Pharming Group N.V.

Oppenheimer 34th Annual Healthcare Life Sciences Conference

February 13, 2024

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 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.

Building a leading global rare disease biopharma company

Pharming®

Market RUCONEST® for acute HAE attacks in key markets – U.S. focus

Positive cash flow from RUCONEST® revenue funds Joenja® (leniolisib) launches & pipeline development

- Updated FY23 revenue guidance ~US$227M* (+10% vs. FY22)
- Significantly exceeding previous guidance of low single digit revenue growth

Global approvals and commercialization of Joenja® (leniolisib) for APDS

Successful commercialization of Joenja® (leniolisib) for APDS

- Joenja® - First and only FDA approved treatment for APDS
- FY23 revenue guidance ~US$18M*
- Regulatory reviews ongoing in EUR, CAN, AUS, ISR
- Pediatric and Japan clinical trials ongoing

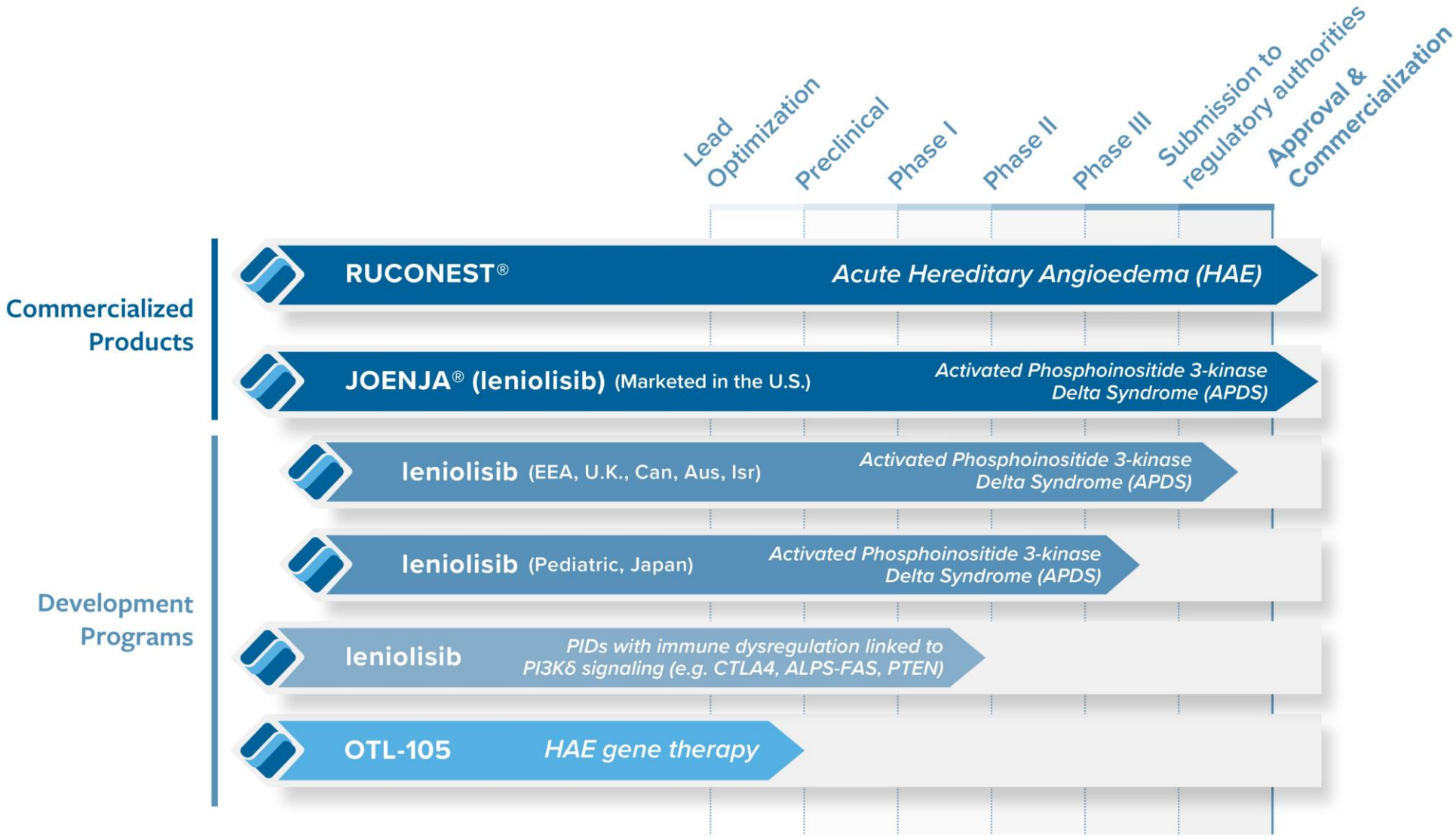
Ongoing pipeline development and management of rare disease assets

Advance internal projects and rare disease in-licensing and acquisition strategy

- Developing leniolisib for Primary Immunodeficiencies with immune dysregulation beyond APDS
- Partnership focus on early to late-stage clinical programs in immunology, hematology, respiratory and gastroenterology

*2023 financial guidance based on preliminary selected financial results that are unaudited and subject to adjustment. Pharming expects to issue full financial results for the fourth quarter and full year 2023 in March 2024. The Company has not completed its financial closing procedures for the quarter or year ended December 31, 2023 and actual results could differ from these preliminary financial results.

