Strategic Business Review

Pharming in focus

Pharming Group is a global biotechnology company that develops and commercializes innovative therapies for rare and ultra-rare diseases with significant unmet need. We focus on immunological and genetic conditions where our scientific and commercial expertise can help advance care over the long term.

Patients are at the heart of everything we do. Their insights along with those of caregivers and the scientific community shape our strategy, guide our clinical study designs, and influence how we manage our approved therapies.

Our teams combine deep scientific, medical, and operational expertise in rare disease drug development and commercialization. We leverage proven and efficient clinical development, supply chain, and commercial infrastructure to advance and expand our portfolio and pipeline and increase access for patient communities that currently lack adequate treatment options.

We execute with discipline and urgency, pursuing strategic growth with focused resource allocation. By strengthening our commercial portfolio and advancing high-value pipeline programs, we aim to deliver sustainable long-term value for patients, healthcare providers, employees, partners, and shareholders.

Founded in
1988

Headquarters: Leiden, the Netherlands
U.S. Headquarters: Warren, New Jersey

407
employees globally

Dual listed:
Euronext Amsterdam (PHARM)
Nasdaq (PHAR)

Scalable infrastructure

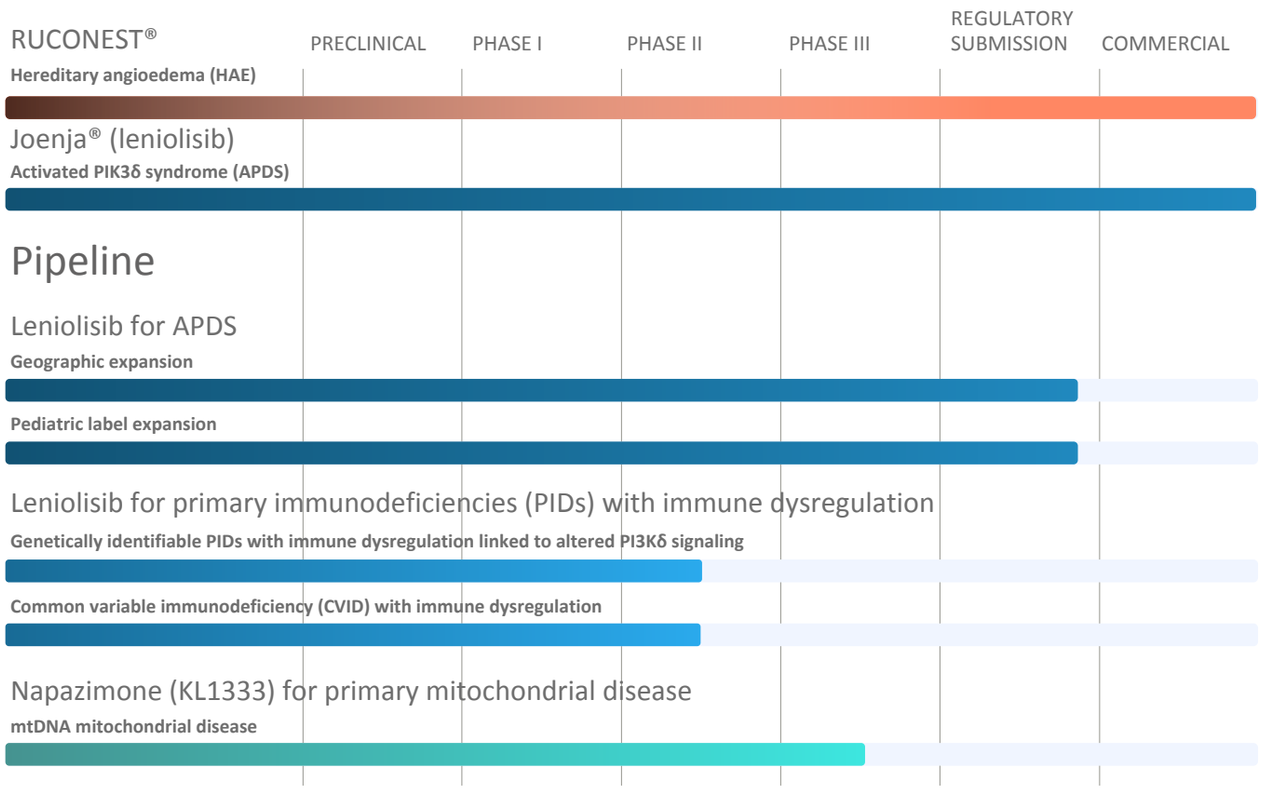
driving rare disease innovation, commercial excellence, and patient-focused value creation

High-growth commercial portfolio

High-value pipeline

Business and strategic direction

Commercial



Pipeline

Our vision is to 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

Our business

Pharming is an integrated biotechnology company with proven capabilities across clinical development, manufacturing, regulatory affairs, and commercialization. Our model supports us in efficiently developing and delivering innovative therapies to expand treatment options and improve outcomes for rare disease communities with significant unmet needs.

