



Pharming Group N.V.
Jefferies Healthcare
Conference

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

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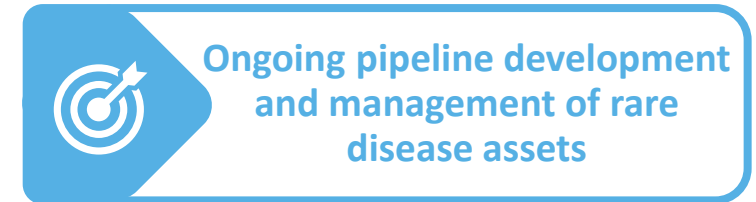
Positive cash flow from >\$200 million TTM sales RUCONEST® funds Joenja® (leniolisib) launches & pipeline development

- ◆ RUCONEST® strong revenue growth 3Q23 +18% vs 2Q23 and +11% vs 3Q22
- ◆ 9M23 RUCONEST® revenue +2% vs 9M22
- ◆ On track for low single digit revenue growth for 2023



Successful commercialization of Joenja® (leniolisib) for APDS

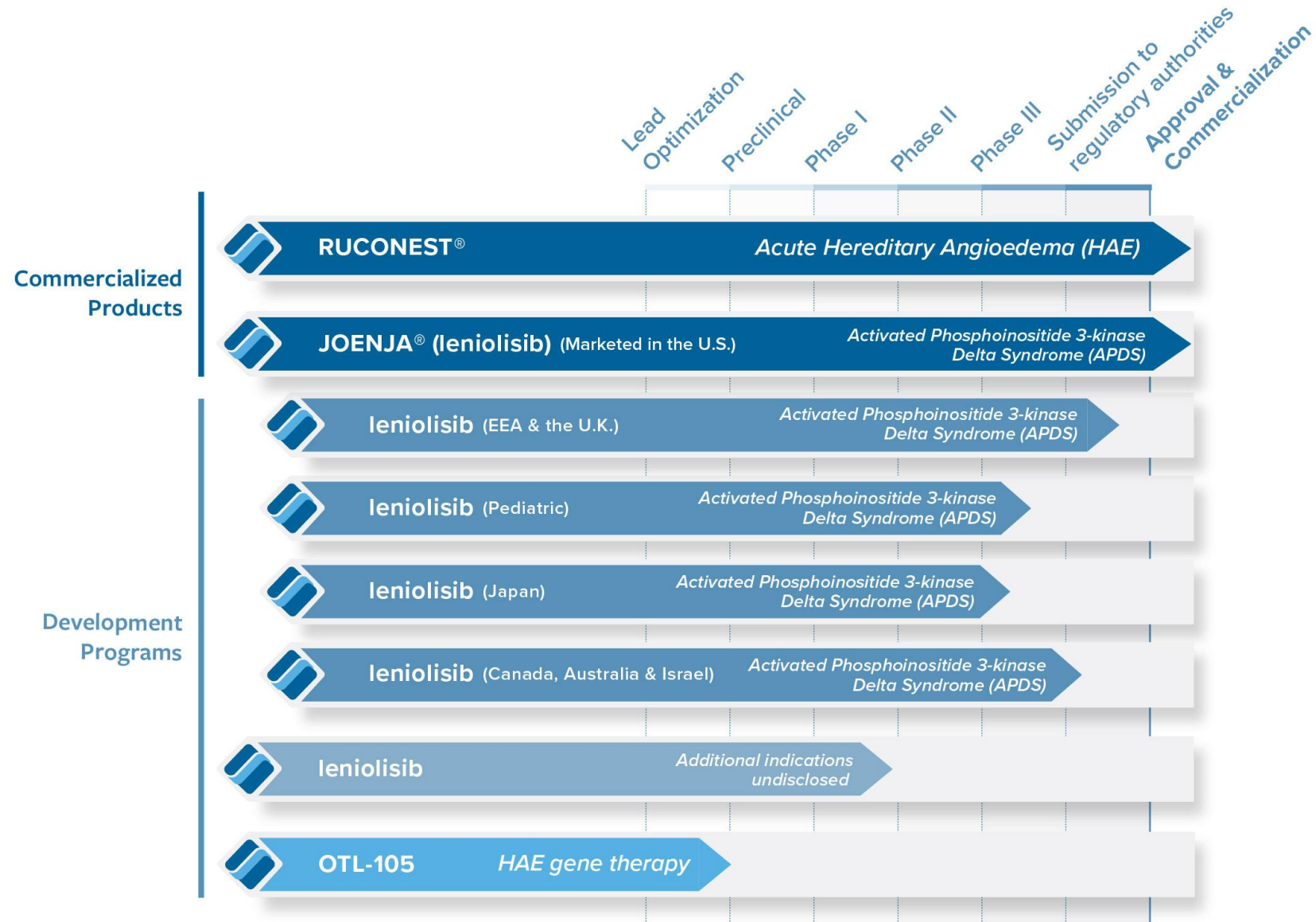
- ◆ MAR: FDA approval for Joenja® SEP: Strong 3Q U.S. revenues US\$6.5M / YTD US\$10.3M
- ◆ Regulatory reviews ongoing in EUR, CAN, AUS, ISR
- ◆ Pediatric clinical program ongoing