Pipeline – multiple commercial stage rare disease products



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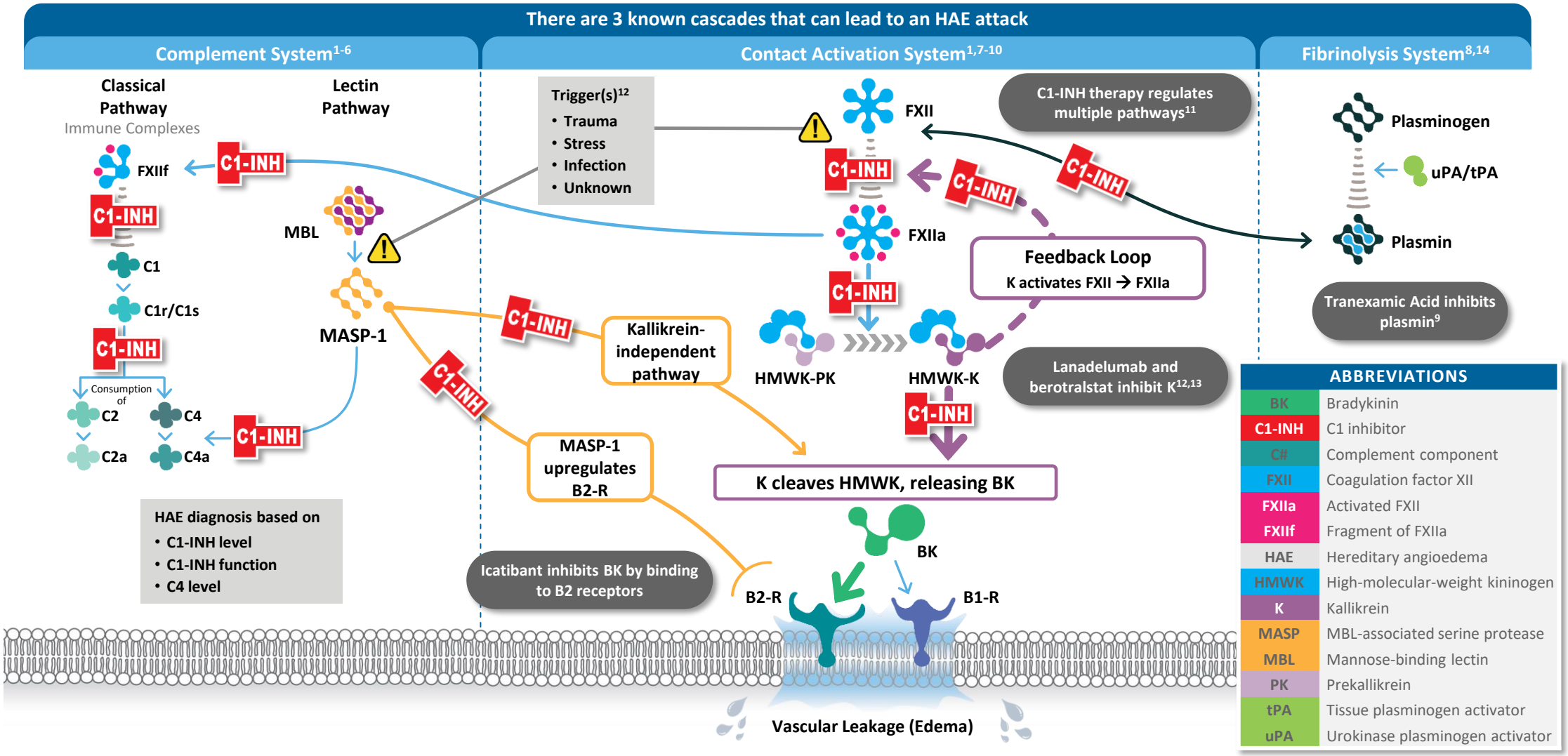
RUCONEST®

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® (rhC1INH) for HAE: still growing after 10+ years

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The only recombinant treatment that targets the root cause of HAE by replacing missing or dysfunctional C1-INH

Second most prescribed product detailed for acute attacks

Well-tolerated and effective treatment option for acute hereditary angioedema (HAE) - including breakthrough attacks

97%: needed just 1 dose of RUCONEST®[1]
93%: acute attacks stopped with RUCONEST® for at least 3 days[2]

Revenue:
FY22 $205.6M
FY23 guidance ~US$227M (+10%)

Strong U.S. in-market demand – New patient enrollments up 25% in FY23 vs. FY22

Significantly increased previous guidance of low single digit revenue growth

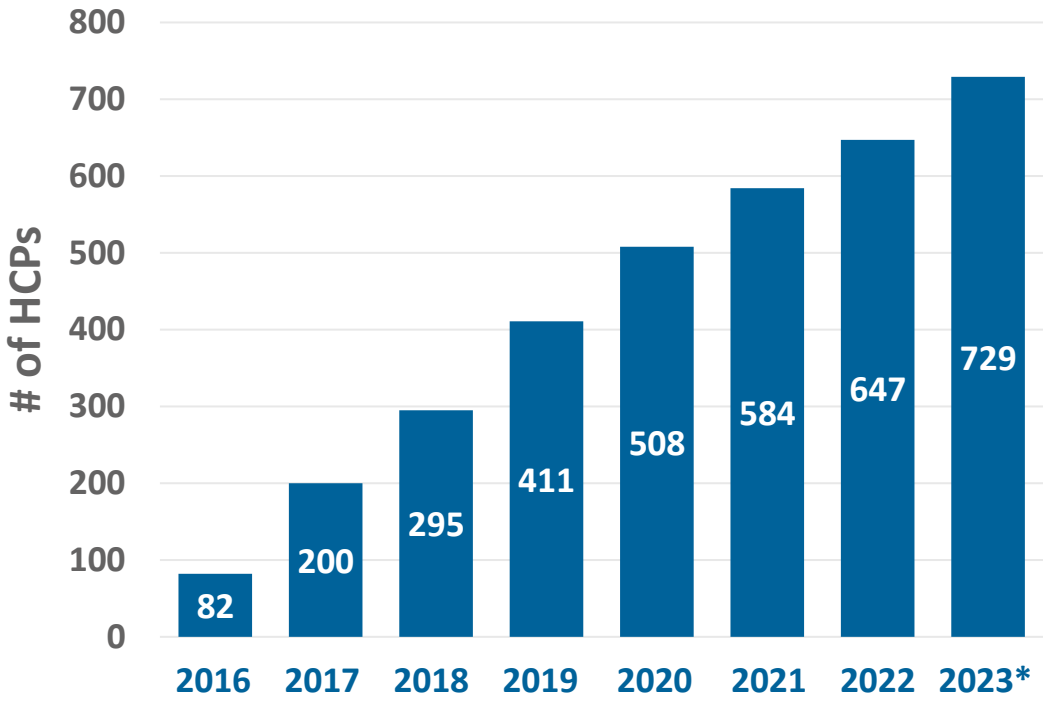
Performing well in leading revenue indicators in the U.S.: active patients, vials shipped, # physicians prescribing

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.

