Pharming Group N.V.

25th Annual Needham
Virtual Healthcare Conference

April 13, 2026

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

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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 2025 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2025, 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

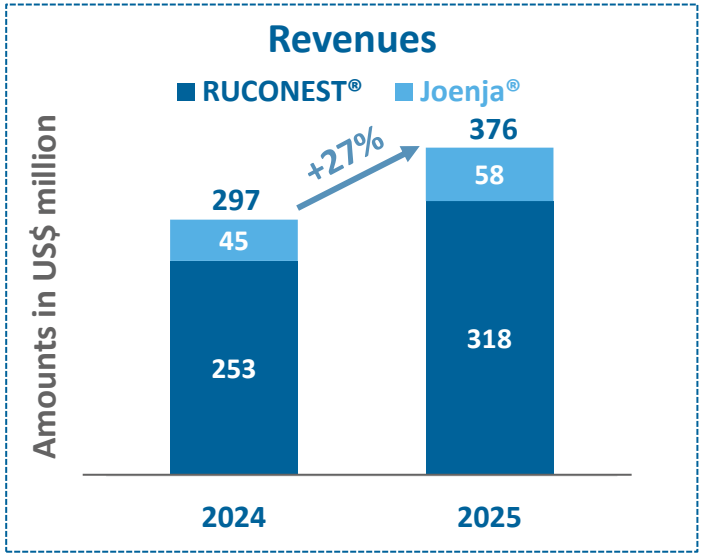
HAE: Hereditary Angioedema, **PIDs:** Primary Immunodeficiencies, **PMD**: Primary Mitochondrial Disease, **CVID:** Common Variable Immunodeficiency

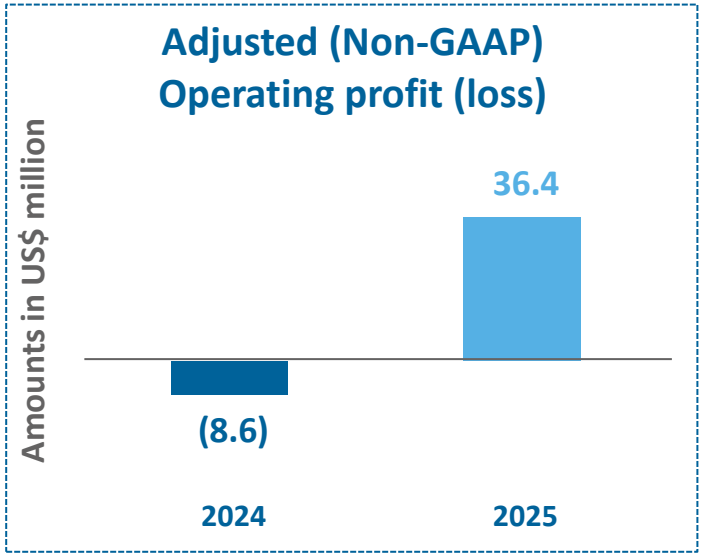
1. Walsh et al., Scalable generation and functional classification of genetic variants in inborn errors of immunity to accelerate clinical diagnosis and treatment, Cell (2025), https://doi.org/10.1016/j.cell.2025.05.037

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 2025 performance
Revenue growth and profitability inflection





- Total revenues up 27% – exceeding guidance
- Continued growth of RUCONEST® and acceleration in Joenja® APDS uptake
- Strong revenue growth and disciplined cost management drove inflection in operating profit and cash flow from operations (+US$55M)
- Added napazimone (KL1333) to expand our late-stage pipeline in rare disease

2026 Outlook – financial, commercial and pipeline progress

Revenue guidance – US$405 million - US$425 million (8% to 13% growth)

Operating expense guidance – US$330 million - US$335 million (6% to 8% growth)

Joenja® APDS – geographic and pediatric label expansion

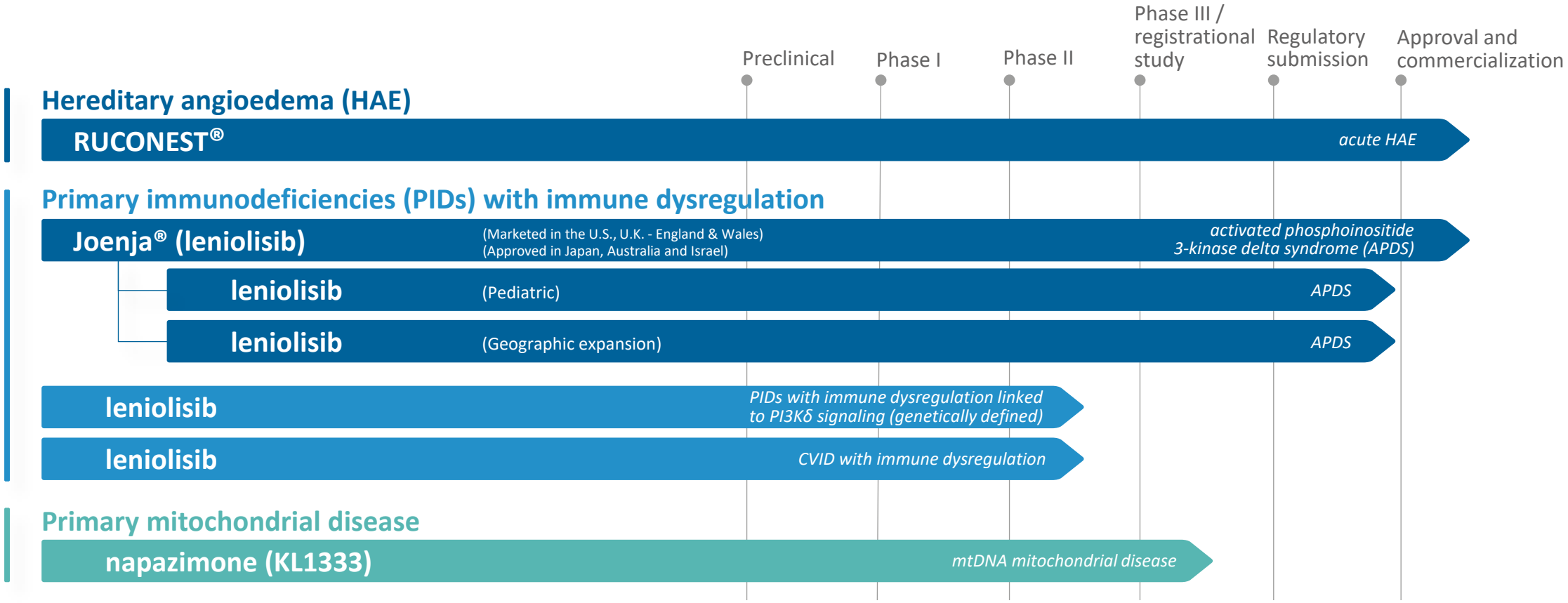
- ✓ Europe (12+) – CHMP positive opinion March 2026, potential EC approval Q2 ‘26
- ✓ Japan (4+) – Approved March 2026
- • FDA Type A meeting held in March to provide clarity on US 4-11 pediatric resubmission