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

- ◆ Advanced development plans for 2nd leniolisib indication – further details by end 2023
- ◆ Investments and continued focus on in-licensing or acquisitions of mid to late-stage opportunities in rare diseases.

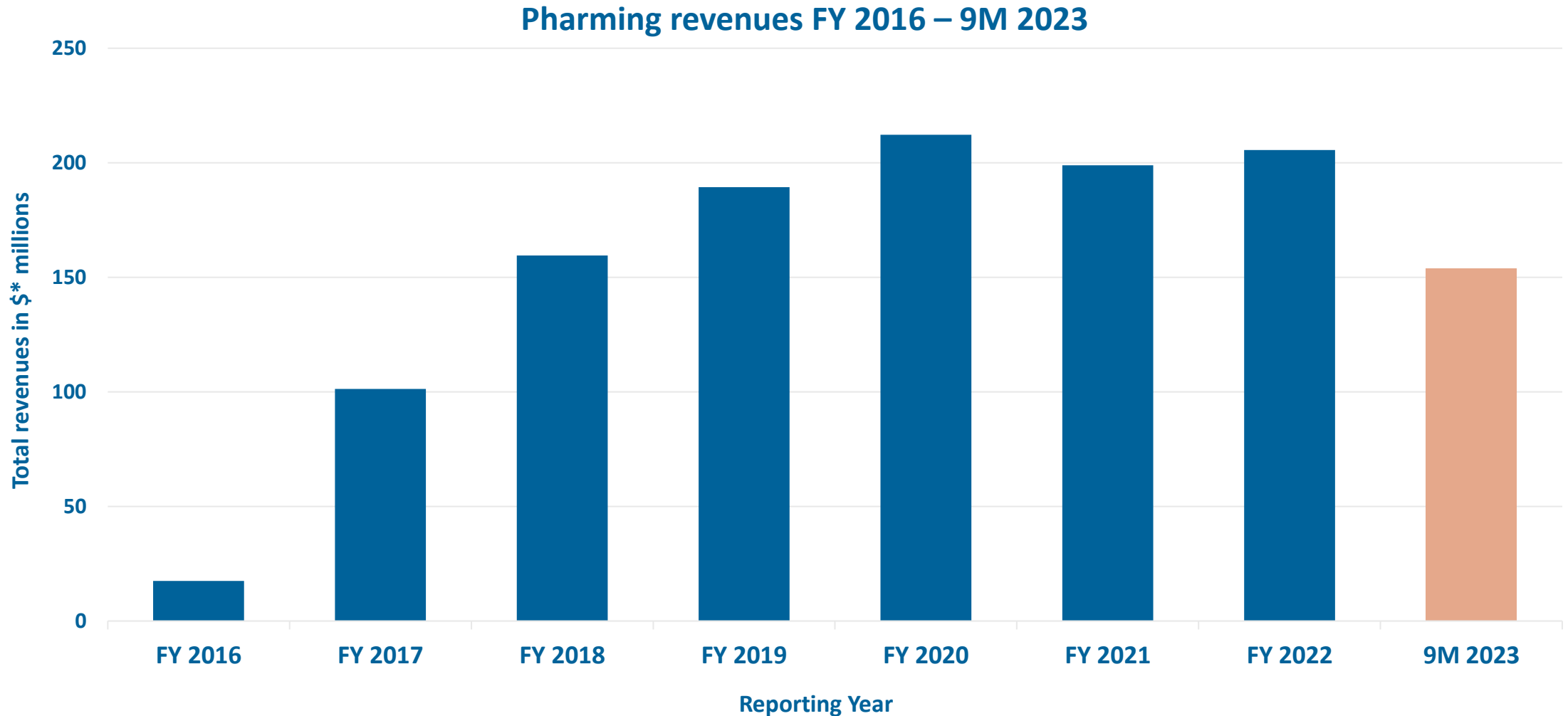
Pipeline – multiple commercial stage rare disease products