Strong commitment to HAE community

- Strong patient organization support since 2000
- More than 720 U.S. physicians (and growing) prescribing RUCONEST®
- >2,000 patients with HAE have been prescribed RUCONEST®

of unique U.S. physicians prescribing



*Data thru December 31, 2023

US HEREDITARY ANGIOEDEMA ASSOCIATION

HAEi HAE International

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APDS Overview

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

Activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)

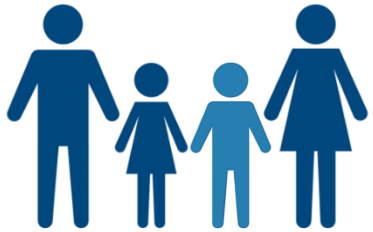
Global prevalence estimated at 1.5 patients per million population*

To date, Pharming has identified >840 diagnosed APDS patients in global markets targeted for commercialization*

(as of December 31, 2023)

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



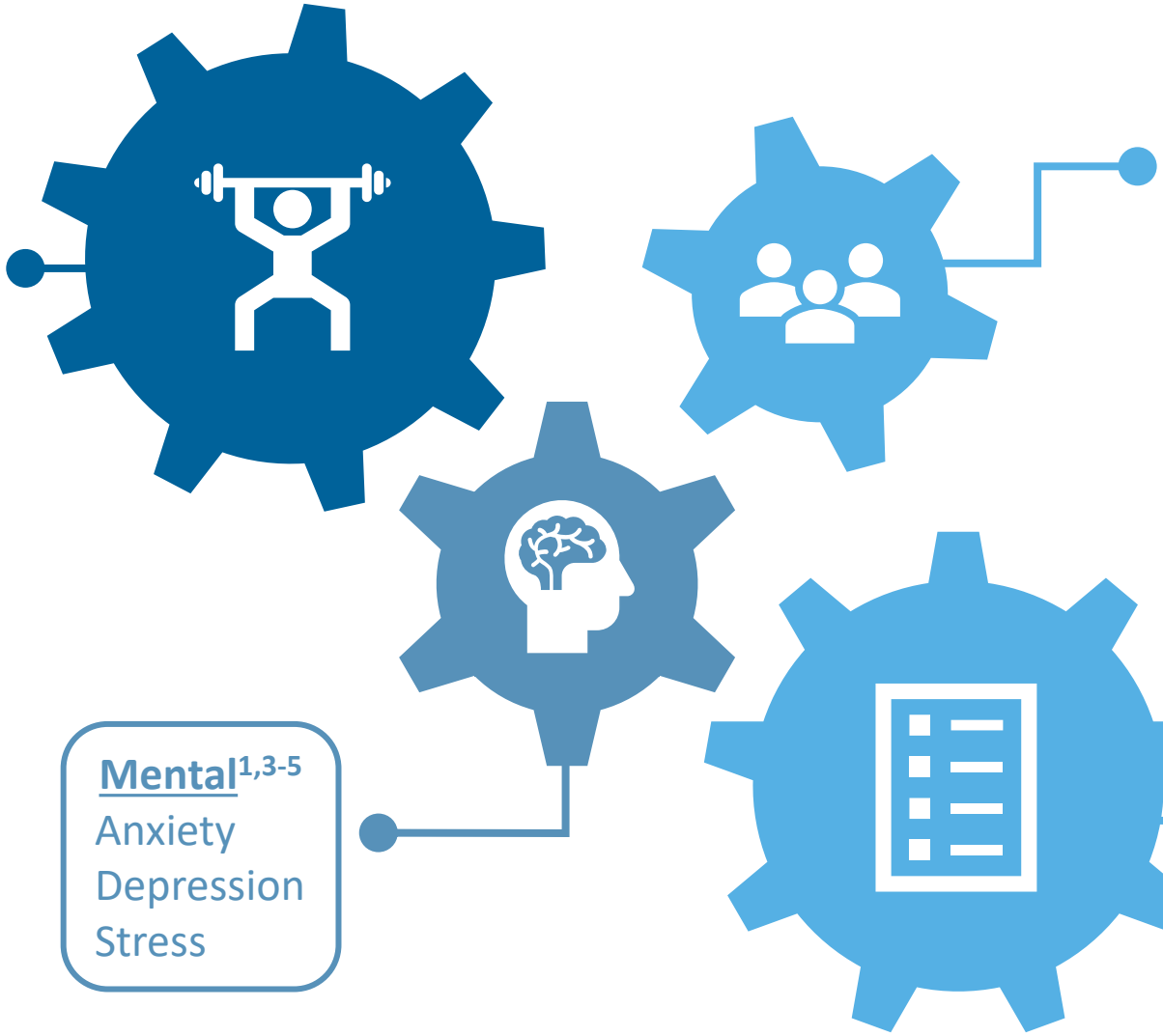
The signs and symptoms of APDS vary widely, even among family members with the same genetic variant, resulting in potential delays in diagnosis and care

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)
>840 patients identified in global markets targeted by Pharming for commercialization
>730 of these patients are in key global launch markets in the U.S., Europe, U.K., Japan, MENA, Turkey, Asia, Canada, Australia, and Israel with total prevalence of ~2000 APDS patients