Advancing clinical pipeline for PIDs with immune dysregulation and PMD

- • Leniolisib Phase II clinical trials in PIDs with immune dysregulation – Targeting topline data in H2
- • Napazimone (KL1333) pivotal FALCON clinical study – Targeting enrollment completion in H2 ‘26

Enhancing capital allocation to drive growth and build a leading rare disease company

Diverse rare disease portfolio and pipeline



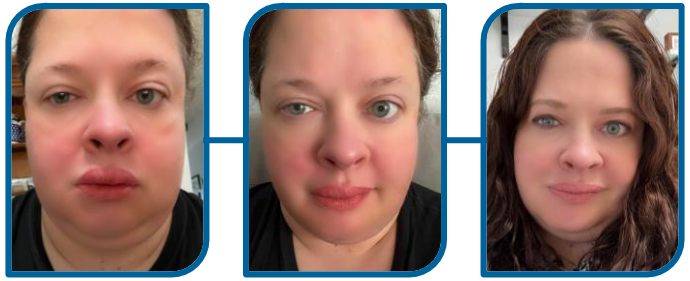
Pharming®

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

- Mostly used by patients experiencing more severe / frequent attacks (Type 1, Type 2, and Normal C1-INH HAE patients)



- 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.

Indirect Treatment Comparison (ITC) of rhC1-INH and sebetralstat for HAE on-demand therapy

Pharming®

Indirect Treatment Comparison (ITC) of Recombinant C1 Inhibitor and Sebetralstat for Hereditary Angioedema (HAE) On-Demand Therapy

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KEY TAKEAWAYS

Complete symptom resolution was 4.5 times more likely with rhC1-INH 50 U/kg than sebetralstat 600 mg

Redosing was 82% lower with rhC1-INH compared with sebetralstat

rhC1-INH showed favorable results across the 10 analyses for complete resolution and 6 analyses for redosing relative to sebetralstat

ABBREVIATIONS

ESS, effective sample size; HAE, hereditary angioedema; HR, hazard ratio; ITC, indirect treatment comparison; LTP, long-term prophylaxis; MAIC, matching-adjusted indirect treatment comparison; NCT, National Clinical Trial; OR, odds ratio; pdC1-INH, plasma-derived C1 esterase inhibitor; PGI-S, Patient Global Impression of Severity; rhC1-INH, recombinant human C1 esterase inhibitor; RMAIC, reduced matching-adjusted indirect treatment comparison; TTCR, time to complete resolution; VAS, visual analog scale.

REFERENCES

1. Busse PJ, et al. *J Allergy Clin Immunol Pract*. 2021;9(1):132-150. 2. Cohn DM, et al. *J Allergy Clin Immunol*. 2025;155(3):726-739. 3. Zuraw B, et al. *J Allergy Clin Immunol*. 2010;126(4):821-827.e14. 4. Riedl MA, et al. *Ann Allergy Asthma Immunol*. 2014;112(2):163-169. 5. Riedl MA, et al. *N Engl J Med*. 2024;391(1):32-43. 6. Li HH, et al. *Allergy Asthma Clin Immunol*. 2025;21(1):10. doi:10.1186/s13223-025-00655-6

DISCLOSURES

JA is a speaker bureau member for CSL Behring, Pharming Healthcare, Inc., BioCryst Pharmaceuticals, Takeda, AstraZeneca, and GSK; has received consulting fees from CSL Behring, Pharming Healthcare, Inc., BioCryst Pharmaceuticals, Pharvaris, Ionis Pharmaceuticals, Takeda, and Novartis AG; and is a clinical trial investigator for BioCryst Pharmaceuticals, CSL Behring, Pharvaris, KalVista Pharmaceuticals, Astria Therapeutics, Takeda, Novartis AG, and Cellidex Therapeutics. DJ is a consultant for Pharming Healthcare, Inc., KalVista Pharmaceuticals, Takeda, CSL Behring, BioCryst Pharmaceuticals, and Pharvaris. AS reports financial relationships with AstraZeneca, BioCryst, Optinose, Pharming Group, and Takeda. HHL has received research or consulting support from or provided speaker presentations for CSL Behring, BioCryst Pharmaceuticals, Pharvaris, Argo Biopharma, KalVista Pharmaceuticals, Takeda, Intellia Therapeutics, Pharming Healthcare, Inc., and Ionis Pharmaceuticals. EA, NR, and HK are employed by Lumanity, which received consulting fees from Pharming Healthcare, Inc. NN, AR, and AH are employees of Pharming Healthcare, Inc.

