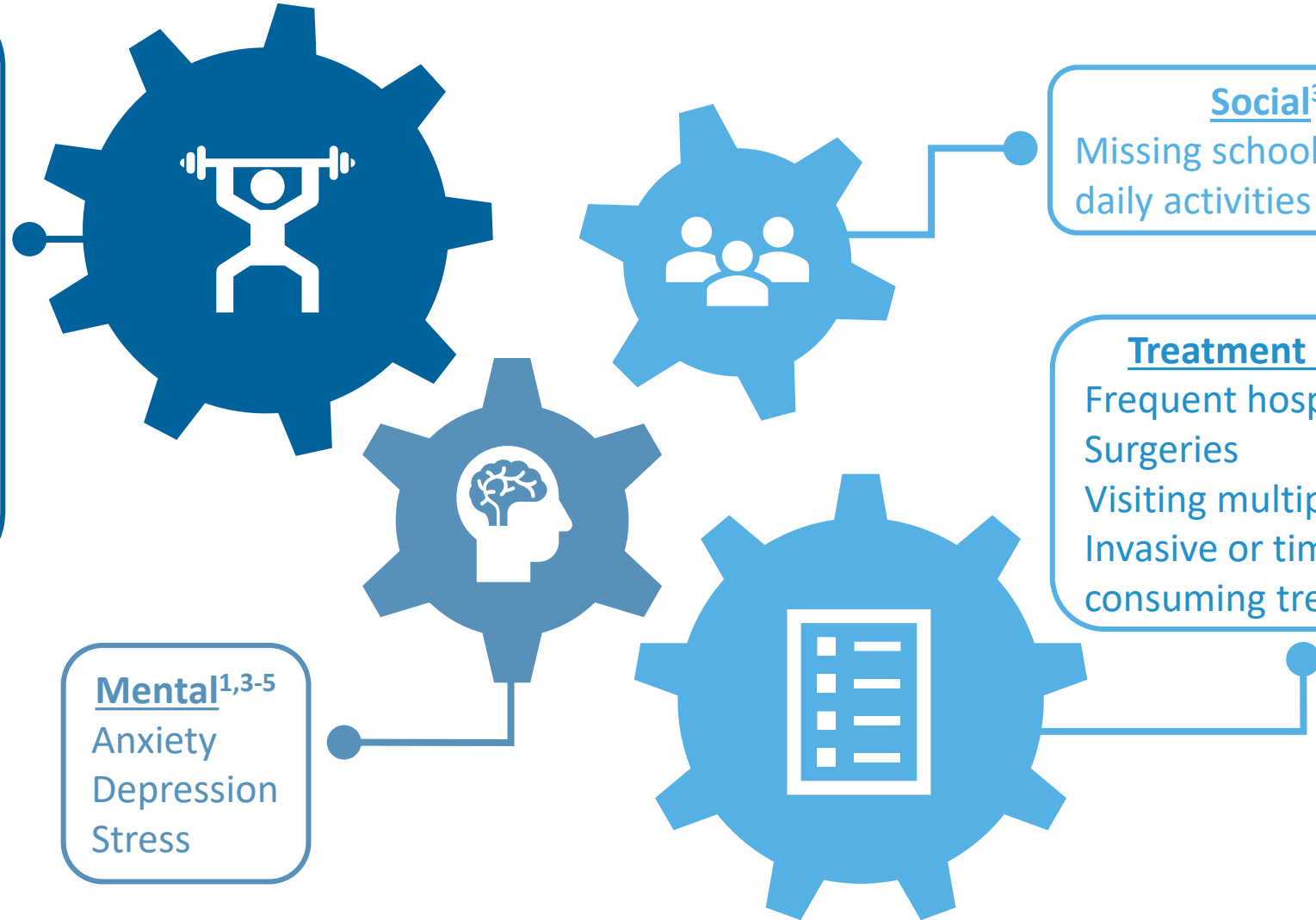
A genetic test can provide a definitive diagnosis of APDS

*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^{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.