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 Burden[1-4]
Frequent hospitalizations
Surgeries
Visiting multiple doctors
Invasive or time-consuming treatments

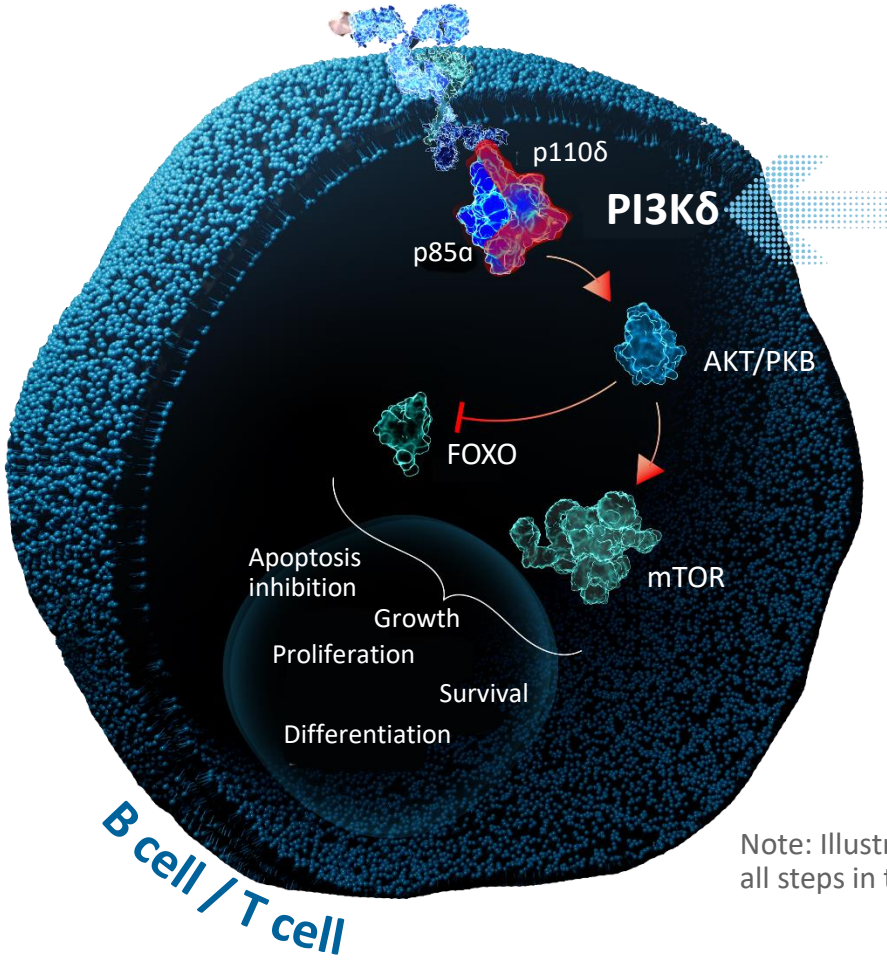
Mental[1,3-5]
Anxiety
Depression
Stress

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 PI3Kδ hyperactivity, disrupting immune cell balance

Hyperactive PI3Kδ results in dysregulated B and T cell development[1-3]

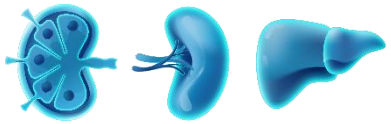


The PI3Kδ enzyme is at the beginning of a complex signaling pathway

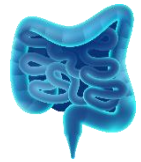
Note: Illustration does not include all steps in the signaling pathway.

Immune imbalance leads to diverse signs and symptoms[1,4-6]

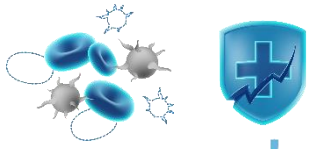
Severe, recurrent, persistent infections
- Sinopulmonary
- Herpesvirus (especially EBV and CMV)



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; PI3Kδ, 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.

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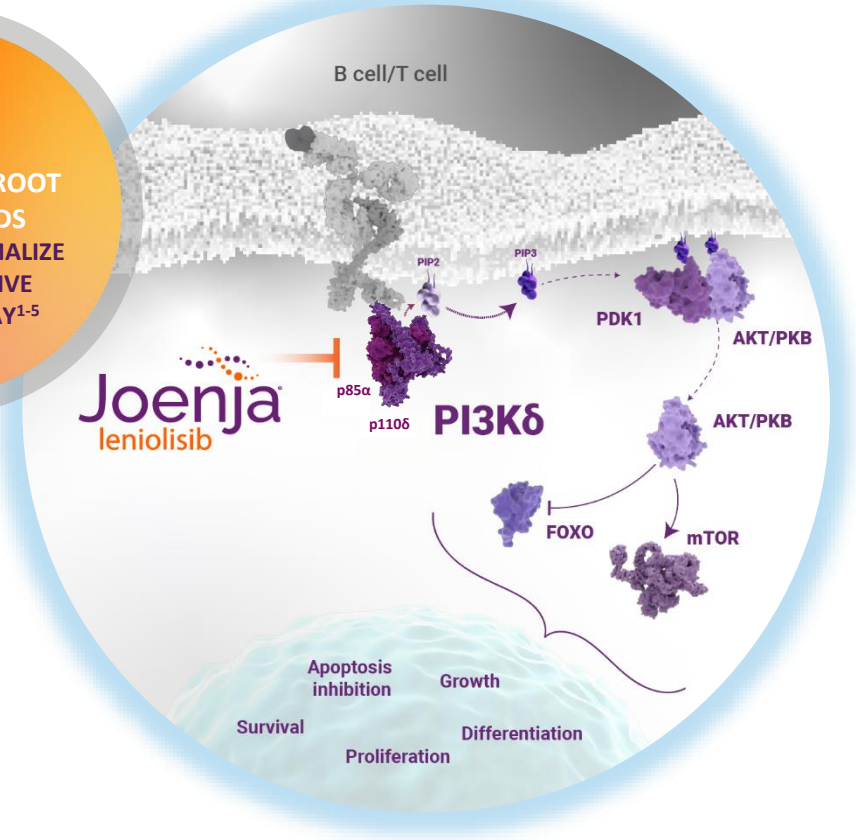
Joenja® (leniolisib)