Through disciplined execution across our commercial portfolio and pipeline, we aim to deliver sustainable growth while continuing to invest in innovation and long-term value creation for patients, healthcare providers and shareholders.

Commercial

We currently commercialize two approved therapies for distinct rare and ultra-rare disease areas.

RUCONEST®, our first approved product, is the first and only recombinant C1 esterase inhibitor (rhC1-INH) protein replacement therapy indicated for the treatment of acute hereditary angioedema (HAE) attacks in adult and adolescent patients. It has received regulatory approval in the European Economic Area (2010), the United Kingdom (2010) and the United States (2014).

Our commercial focus for RUCONEST® is the United States. In other approved regions, we continue to support patients where appropriate.

Joenja® (leniolisib), our second marketed therapy, is an oral, selective phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved in the United States, United Kingdom, Australia, and Israel, as the first and only targeted treatment indicated for activated phosphoinositide 3-kinase delta syndrome (APDS), an ultra-rare primary immunodeficiency (PID), in adult and pediatric patients 12 years of age and older, and in Japan for adult and pediatric patients aged 4 years and older.

We currently commercialize Joenja® in the United States and the United Kingdom through our own sales and marketing infrastructure.

Pipeline

Pharming is advancing a focused pipeline centered on rare immunological and genetic diseases.

Leniolisib for APDS

To expand access and generate evidence for broader use, we have active regulatory and clinical development efforts aimed at making leniolisib available to APDS patients across all age groups in key markets. In addition, we are working to further characterize the disease-causing impact of mutations in the APDS causing genes, currently classified as variants of uncertain significance (VUSs), and are undertaking further research to determine the overall prevalence of APDS.

Leniolisib for PIDs with immune dysregulation

Beyond APDS, leniolisib is being evaluated in two Phase II studies targeting additional PIDs with immune dysregulation:

- **Genetically confirmed PIDs** linked to altered PI3Kδ signaling
- **Common variable immunodeficiency (CVID) with immune dysregulation**, regardless of underlying genetic confirmation

These programs provide the opportunity to address significantly larger patient populations that currently lack targeted therapeutic options.

Napazimone (KL1333) for primary mitochondrial disease

In 2025, our late-stage pipeline was strengthened through the acquisition of Abliva AB and its lead asset, KL1333. Napazimone (KL1333), the newly named compound, is a potential first-in-disease therapy for mitochondrial DNA (mtDNA)-driven primary mitochondrial disease (PMD). It is currently in a pivotal clinical trial, designed to support regulatory approval, which passed a futility analysis on its two primary end points.

Our commitment to rare diseases

Pharming is committed to advancing care for people living with rare and ultra-rare diseases. We focus on areas of significant unmet need where our scientific and commercial expertise may make a meaningful difference - and where specialist care, diagnostic readiness, and reimbursement pathways enable responsible patient identification and sustainable access.

We align our clinical development, business development, and capital allocation decisions around rare diseases where we can build competitive advantage and create durable growth - strengthening our potential to deliver long-term value while expanding access for patients who often have limited or no treatment options.

Hereditary angioedema (HAE)

HAE is a rare, potentially life-threatening genetic condition characterized by unpredictable swelling attacks that may affect the skin, gastrointestinal tract, and upper airway (laryngeal edema). Global prevalence is estimated at ~1 in 50,000 to ~1 in 10,000. Symptoms are driven by excess bradykinin resulting from uncontrolled kallikrein activity, which leads to fluid leakage into tissues. Despite broader use of prophylactic therapies, breakthrough attacks remain common, and clinical guidelines recommend ready access to effective on-demand treatment for all patients, including those on prophylaxis.

Pharming's market emphasis

We are focused on the U.S. HAE market as the core commercial opportunity for acute treatment.

Market size & growth (U.S.)