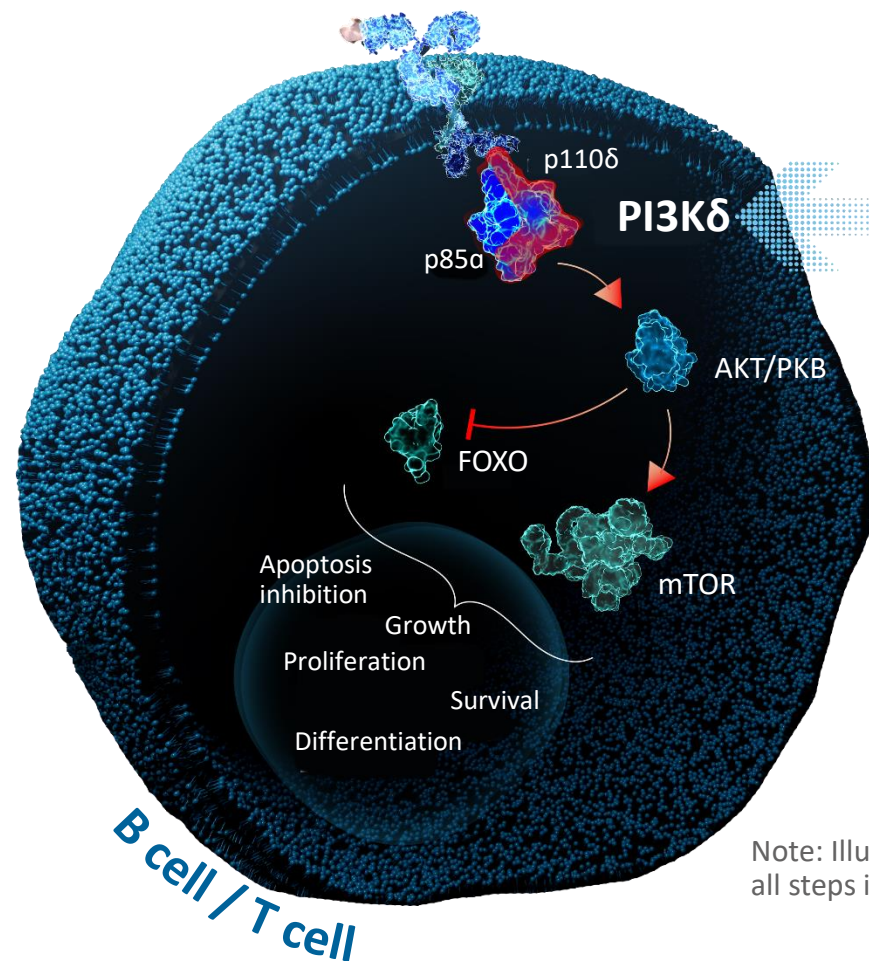
4. Jiang F, et al. *Allergy Asthma Clin Immunol.* 2015;11:27. 5. Kuburovic NB, et al. *Patient Prefer Adherence.* 2014;8:323-330.