 Pharming® | 35 years

RUCONEST®

Pharming Group revenues since reacquiring RUCONEST® rights from Valeant Pharmaceuticals



- 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



Revenues increased 11% in 3Q23 (US\$60.2m) vs 3Q22
Revenues increased 2% in 9M23 (US\$153.8m) vs 9M22



Performed well in leading revenue indicators in the U.S. including active patients, vials shipped, and number of physicians prescribing



Strong U.S. in-market demand – over 70 new patient enrollments for 3 straight quarters



On track for low single digit revenue growth for 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*

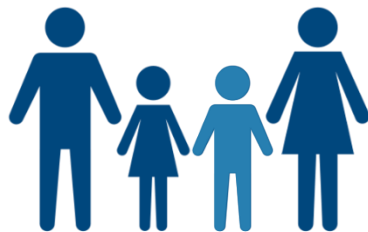
To date, Pharming has identified >640 of these patients in key global markets

(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



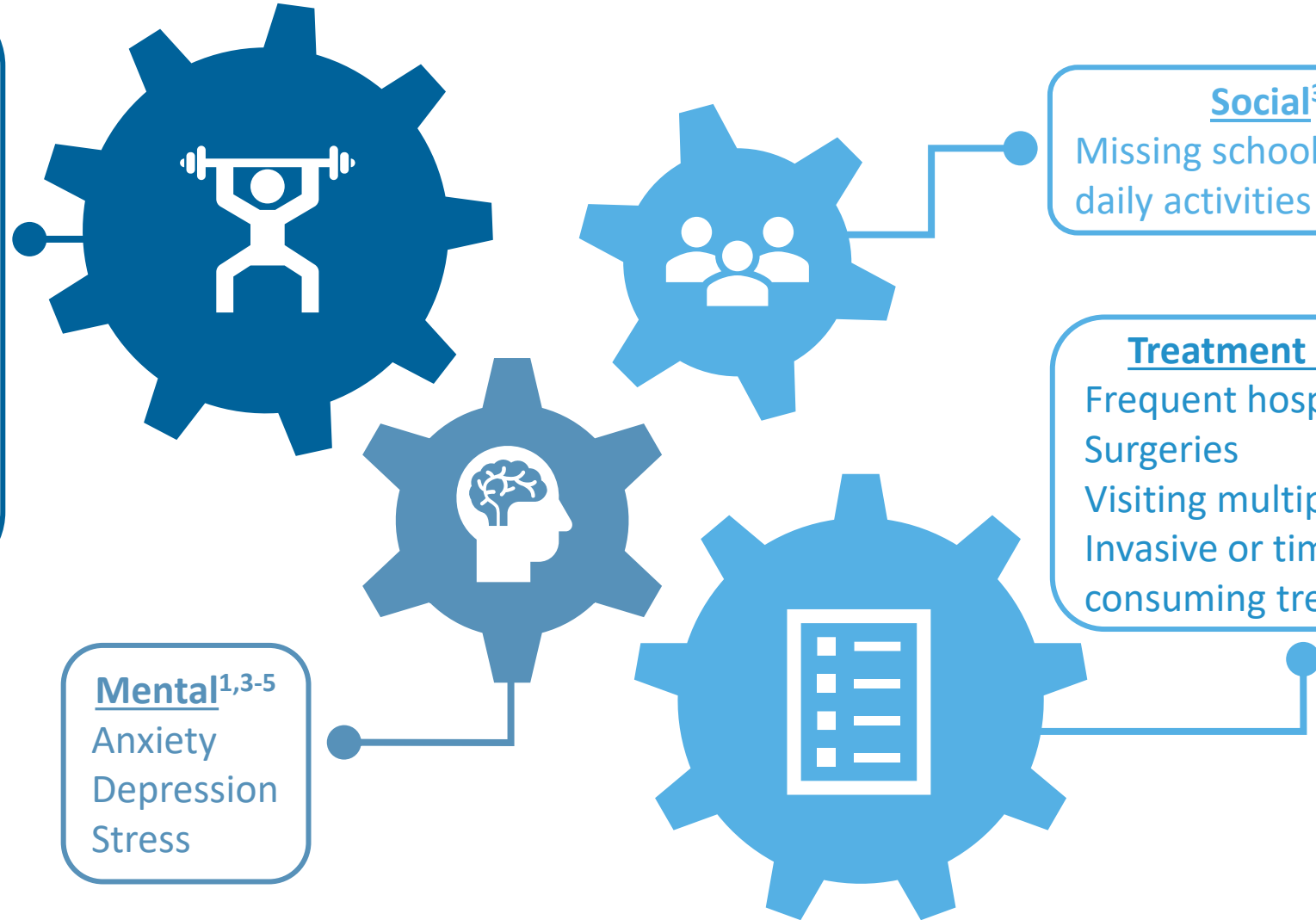
A genetic test can provide a definitive diagnosis of APDS

*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.