American College of Allergy, Asthma & Immunology (ACAAI) 2025 Annual Scientific Meeting; November 6-10, 2025; Orlando, FL

INTRODUCTION

- HAE is a rare disorder associated with recurrent, unpredictable, and debilitating swelling attacks requiring access to effective on-demand therapies[1]
- Five on-demand therapies for HAE have been approved globally: pdC1-INH, ecallantide, icatibant, rhC1-INH, and sebetralstat[2]
- rhC1-INH, a recombinant human C1 inhibitor, has been studied in several phase 2/3 and phase 3 trials, including C1 1205 (NCT00225147), C1 1304 (NCT00262301), and C1 1310 (NCT01188564)[3,4]
- Sebetralstat, a plasma kallikrein inhibitor, was studied in the phase 3 KONFIDENT trial (NCT05259917)[5]
- With a lack of head-to-head comparisons between on-demand HAE therapies, an ITC, using population-adjustment methods, can be used to compare the clinical outcomes of these on-demand treatments[6]

OBJECTIVE

- This ITC follows a rigorous approach to compare the efficacy of rhC1-INH and sebetralstat by adjusting for differences in patient and attack characteristics between populations, and using rederivation methods to align endpoints

METHODS

- A systematic literature review of on-demand HAE therapies informed this ITC of sebetralstat 600 mg (NCT05259917), with pooled data for rhC1-INH 50 U/kg from 3 trials (NCT01188564; NCT00225147; NCT00262301 [placebo group only]) (**Table S1** and **Fig S1**)[3-5]
- To improve comparability, the following steps were taken:
  1. Mapped VAS measures to PGI-S using an algorithm informed by patient and clinician input
  2. Rederived rhC1-INH trial data to align the trial endpoint of TTCR to use a PGI-S definition of "none" within 24 hours
  3. Matched and adjusted for key study, patient, and attack differences (ie, attack severity, time to treatment, prophylaxis use, and attack location)
- The relative effects for TTCR and redosing outcomes were assessed using unanchored and anchored MAICs (**Fig 1**). The number of matching variables were varied (**Fig 2**)
- Subgroup analyses accounted for matching key variables excluded from RMAIC, such as PGI-S severity and time to treatment. Results were reported as HRs for TTCR and ORs for redosing with associated with 95% CIs

Figure 1 Network Diagram for Unanchored and Anchored ITC Analyses

RESULTS

- Demographic characteristics are reported in **Table 1 and Table S2**
- Attack severity differed, with 100% of patients treated with rhC1-INH experiencing severe or very severe attacks vs 17.5% in the sebetralstat study population. Common attack locations were peripheral (41.7%-82.4%) and abdominal (35.0%-45.2%) across treatment groups
- Treatment timing varied due to differing study designs[3-5]:
  - rhC1-INH trials required clinic visits, delaying treatment (median, 242-267 minutes)
  - Sebetralstat trials allowed self-administration, enabling faster treatment (median, 41-51 minutes)

RESULTS (cont)

Table 1 Baseline Demographics for KONFIDENT and Pooled rhC1-INH Trials

	Sebetralstat population: KONFIDENT		Pooled overall rhC1-INH population	
	Sebetralstat 600 mg (n=83)	Placebo (n=84)	C1 1310, C1 1205 rhC1-INH 50 U/kg (n=45[a])	C1 1205, C1 1304, C1 1310 Placebo (n=69)
Age, median (IQR), y	39 (25-49)	38 (25-49)	39 (31-47)	39 (29-53)
Male, n (%)	37 (39.8)	29 (34.5)	15 (31.2)	20 (33.3)
White, n (%)	80 (86.0)	73 (86.9)	46 (95.8)	57 (95.0)
Treatment approach, n (%)				
On-demand only	72 (77.4)	66 (78.6)	22 (45.8)	21 (35.0)
On-demand + LTP	21 (22.6)	18 (21.4)	26 (54.2)	39 (65.0)
Baseline severity on PGIS, n (%)				
None	0	2 (2.4)	0	0
Mild	41 (44.1)	36 (42.9)	0	0
Moderate	34 (36.6)	33 (39.3)	0	0
Severe	16 (17.2)	10 (11.9)	11 (22.9)	7 (11.7)
Very severe	2 (2.2)	3 (3.6)	37 (77.1)	53 (88.3)
Attack location, n (%)				
Abdominal	42 (45.2)	37 (44.0)	17 (35.4)	21 (35.0)
Peripheral	58 (62.4)	46 (54.8)	23 (47.9)	25 (41.7)
Head and neck[b]	13 (14.0)	13 (15.5)	8 (16.7)	14 (23.3)
Pooled attack location, n (%)				
Mucosal	44 (47.3)	40 (47.6)	17 (35.4)	21 (35.0)
Subcutaneous	49 (52.7)	44 (52.4)	31 (64.6)	39 (65.0)
Time from attack onset to first administration, median (IQR), min	41 (5-142)	51 (6-166)	242 (186-286)	257 (211-316)

[a]One patient assigned to rhC1-INH did not receive treatment and did not have baseline characteristics collected; percentages calculated for this group using data from 48 patients.
[b]Head, face, neck, larynx, and throat (alone or in combination) for KONFIDENT. Facial oropharyngeal-laryngeal (1310), orofacial pharyngeal-laryngeal (1304), and orofacial laryngeal (1205) for the pooled rhC1-INH population.[3,5]

Figure 2 Unanchored and Anchored Results for TTCR in Patients With HAE Receiving rhC1-INH 50 U/kg[a] or Sebetralstat 600 mg

Analysis	Matching variables	ESS for rhC1-INH	Original sample size	Relative effect HR (95% CI)[b]
Unanchored: overall				
MAIC	PGI-S severity, prophylaxis use, attack location	5.7	48	7.98 (1.60-98.26)*
RMAIC	Prophylaxis use, attack location	33.2	48	4.62 (2.84-7.18)*
Bucher ITC	Unadjusted	48	48	3.64 (2.36-5.62)*
Anchored: overall				
MAIC	PGI-S severity, prophylaxis use, attack location	5.7	108	11.28 (1.69-76.36)*
RMAIC	Prophylaxis use, attack location	67.1	108	1.41 (0.53-3.18)
Bucher ITC	Unadjusted	108	108	1.47 (0.73-2.99)

*P value <0.05.
[a]rhC1-INH 50 U/kg for patients <84 kg or <200 U otherwise.
[b]HRs with 95% CIs were reported from weighted Cox model.