The U.S. HAE market continues to expand, driven by improved disease awareness, broader specialist access, and new therapeutic options across both prophylactic and on-demand classes. Recent real-world U.S. claims analyses indicated that the diagnosed patient population may be larger than historically assumed at 2.67 per 100,000. This larger-than-expected patient base reinforces the need for robust prophylaxis and reliable on-demand options.[1]

By the end of 2024, the U.S. market included multiple FDA-approved options across both acute treatment and long-term prophylaxis. In 2025, the therapeutic landscape broadened further with FDA approvals introducing new mechanisms and modalities, including:

- an activated Factor XII (FXIIa) inhibitor for prophylaxis;
- a prekallikrein-directed antisense oligonucleotide for prophylaxis; and
- an oral plasma kallikrein inhibitor for on-demand treatment of acute attacks.

Collectively, these developments are expanding therapeutic choice and increasing the complexity of treatment decision-making across both prophylactic and acute settings.

Competitive dynamics (acute & prophylaxis)

The U.S. HAE market spans therapies that target C1 esterase inhibitor (C1-INH) replacement and key components of the contact activation system, including plasma kallikrein and FXIIa, delivered via intravenous, subcutaneous, and oral modalities. Long-term prophylaxis has become the dominant treatment paradigm. In a U.S. patient survey, ~68.5% reported having received or currently receiving long-term prophylaxis (LTP).[2] However, increased prophylaxis uptake has not eliminated the need for acute therapy.

The U.S. HAEA Medical Advisory Board guidelines emphasize that patients must have ready access to effective on-demand medication, and that all patients with laboratory-confirmed HAE should have access to at least two standard doses of an FDA-approved on-demand therapy.[3]

Breakthrough attacks remain clinically meaningful and common despite the widespread adoption of LTP. Randomized studies indicate that approximately 50% of patients receiving prophylaxis continue to experience breakthrough attacks[4], and many patients treated with prophylaxis also report attacks in controlled settings. In addition, certain acute therapies may require re-dosing, highlighting persistent unmet needs in acute management and reinforcing the need for therapies that restore functional C1-INH activity.

Role of RUCONEST® (recombinant C1-INH, IV)
RUCONEST® is the first and only recombinant C1-INH protein replacement therapy, providing a differentiated on-demand treatment for acute attacks in patients with Type I, Type II, and normal C1-INH HAE.

By restoring functional C1-INH, RUCONEST® addresses the underlying deficiency that triggers attacks and regulates the contact activation system through inhibition of Factor XII and kallikrein, reducing downstream bradykinin and related mediators. This mechanism supports its positioning as targeting the root cause of HAE across multiple biological pathways rather than a single cascade.

Its intravenous administration provides immediate, complete bioavailability, enabling rapid, high-dose intervention to halt attack progression. In a market increasingly shaped by prophylaxis, RUCONEST® continues to serve as a cornerstone on-demand treatment, particularly for patients experiencing more severe or frequent attacks or those who continue to experience breakthrough attacks despite prophylactic treatment.

Primary Immunodeficiencies (PIDs)

PIDs are a heterogeneous group of rare immune disorders that lead to recurrent infections and immune dysregulation.

The global PID therapeutics market is estimated at ~US$8 billion in 2025, with steady growth expected at a compound annual growth rate (CAGR) of ~6% to ~US$14 billion by 2034[5], supported by earlier genetic diagnosis, better care pathways, and sustained demand for immunoglobulin and targeted therapies.

Underdiagnosis remains significant, and expanded testing continues to increase the identifiable patient population.

APDS
APDS (activated PI3Kδ syndrome) is an ultra-rare, progressive PID first characterized in 2013[6]. Literature estimates prevalence at approximately 1.5 patients per million[6,7]. Emerging evidence suggests that the prevalence may be much higher, underscoring the importance of efforts to further characterize the disease, and understand the impact of mutations in the APDS causing genes currently classified as VUSs.

APDS presents with a clinically heterogeneous profile that may include severe and recurrent sinopulmonary infections; persistent or recurrent herpesvirus infections (notably EBV and CMV); lymphadenopathy, hepatosplenomegaly, and nodular lymphoid hyperplasia; autoimmune cytopenias; enteropathy; and bronchiectasis. Patients also face a heightened risk of malignancy, particularly lymphoma, due to dysregulated lymphoproliferation.

Delayed diagnosis can lead to the accumulation of irreversible organ damage, and published analyses indicate that survival probability may be up to 28% lower than the general population, with lymphoma and infections representing the leading causes of mortality. Despite increased awareness since its identification, APDS continues to be under-recognized and misdiagnosed, particularly outside specialist care settings.