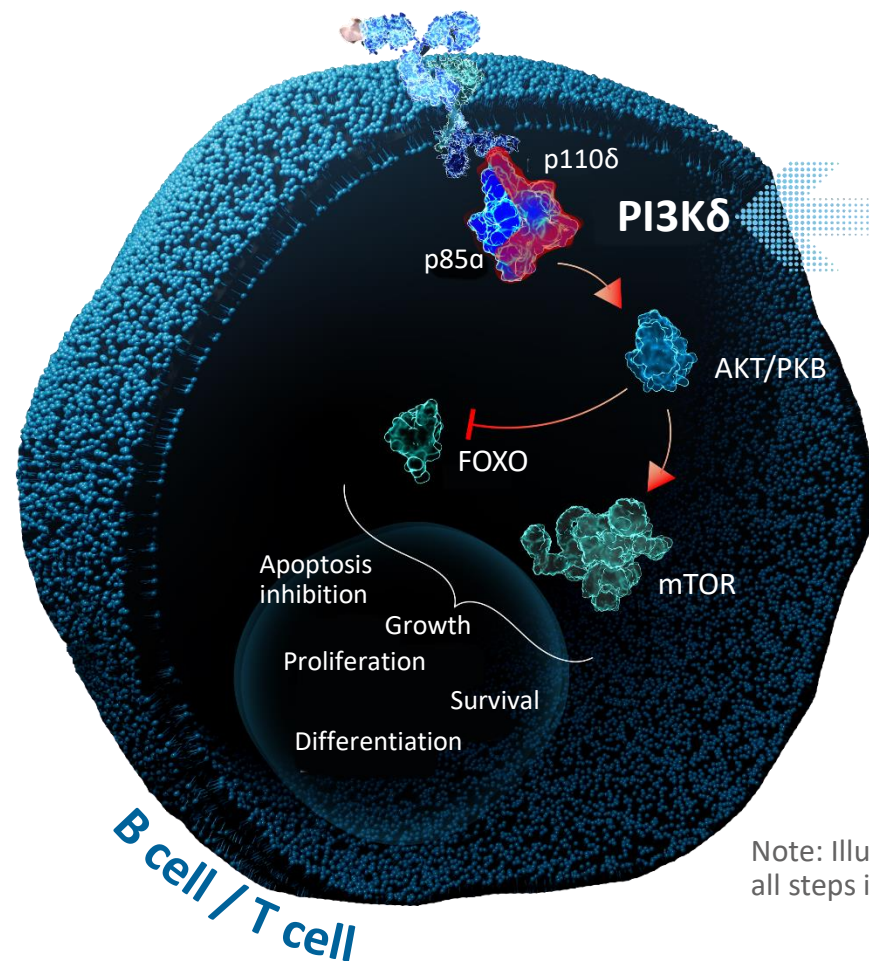
4. Jiang F, et al. *Allergy Asthma Clin Immunol.* 2015;11:27. 5. Kuburovic NB, et al. *Patient Prefer Adherence.* 2014;8:323-330.