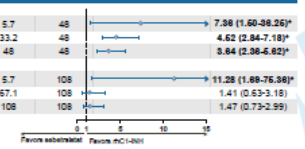


Figure 4 Subgroup Analysis of Severe/Very Severe and Early Treatment: Unanchored Results for TTCR in Patients With HAE Receiving rhC1-INH 50 U/kg[a] or Sebetralstat 600 mg

Analysis	Matching variables	ESS for rhC1-INH	Original sample size	Relative effect HR (95% CI)[b]
Unanchored: severe/very severe attacks				
RMAIC	Prophylaxis use, attack location	33.2	48	6.39 (2.81-11.12)*
Bucher ITC	Unadjusted	48	48	4.29 (2.10-8.77)*
Unanchored: early treatment group[c]				
RMAIC	Prophylaxis use, attack location	19	26	6.63 (3.22-9.60)*
Bucher ITC	Unadjusted	26	26	4.09 (2.44-6.86)*

*P value <0.05.
[a]rhC1-INH 50 U/kg for patients <84 kg or <200 U otherwise.
[b]HRs with 95% CIs were reported from weighted Cox model.
[c]Included patients treated within the median time to treatment of 242 minutes for rhC1-INH and 267 minutes for placebo.

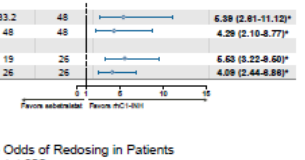
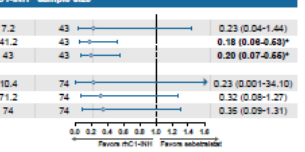


Figure 5 Unanchored and Anchored Results for the Odds of Redosing in Patients With HAE Receiving rhC1-INH 50 U/kg[a] or Sebetralstat 600 mg

Analysis	Matching variables	ESS for rhC1-INH	Original sample size	OR (95% CI)[b]
Unanchored				
MAIC	PGI-S severity, attack location	7.2	43	0.23 (0.04-1.44)
RMAIC	Attack location	41.2	43	0.18 (0.08-0.63)*
Bucher ITC	Unadjusted	43	43	0.20 (0.07-0.66)*
Anchored				
MAIC	PGI-S severity, attack location	10.4	74	0.23 (0.001-34.10)
RMAIC	Attack location	71.2	74	0.32 (0.08-1.27)
Bucher ITC	Unadjusted	74	74	0.35 (0.09-1.31)

*P value <0.05.
[a]rhC1-INH 50 U/kg for patients <84 kg or <200 U otherwise.
[b]ORs were reported from weighted logistic regression model.



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CONCLUSIONS

- Robust estimates show rhC1-INH is 4.5 times more likely to resolve an HAE attack than sebetralstat
- Redosing was significantly lower with rhC1-INH, with an 82% reduction compared with sebetralstat, indicating a sustained efficacy response
- Protocol differences in time to treatment, severity of attacks, and placebo administration were accounted for, and results remained clinically meaningful[3-5]

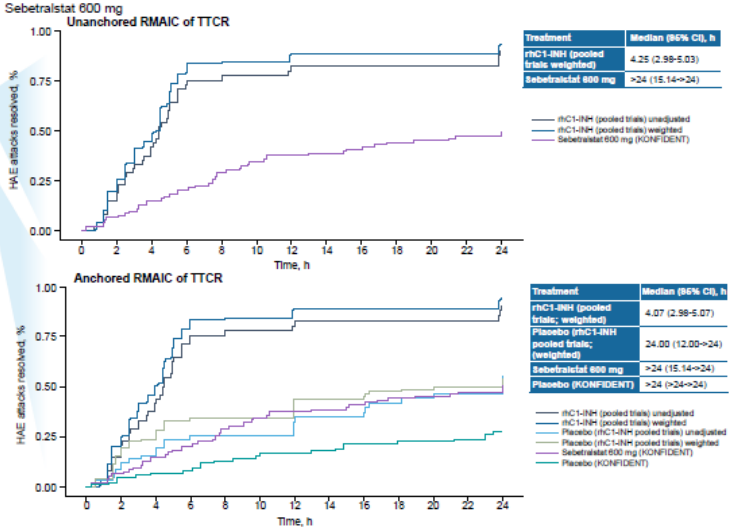
Overall Population TTCR Analyses (Figs 2-3, Table S3)

- Anchored analyses were limited due to differences in the redosing protocols of the placebo groups, evidenced by the overlap of the rhC1-INH placebo arm with the sebetralstat 600 mg active treatment arm (**Fig 3**)
- The RMAIC reduced uncertainty in the model by increasing the ESS, resulting in more narrow CIs
- Therefore, the most robust estimate for this comparison was determined to be the unanchored RMAIC, with rhC1-INH reaching complete resolution 4.5 times more likely than sebetralstat

Subgroup TTCR Analyses

- Subgroup analyses showed a greater difference between rhC1-INH and sebetralstat than in the overall population
- All subgroup findings were statistically significant and aligned with the overall population results (**Fig 4, Table S4**)

Figure 3 Unanchored and Anchored Results for TTCR in Patients With HAE Receiving rhC1-INH 50 U/kg or Sebetralstat 600 mg