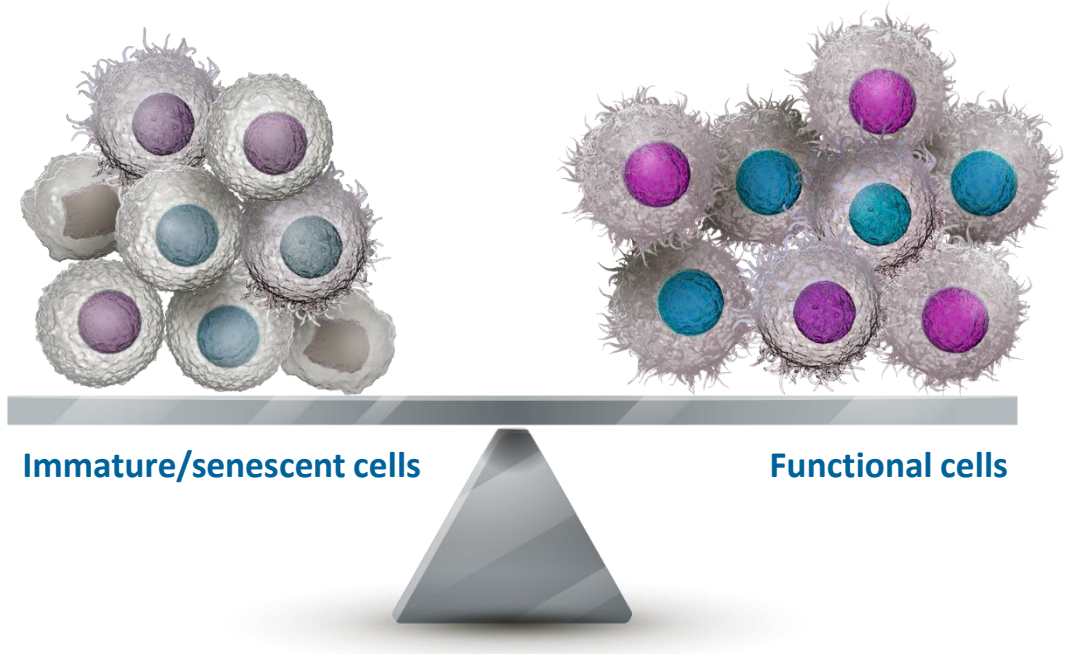
Joenja®: immune modulator that targets the root cause of APDS

JOENJA WAS DESIGNED TO TARGET THE ROOT CAUSE OF APDS TO HELP NORMALIZE THE HYPERACTIVE PI3Kδ PATHWAY[1-5]



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

Joenja® facilitates a balanced PI3Kδ pathway to support proper immune function[6]



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.

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 (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

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

Rao VK, et al. Blood. 2023;141(9):971-983
Rao VK, et al. Poster presented at: 64th Annual American Society of Hematology Annual Meeting; December 10-13, 2022; New Orleans, LA.

Joenja® launch update: continued strong commercial execution

- Strong commercial execution 9 months into U.S. launch
- Continue to enroll patients and add patients on paid therapy in 4Q23
  92 enrollments, of which 81 patients on paid therapy at end 4Q23
- APDS Assist program ensures eligible patients have access to therapy
- FY23 revenue guidance ~US$18M
- Significant focus on genetic family testing
- Validation studies to confirm which variants of uncertain significance (VUS) should be classified as APDS to complete in 4Q24, focused on >1100 patients identified in the U.S. with VUSs

Joenja® – looking beyond FDA approval

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- **Europe – CHMP opinion on MAA expected 1Q24 (approval ~ 2 months later)***
- **UK – MHRA filing expected 1Q24****
- **Japan clinical study – 1st patient enrolled Aug 2023**
- **AUS, CAN, ISR submissions under regulatory review**
  - CAN & AUS approval 2Q24***
  - ISR approval 1H24***
- **Pediatric study for 4 to 11 years: enrollment majority complete**
- **Pediatric study for 1 to 6 years ongoing (first patient dosed)**
- **Named patient program ongoing**
- **Leniolisib development for PIDs with immune dysregulation (start 1st Phase 2 trial 2Q24)**

* Received CHMP Day 180 second list of outstanding issues in November 2023. CHMP rescheduled the Ad-hoc Expert Group (AEG) meeting to the end of November 2023. Approval is subject to positive outcomes of the EMA CHMP review.
** Pharming intends to file an MAA through the International Recognition Procedure (IRP). MHRA would have 110 days from the date the IRP submission is validated to review and issue its decision.
*** Subject to positive AUS, CAN, ISR decisions

Hiding in plain sight: Patient finding strategy

Medical education to raise awareness of APDS and share leniolisib data

- Conferences and congresses
- Abstracts
- Publications

IPIC2023 INTERNATIONAL PRIMARY IMMUNODEFICIENCIES CONGRESS

AMCP NEXUS

American College of Allergy, Asthma & Immunology

Genetic testing

- Sponsored, no-cost testing program

navigateAPDS by Pharming

- Genetic counselors to assist with testing and reviewing results
- Partnering with genetic testing companies to identify previously and newly diagnosed APDS patients

Family testing

- Inherited disease* but most APDS patients do not have diagnosed family members
- Patients may not be aware of genetics or have access to specialty physicians
- Cooperating with clinicians to encourage family testing
- Patients can request a genetic test through partner Genome Medical (if suspect APDS for themselves or family members)
- Reduces barrier for easier testing of those suspected with APDS

*APDS genes are autosomal dominant meaning there is a 50% chance that a blood relative of an APDS patient may also carry that gene and in turn have APDS.