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

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

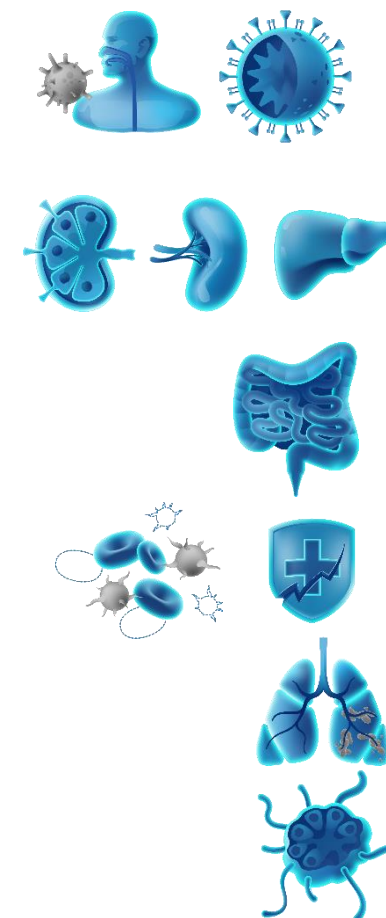


Immune imbalance leads to diverse signs and symptoms^{1,4-6}



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

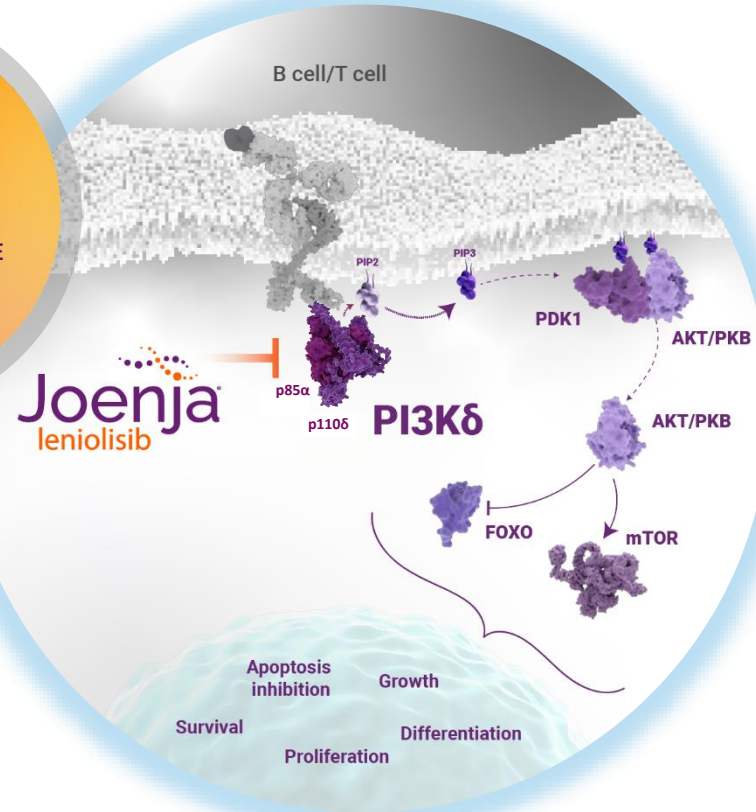
Lymphoma

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.

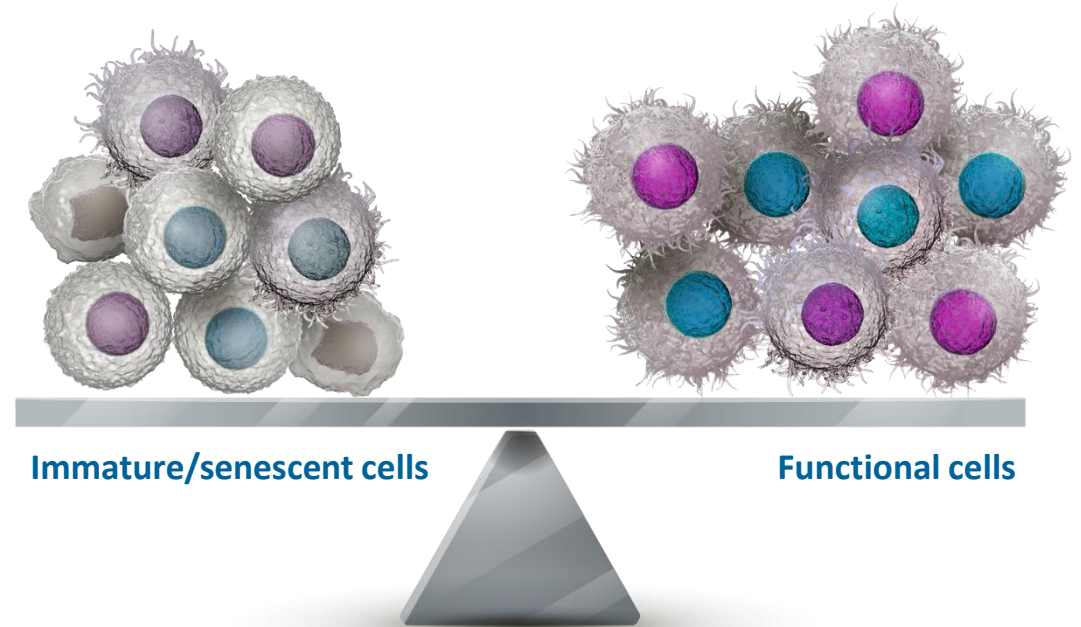
Joenja[®]: immune modulator that targets the root cause of APDS

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.