Redosing

- All 6 unanchored and anchored analyses showed numerically favorable results for rhC1-INH compared with sebetralstat, with the unanchored Bucher ITCs and RMAICs demonstrating statistical significance (**Fig 5**)
- The unanchored RMAIC, the most robust analysis for this comparison, showed rhC1-INH had 0.18 times the odds of redosing compared with sebetralstat, indicating an 82% reduction in redosing with rhC1-INH

rhC1-INH demonstrated significantly shorter time to complete resolution compared to sebetralstat

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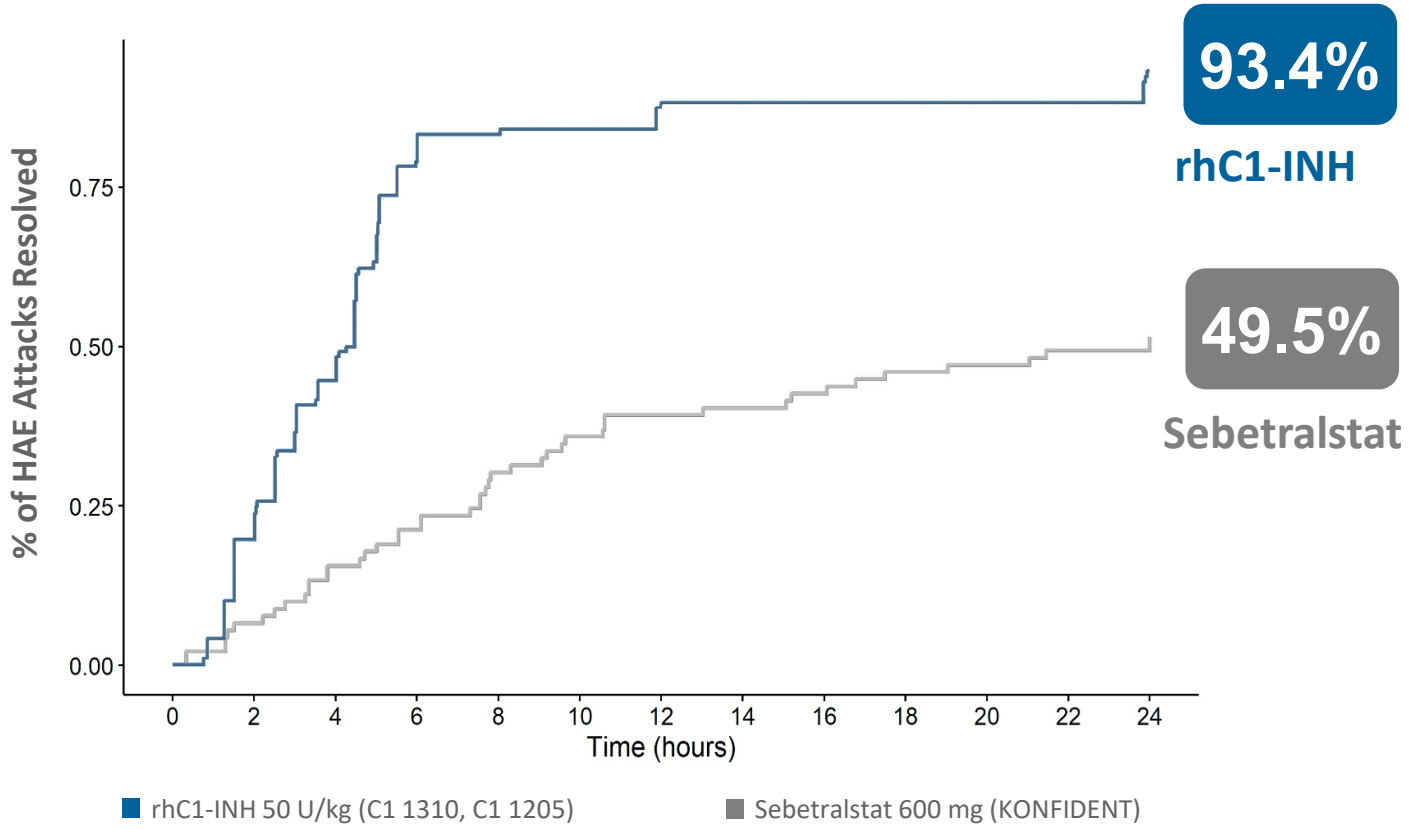
Time to Complete Resolution of rhC1-INH vs Sebetralstat



93.4%

rhC1-INH

49.5%

Sebetralstat

Patients on rhC1-INH were

4.5x more likely

to achieve complete symptom resolution than sebetralstat*

Reference: Anderson J, *et al.* Indirect treatment comparison (ITC) of recombinant C1 inhibitor and sebetralstat for hereditary angioedema on-demand therapy. *Ann Allergy Asthma Immunol.* 2025;135(5 Suppl 1):S29.ǂ

*HR = 4.5 (95% CI: 2.8 – 7.2; *P-value < 0.05*); Derived from an MAIC by taking the ratio of slopes of rhC1-INH and Sebetralstat treatment curves adjusting for attack location and prophylaxis use based on a 24-hour observation period. HR for subgroup, sensitivity, and scenario analysis were consistent and ranged from 3.64 to 7.36

ǂStudy limitations listed in slide footnote and may be provided upon request

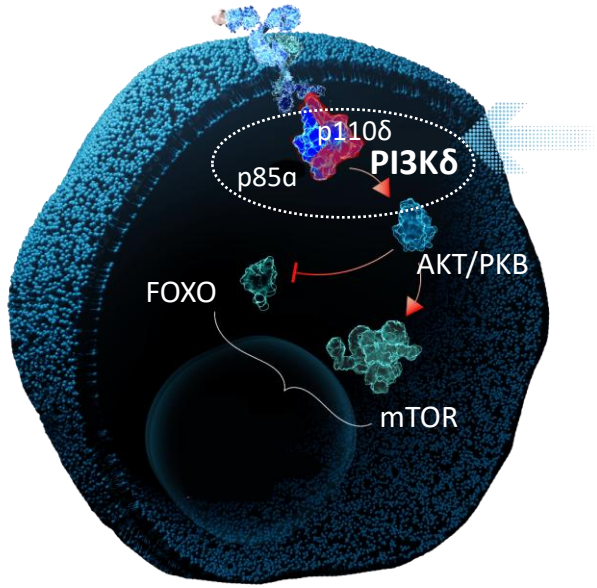
CI, Confidence Interval; **HR**, Hazard Ratio; **MAIC**, Matched Adjusted Indirect Treatment Comparison