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

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

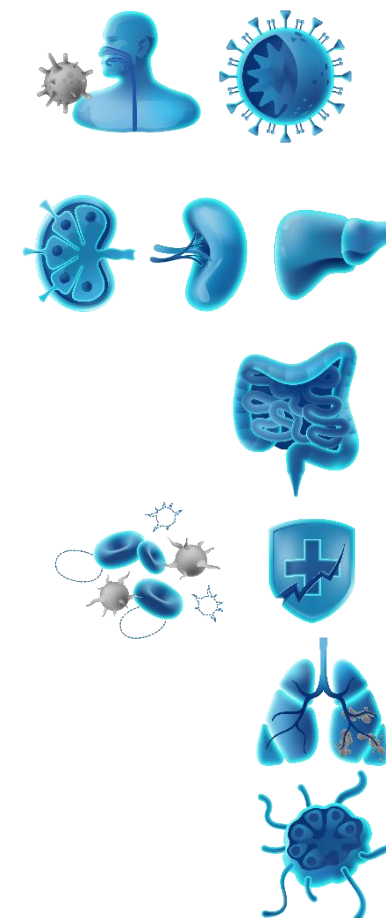


Immune imbalance leads to diverse signs and symptoms^{1,4-6}



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

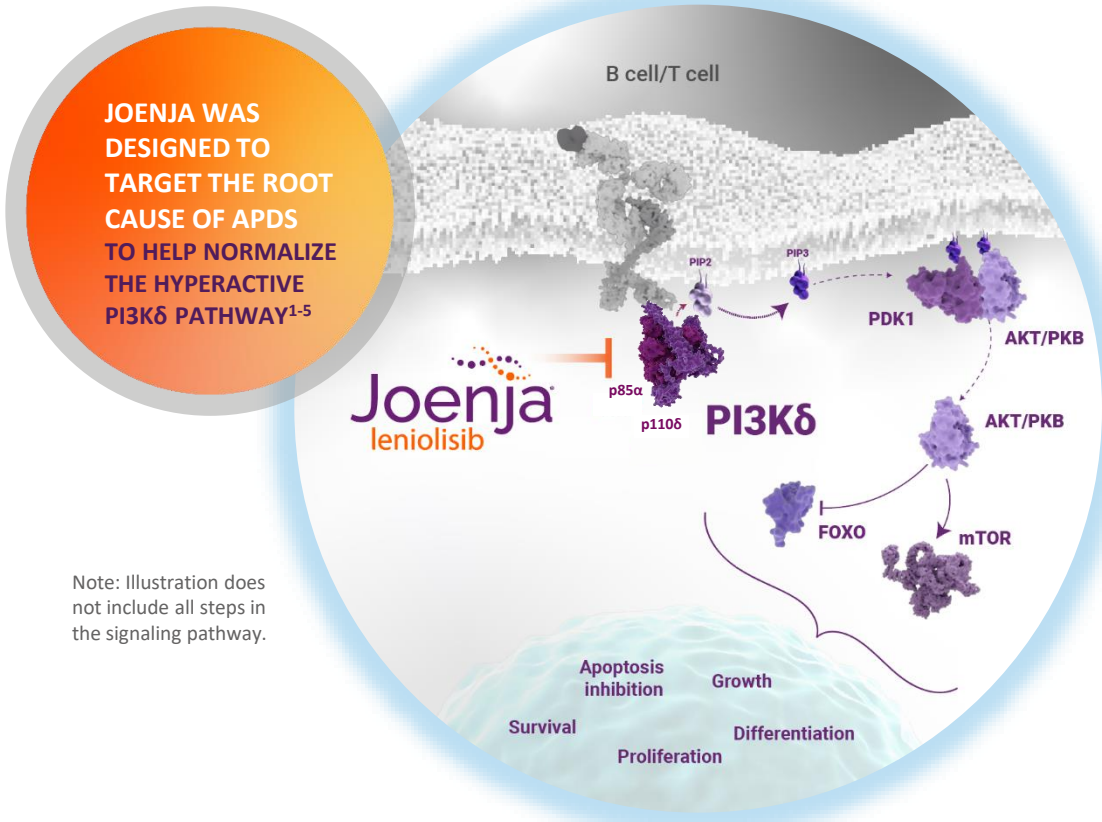
- Cytopenias
- Autoimmune disorders
- Autoinflammatory disorders