Helping diagnose APDS patients: Variant of Uncertain Significance (VUS) resolution

Genetic testing frequently leads to inconclusive results - previously unseen genetic variants:

Patients have clinical symptoms compatible with APDS, but genetic variant test is inconclusive

Frustrating for patients and clinicians

Need to determine if Variant of Uncertain Significance (VUS) causes APDS

Pharming initiatives/partnerships to resolve VUSs

Variant Curation

- ClinGen expert panels develop gene/disease specific thresholds and criteria for classifying variants
- Partnership with Genomenon to develop Genomic Landscape (comprehensive, systematic review of all published variant data)

Functional testing

- Improve access to directly measure PI3K pathway activity in patient blood samples
- Sharing of results via public databases (ClinVar)

Multiplexed assays of variant effect (MAVE)

- Test nearly all possible variants in a single experiment
- Generate variant effect map, including variants already found and those not yet found (proactive)

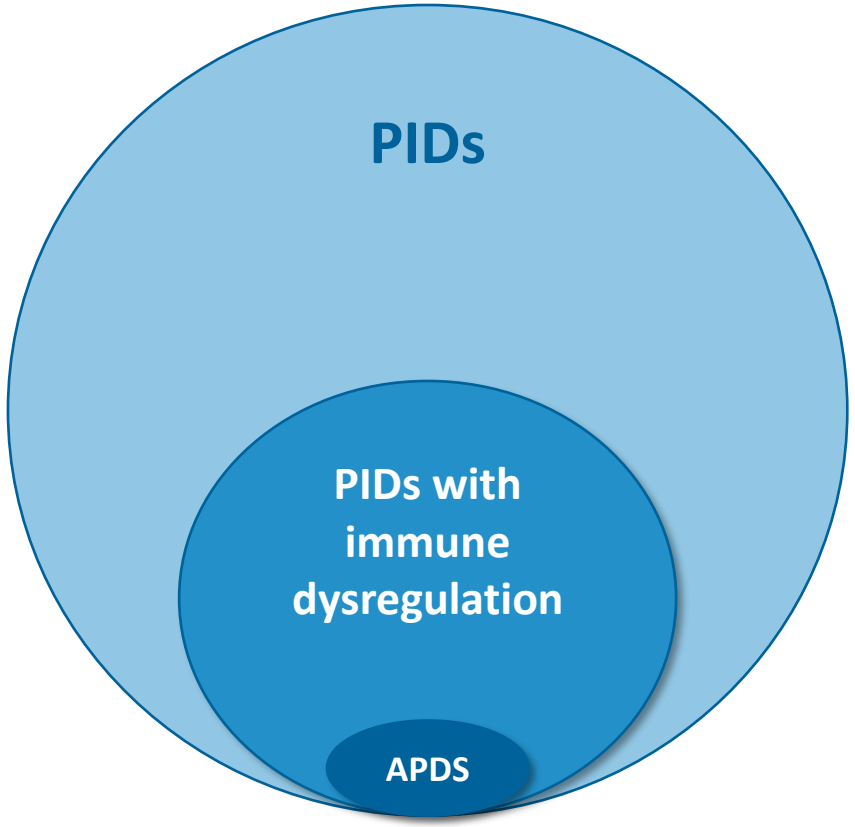
To date Pharming has identified more than 1,100 patients in the U.S. with VUSs. As results become available, patients with validated variants could be diagnosed with APDS and be eligible for Joenja® treatment. Completion of these studies is expected during 4Q 2024.

Primary Immunodeficiencies (PIDs) with immune dysregulation

PIDs are a broad group of disorders[1] with key features:

- Genetic basis, i.e., not secondarily caused by another disease
  'Inborn Errors of Immunity' (IEI) is used interchangeably with PID
- An increased risk of infection may be the predominant manifestation, due to poor immune system function
- PID patients may have a predominance of **immune dysregulation**, for example: lymphoproliferation and autoimmunity[2]