Pharming®

Joenja® (leniolisib)
APDS & PID indications

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

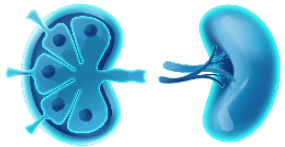
PI3Kδ is a master regulator of the immune system and influences



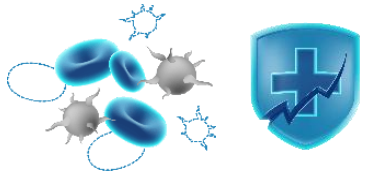
↑Cell trafficking ↑Cell Growth ↑Cell proliferation
↑Cell Differentiation ↑Apoptosis inhibition/survival

Immune dysregulation pathology

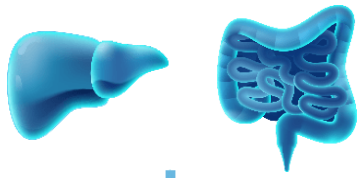
Lymphoproliferation



Autoimmunity



GI Disease

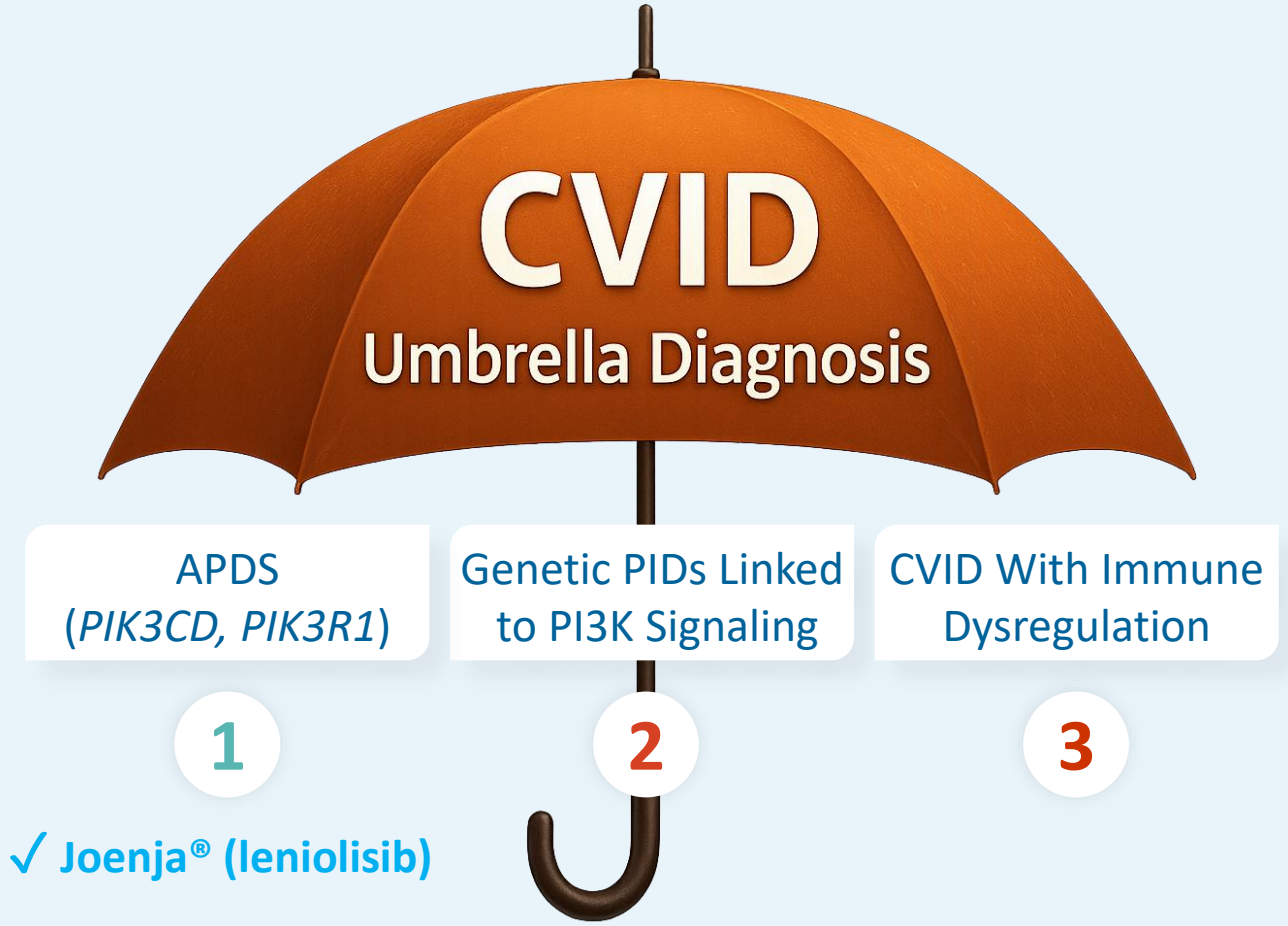


Pulmonary Disease

Shared pathology under the influence of PI3Kδ

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

PIDs with immune dysregulation under the influence of PI3Kδ



Reports of Joenja® changing patients' lives

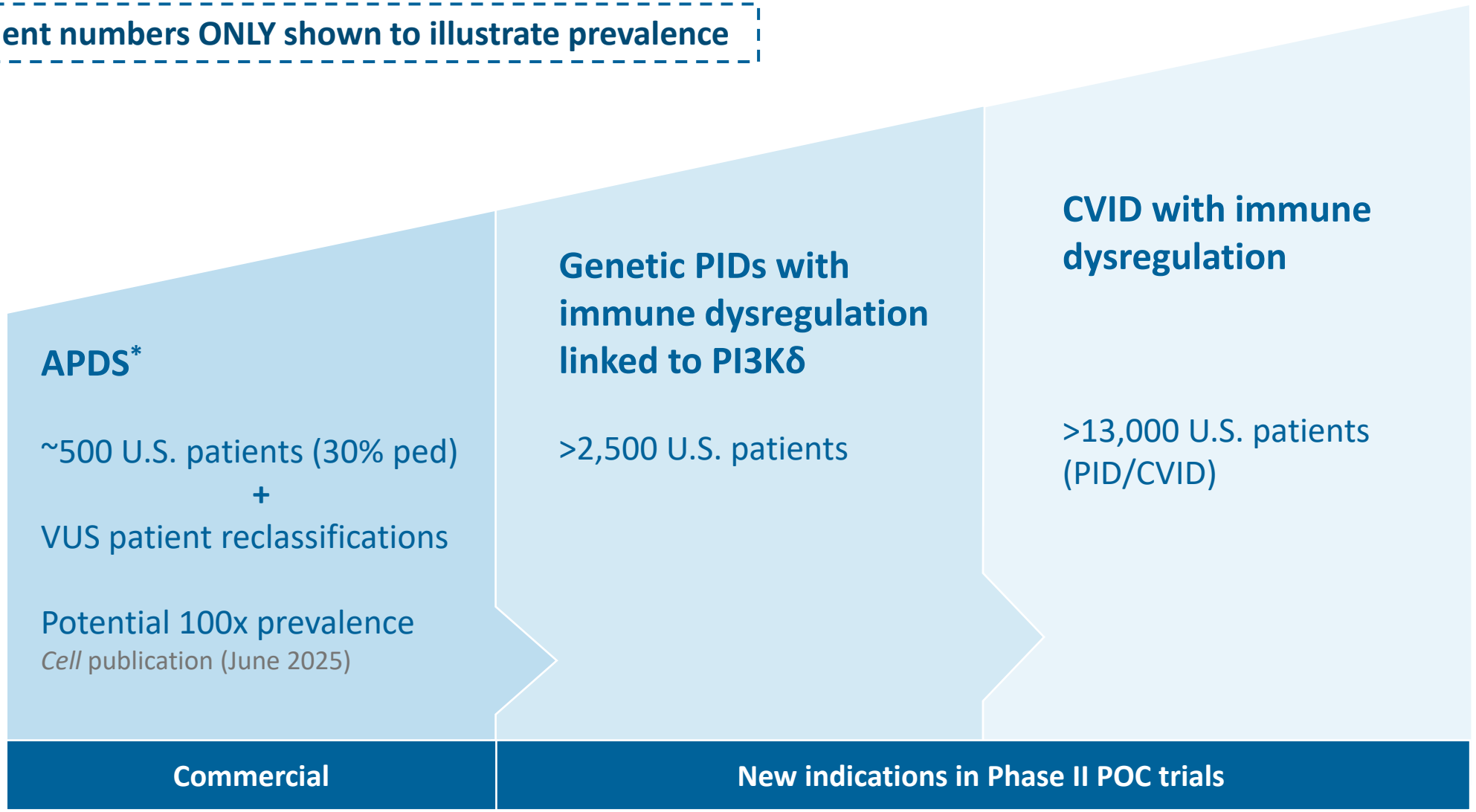
24-year-old male with APDS whose progress was followed in the Joenja® open-label extension study for 6 years

	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



*APDS prevalence est. ~1.5 patients / million. 274 patients identified in the U.S. (181 eligible 12+, 72 eligible pediatric), 998 identified globally (as of December 31, 2025).

Joenja® (leniolisib) – near-term APDS regulatory milestones

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U.S.

Pediatric label expansion – sNDA for patients aged 4-11

- FDA Type A meeting held March 26, 2026, awaiting meeting minutes

Europe

MAA for patients aged 12+

- ✓ CHMP (EMA) positive opinion March 2026
- Potential EC approval Q2 ‘26

Japan

NDA for patients aged 4+

- ✓ Approved March 2026, first global approval including children aged 4-11
- Commercial launch following agreement on National Health Insurance drug price

Canada

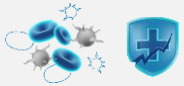
Regulatory submission for patients aged 12+

- Submitted response to Health Canada with additional CMC data in January
- Expect mid-2026 regulatory decision