Bronchiectasis

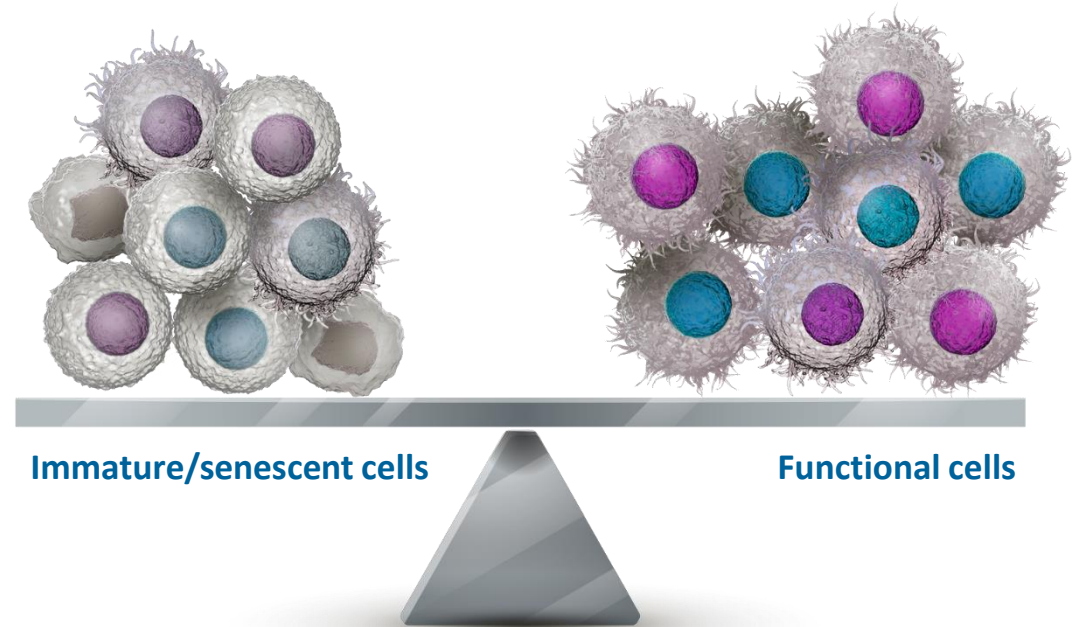
Lymphoma

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[®] facilitates a balanced PI3Kδ 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; 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[®] (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 (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

Strong start to Joenja® launch with 76 enrollments & 63 patients on paid therapy as of September 30, 2023

-  Strong commercial execution 6 months into U.S. launch
-  Continue to add enrollments
76 enrollments, of which 63 patients on paid therapy at end 3Q23
-  All but one pre-existing OLE/EAP patients enrolled or are on paid therapy
37 patients on paid therapy were previously untreated patients or naïve
-  3Q23 revenues: US\$6.5 million
9M23 revenues: US\$10.3 million
-  Significant focus on genetic family testing
Ramp up in 4Q23 and 1Q24
-  Productive ongoing engagement with both national and regional payers
Annual cost (WAC) - US\$ 547,500





Medical education to raise awareness of APDS and share leniolisib data

- ◆ Conferences and congresses
- ◆ Abstracts
- ◆ Publications



Genetic testing

- ◆ Sponsored, no-cost testing program



- ◆ 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)



Europe – CHMP opinion on MAA expected 1Q24 (approval ~ 2 months later)*



Japan clinical study – 1st patient enrolled Aug 2023



Named patient program ongoing



Provide details on development plans for 2nd indication for leniolisib in 4Q23



UK – MHRA filing expected 1Q24 (IRP route will be followed)**



AUS, CAN, ISR submissions processing as anticipated

CAN & AUS approval 2Q24***
ISR approval 1H24***



Pediatric study for 4 to 11 years: enrollment majority (11/15) complete



Pediatric study for 1 to 6 years:
Trial now recruiting

* Received CHMP second Day 180 list of outstanding issues in November. 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