APDS is an example of a PID with immune dysregulation



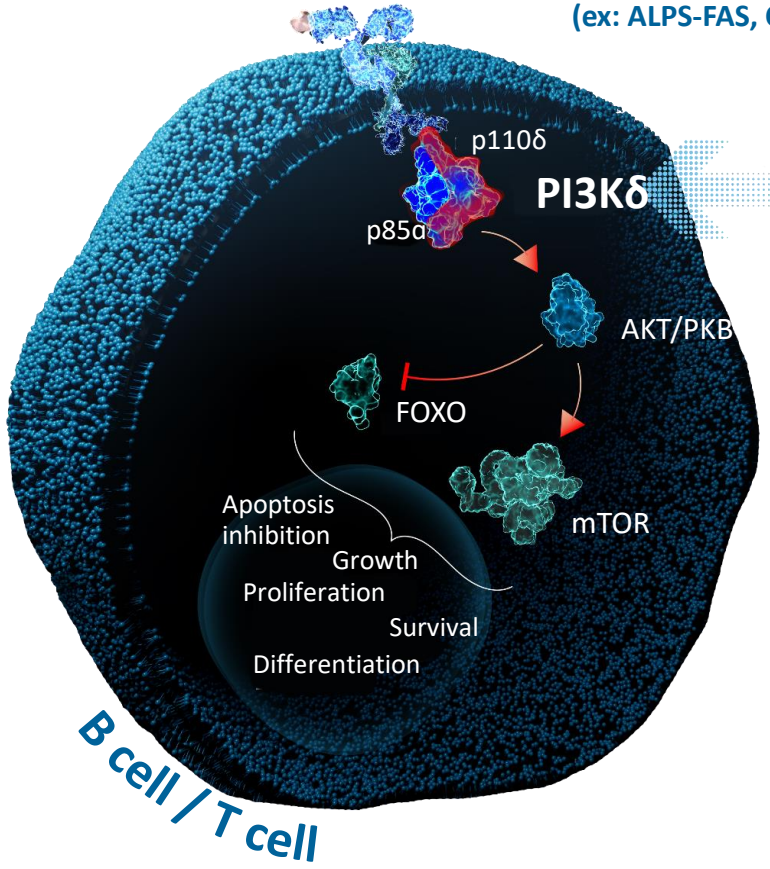
Not to scale with population sizes

1. Bousfiha et al 2022 IUIS categorization
2. Chan and Torgerson 2020 Curr Opin Allergy Clin Immunol 20(6): 582-590

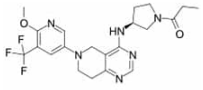
Given importance of PI3Kδ in B & T cells, immune dysregulation in PIDs can occur via alterations in PI3Kδ signaling

Altered PI3Kδ signaling can occur in multiple PID genetic disorders beyond APDS

(ex: ALPS-FAS, CTLA4, PTEN)



leniolisib



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

High unmet medical need

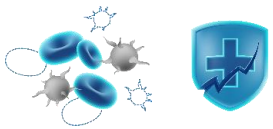
- no approved therapies other than Joenja® (leniolisib) for APDS:

SOC immunosuppressives (e.g. rapamycin) have limited efficacy and significant tolerability concerns

Clinical manifestations, disease onset and severity similar to APDS

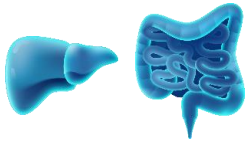
Lymphoproliferation

- Lymphadenopathy
- Splenomegaly/hepatomegaly
- Nodular lymphoid hyperplasia



Autoimmunity

- Cytopenias
- Autoimmune disorders
- Autoinflammation



GI Disease

- Autoimmune enteropathy
- Nodular regenerative hyperplasia

Pulmonary Disease

- GLILD
- Bronchiectasis

Infections

- Sinopulmonary
- Herpesvirus

Lymphoma

FOXO, forkhead box O; mTOR, mammalian target of rapamycin; PI3Kδ, 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.

Leniolisib development for PIDs with immune dysregulation

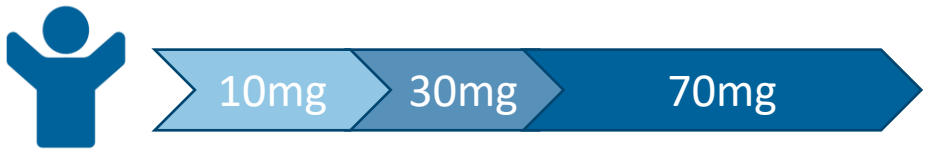
- Based on APDS experience, leniolisib has potential to be an effective & tolerable chronic treatment approach for PIDs with immune dysregulation
- Leniolisib, by reducing PI3Kδ activity, should help rebalance immune dysregulation in PIDs, positively impacting clinical manifestations including lymphoproliferation and autoimmunity
- Initial development in PID genetic disorders with immune dysregulation linked to PI3Kδ signaling in lymphocytes with similar clinical phenotypes to APDS, e.g. ALPS-FAS[1], CTLA4 haploinsufficiency[2], PTEN deficiency[3]
  - Epidemiology suggests **prevalence of ~5/million[4]**
  - FDA review / feedback received on clinical trial plans
- Phase 2 proof of concept clinical trial to commence 2Q 2024

1. Rao VK and Oliveria JB. How I treat autoimmune lymphoproliferative syndrome. Blood 2011; 118(22):5741-51
2. Westerman-Clark et al 2021; Schwab C, Gabrysch A, Olbrich P, Patiño V, Warnatz K, et al. Phenotype, penetrance, and treatment of 133 cytotoxic T-lymphocyte antigen 4-insufficient subjects. J Allergy Clin Immunol. 2018;142(6):1932-1946
3. Eissing M, Ripken L, Schreibelt G, Westdorp H, Ligtenberg M, Netea-Maier R, Netea MG, de Vries IJM, Hoogerbrugge N. PTEN Hamartoma Tumor Syndrome and Immune Dysregulation. Transl Oncol. 2019;12(2):361-367
4. Size based on estimate of 5 patients per million (based on Pharming literature review, KOL feedback and review of patient registries)

PIDs linked to PI3Kδ signaling – Phase 2 study design

Phase 2 proof of concept clinical trial – single arm, open-label, dose range-finding study

Ph2 (N=12)



- Patients with PIDs linked to PI3Kδ signaling, e.g. ALPS-FAS, CTLA4 haploinsufficiency, PTEN deficiency
- Primary: Safety & Tolerability
- Secondary/Exploratory: PK/PD, efficacy measures
- 10/30/70 mg: 4/4/12 wks treatment, respectively
- Pick Best Dose regimen for Ph3

NIH National Institute of Allergy and Infectious Diseases

Lead Investigator: Gulbu Uzel, M.D., Senior Research Physician

Co-Investigator: V. Koneti Rao, M.D., FRCPA, Senior Research Physician
Primary Immune Deficiency Clinic (ALPS Clinic)