Joenja[®] (leniolisib)

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

Joenja[®] (leniolisib) is a prescription medicine that is used to treat activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) in adults and pediatric patients 12 years of age and older

In a randomized placebo-controlled trial of patients with APDS

- Joenja[®] met both primary end points with significant efficacy results
- Demonstrated significant improvement in other secondary and exploratory parameters

There were no drug-related serious adverse events or study withdrawals in Joenja[®] trials



Joenja[®] reported additional findings from an ongoing long-term open-label extension study interim analysis: reductions/discontinuations in IRT and reduction in infection rates

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

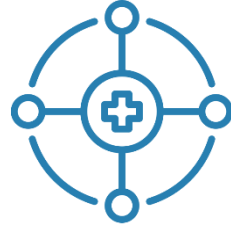
Strong start to Joenja[®] launch with 60 enrollments & 43 patients on paid therapy as of June 30, 2023



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

- ◆ 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

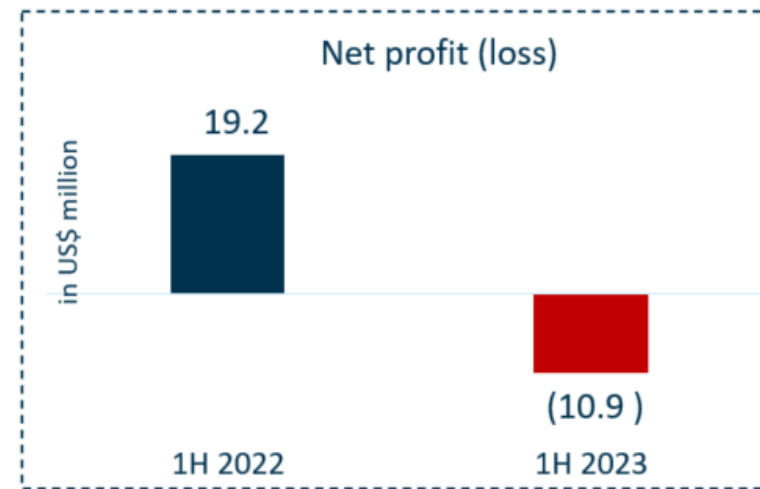
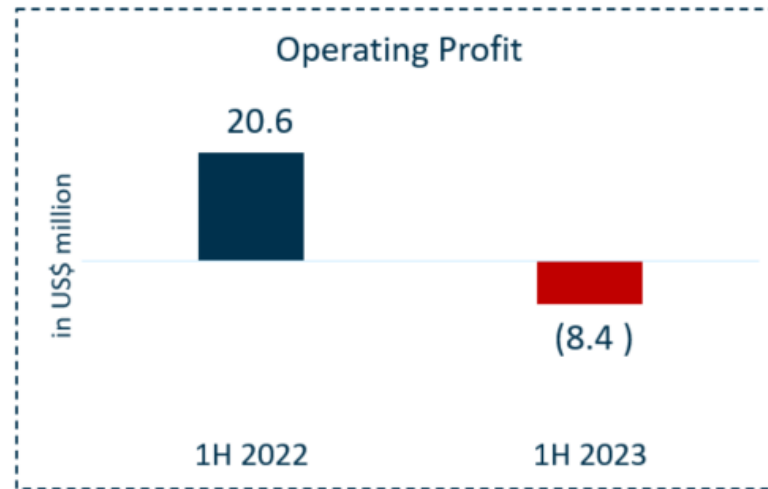
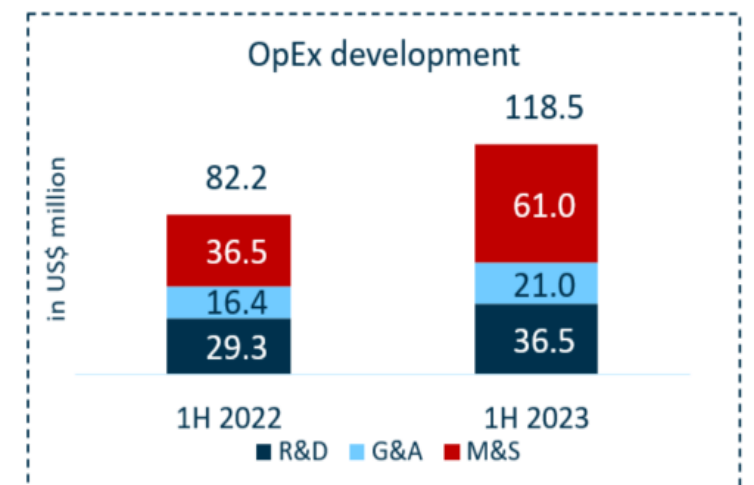
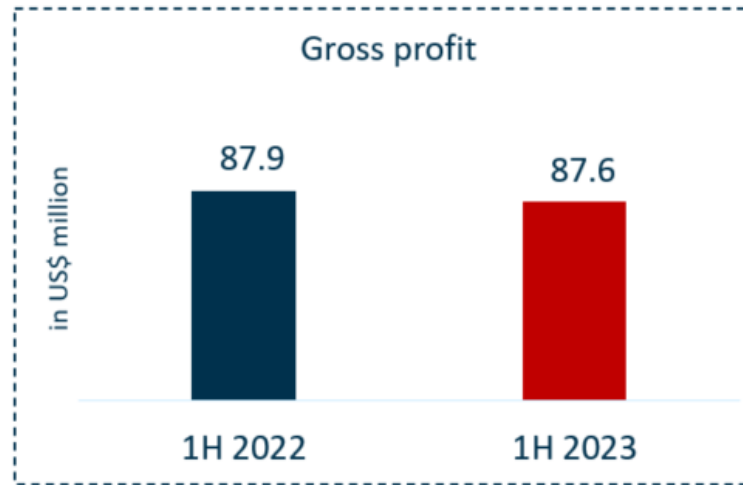
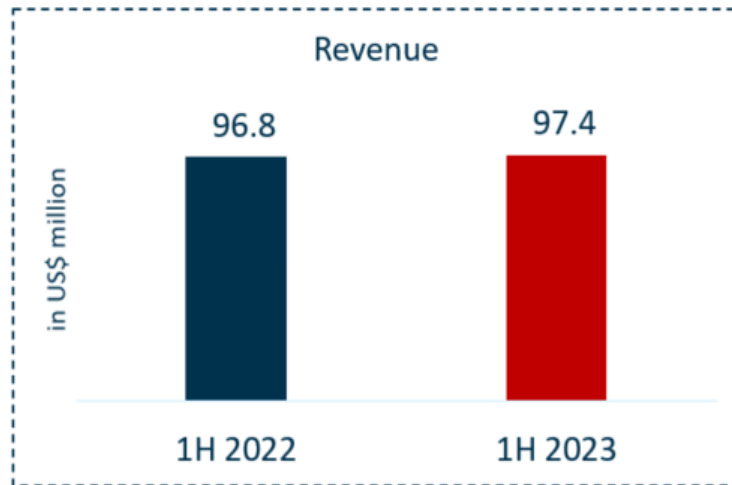
*Received CHMP Day 180 list of outstanding issues in July. CHMP will consult an Ad-hoc Expert Group (AEG) given the rarity of the disease and the unmet medical need for the treatment of APDS patients. Approval is subject to positive outcomes of the EMA CHMP review.

** 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

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



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