** Will now file using the IRP route as of Jan 1, 2024 with FDA approval

*** Subject to positive AUS, CAN, ISR decisions



Financials and Outlook

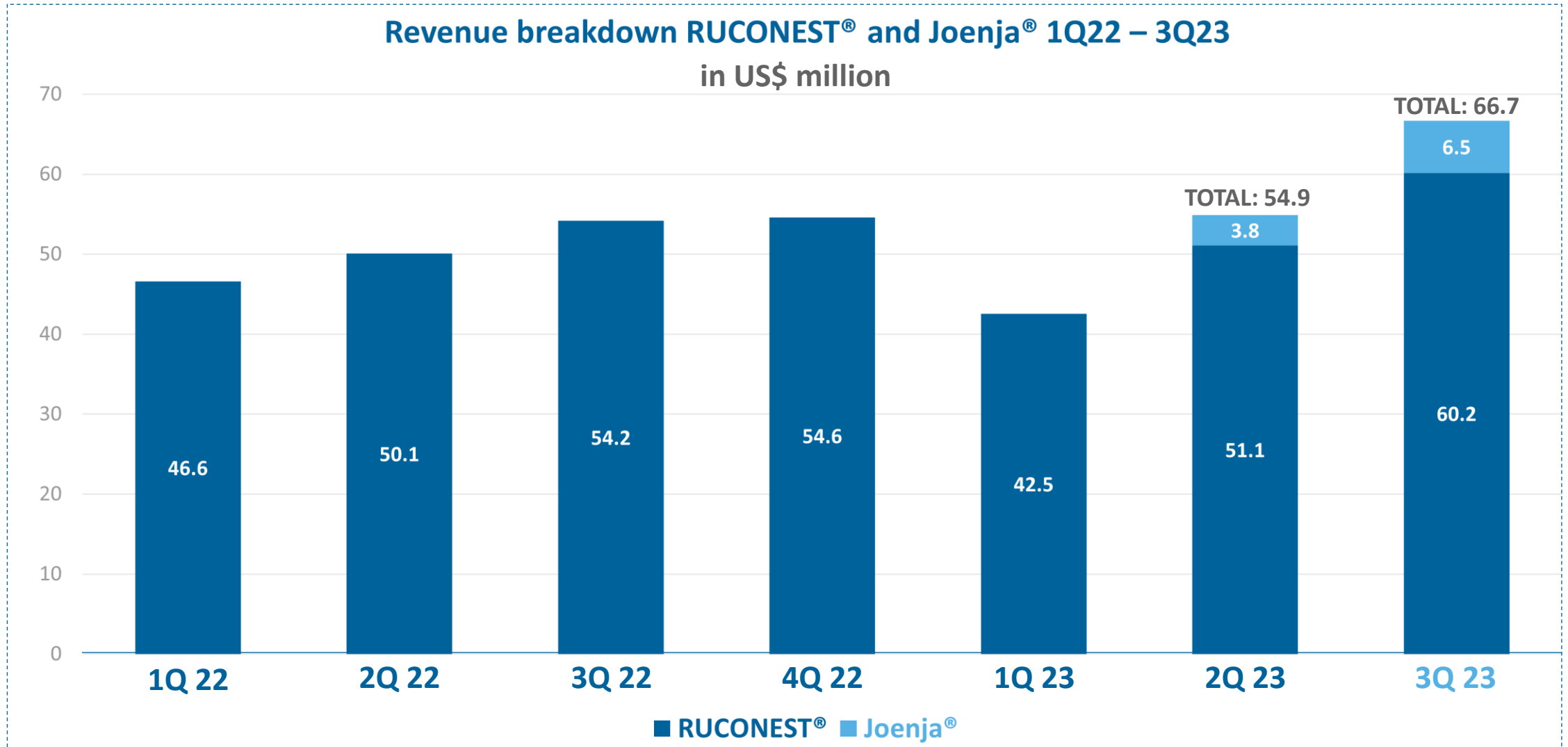
Financial highlights: 3Q 2023 vs 3Q 2022

TOTAL REVENUES 3Q 2022	US\$54.2 million		TOTAL REVENUES 3Q 2023	US\$66.7 million	
GROSS PROFIT 3Q 2022	US\$51.9 million		GROSS PROFIT 3Q 2023	US\$58.4 million	
OPERATING COSTS 3Q 2022	US\$(44.7) million		OPERATING COSTS 3Q 2023	US\$(56.8) million	
OPERATING PROFIT (LOSS) 3Q 2022	US\$7.8 million		OPERATING PROFIT (LOSS) 3Q 2023	US\$1.9 million	
NET PROFIT (LOSS) 3Q 2022	US\$9.1 million		NET PROFIT (LOSS) 3Q 2023	US\$3.5 million	



Cash and cash equivalents, together with restricted cash and marketable securities, increased from US\$194.1M at the end of 2Q23 to US\$199.2M at the end of 3Q23

RUCONEST® and Joenja® driving revenue growth





On track for 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 1Q24, marketing authorization in Europe ~2 months later*



File leniolisib with UK's MHRA following IRP route*



Continued operating cost investments to accelerate future growth



Further details on our plans to develop leniolisib in additional indications to be provided in 4Q 2023



Investment and continued focus on in-licensing or acquisitions of mid to late-stage opportunities in rare diseases

*International Recognition Procedure (IRP) available as of Jan 1, 2024. Would use US FDA approval and MHRA would have 110 days from the date the IRP submission is validated to review and issue its decision.



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