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

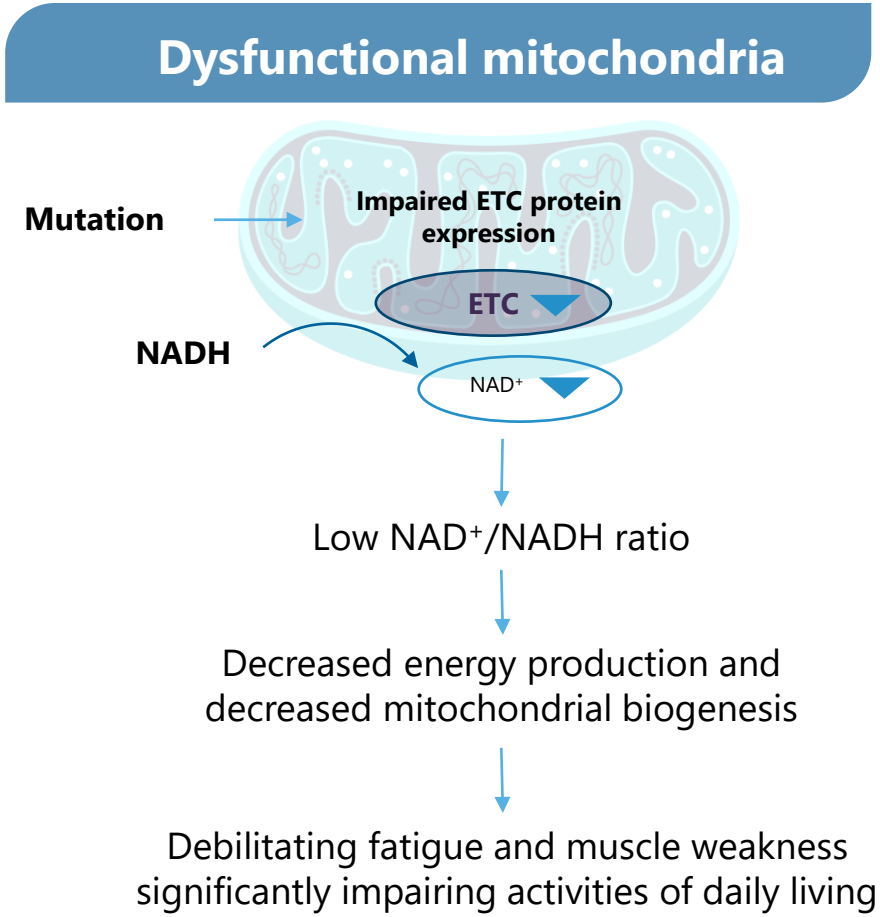
Napazimone (KL1333) for mtDNA-driven primary mitochondrial disease
Aiming for the first disease-modifying treatment

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
- >30,000 diagnosed patients with mtDNA disorders[1]

Registrational clinical study underway

- Clinically-relevant Fatigue, Sit-to-Stand endpoints supported by FDA
- Positive interim analysis – both endpoints cleared futility
- Over 25 sites actively recruiting
- On track to complete enrollment in 2026 and for trial readout late 2027

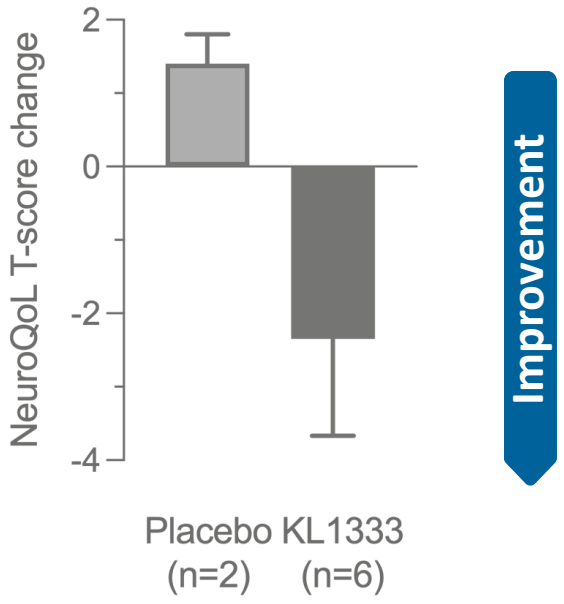


1. In US, EU4 and UK. Diagnoses can include MELAS-MIDD and KSS-CPEO spectrum disorders as well as MERRF syndrome.

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

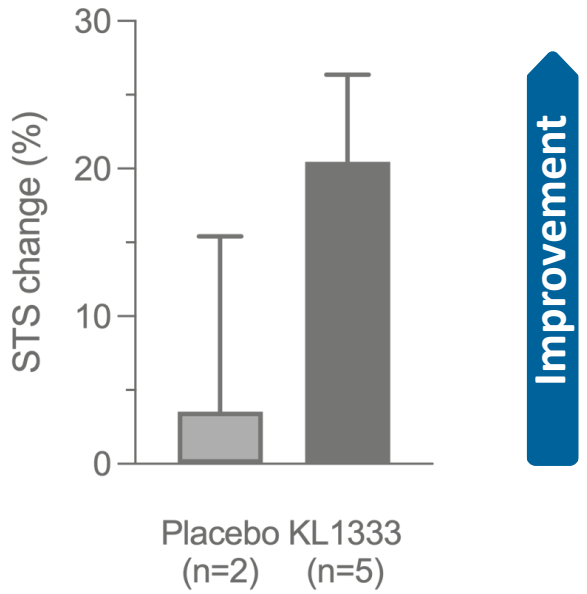
Reduction of fatigue

Changes from baseline to day 10[1]



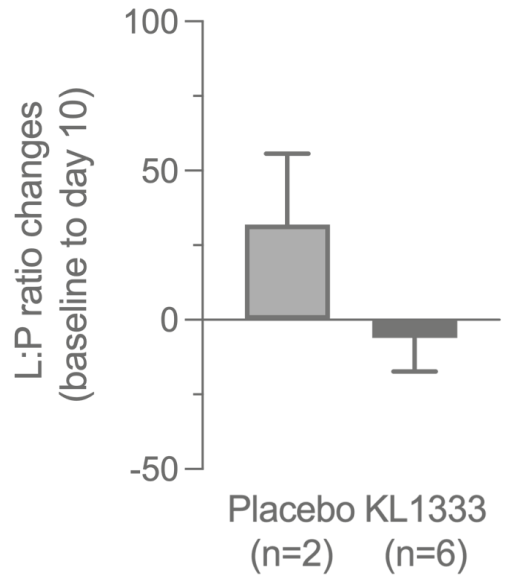
Improvement in muscle function

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

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
- Over 25 active sites
- 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

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Financials and Outlook

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, with quarterly fluctuations
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)

- Significant and accelerating Joenja® growth, and continued RUCONEST® 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 RUCONEST® and Joenja® growth
- Shift to operating profit and positive cash flow
- 2026 revenue guidance $405-425M (+8-13%) – continued RUCONEST® growth and accelerating Joenja® growth

Strategic growth priorities

- Sustained growth of commercial portfolio
- Significant Joenja® APDS growth catalysts
- 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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