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Financials and Outlook

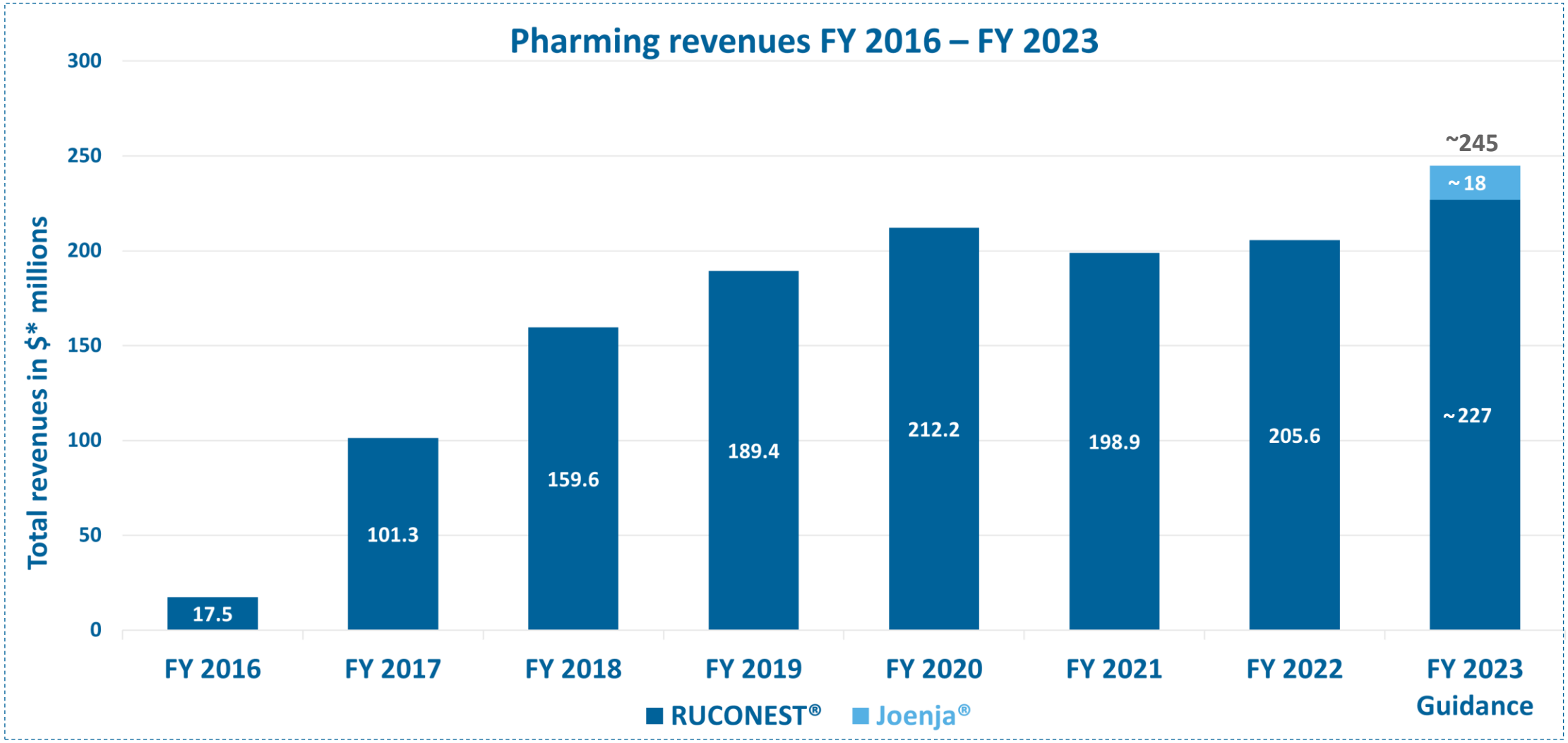
Updated FY 2023 Guidance (preliminary and unaudited)*

	FY 2023 Revenue Guidance (preliminary and unaudited)*	% Growth vs. FY 2022
RUCONEST®	~ US$227 million	10%
Joenja®	~ US$18 million	N/A
Total	~ US$245 million	19%

- RUCONEST® guidance increased from previous low single digit revenue growth
- Cash and cash equivalents, together with restricted cash and marketable securities, are expected to increase to US$215.0 million at the end of 2023, compared to US$199.2 million at the end of 3Q 2023 and US$208.7 million at the end of 2022

*2023 financial guidance based on preliminary selected financial results that are unaudited and subject to adjustment. Pharming expects to issue full financial results for the fourth quarter and full year 2023 in March 2024. The Company has not completed its financial closing procedures for the quarter or year ended December 31, 2023 and actual results could differ from these preliminary financial results.

RUCONEST® and Joenja® driving revenue growth



- From FY 2016 – FY 2020 Pharming Group reported earnings in EUR. Revenues during this time frame have been converted to USD. In 2021, Pharming Group began reporting earnings in USD.
- 4Q 2020 and 1Q 2021 quarterly fluctuations and volatility from COVID-19
- During the first quarter 2020, Pharming restructured and expanded its U.S. salesforce. 2023 was the first full year post-pandemic following this restructuring and expansion.
- 2023 financial guidance based on preliminary selected financial results that are unaudited and subject to adjustment. Pharming expects to issue full financial results for the fourth quarter and full year 2023 in March 2024. The Company has not completed its financial closing procedures for the quarter or year ended December 31, 2023 and actual results could differ from these preliminary financial results.

Pharming summary and outlook

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- Significantly increased RUCONEST® 2023 revenue guidance to ~US$227M (10% growth)
- Joenja® launched early April 2023 – 81 patients on paid therapy & expect ~US$18M revenues in 2023
- Leniolisib CHMP opinion expected in 1Q24, marketing authorization in Europe ~2 months later*
- Additional potential leniolisib regulatory approvals in 2024 – UK, CAN, AUS, ISR**
- Continued operating cost investments to accelerate future growth
- Developing leniolisib for additional PIDs genetic disorders with higher prevalence
  Phase 2 clinical trial in PIDs with immune dysregulation linked to PI3Kδ to start 2Q 24
- Investment and continued focus on in-licensing or acquisitions of early to late-stage rare disease clinical programs in immunology, hematology, respiratory and gastroenterology

* Approval is subject to positive outcomes of the EMA CHMP review.
** Pharming intends to file an MAA in the UK through the International Recognition Procedure (IRP). Subject to positive AUS, CAN, ISR decisions.

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