There are currently over 1,800 known U.S. patients with a VUS in the *PIK3CD* and *PIK3R1* genes implicated in APDS. In 2025, new peer-reviewed research published in leading journal *Cell* demonstrated that functional characterization of PI3Kδ pathway variants can support the reclassification of certain VUSs to APDS.

We expect to provide an estimate of how many of these patients may be diagnosed with APDS following completion of new experiments planned to generate the data needed for genetic testing laboratories to evaluate VUSs identified in patients who have undergone genetic testing for APDS or other immunodeficiencies.

A second conclusion of this research was that APDS may have a broader clinical presentation and significantly higher prevalence than previously assumed, an important consideration for long-term market growth. Further research is on-going on this topic.

Market size & growth
The APDS market remains at an early stage, reflecting the ultra-rare nature of the disease, limited historical diagnostic pathways, and the absence, until recently, of targeted therapeutic options.

However, multiple indicators point to durable market expansion:
- New functional genomics evidence indicates that APDS may be more prevalent than previously understood as increased PI3Kδ pathway activity was identified in variants of the APDS causing genes that have not so far been reported in APDS patients[8] prompting further investigation into the true size of the APDS population.
- Expanded genetic testing and reclassification of VUSs are expected to increase the identifiable patient population.
- Growing understanding of disease burden, including risk of lymphoma and early mortality, is raising clinical urgency and supporting early targeted therapy adoption.[9,10,11,12,13]
- APDS remains the first genetically defined PID with a precision therapy, anchoring a category with long-term growth potential.

Collectively, these trends position APDS as a small but structurally expanding rare-disease market with significant diagnostic uplift ahead.

Competition
Joenja® is currently the only approved therapy for APDS and selective PI3Kδ inhibition remains the defining approach within this therapeutic category. Based on public information, we are not aware of any active clinical development programs in APDS.

PIDs with immune dysregulation beyond APDS

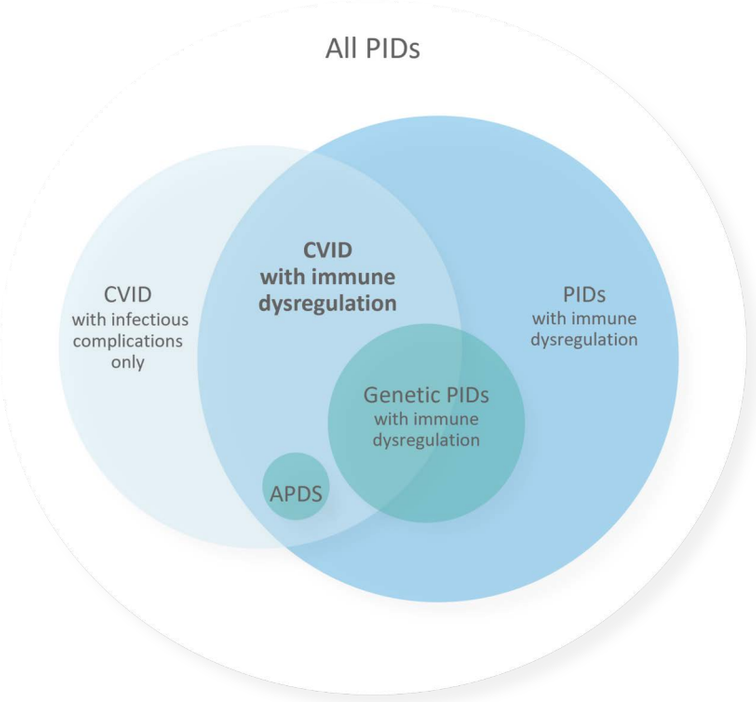
Primary immunodeficiencies (PIDs) with immune dysregulation represent an area of significant unmet medical need, commonly presenting with lymphoproliferation, autoimmune manifestations, and organ-specific inflammation.

Beyond the rare APDS population, several genetically defined PIDs with immune dysregulation linked to altered PI3Kδ signaling affect an estimated ~7.5 patients per million, offering the potential to reach meaningfully broader patient groups.

CVID with immune dysregulation represents an even larger segment within the PID landscape. As the most common symptomatic PID, approximately half of CVID patients develop inflammatory or autoimmune complications, corresponding to a target population of ~39 per million.

Many exhibit APDS-like clinical and immunologic features, reinforcing the rationale for PI3Kδ pathway modulation and importance to address the substantial unmet need across a wider spectrum of immune dysregulation disorders beyond APDS.

We are conducting Phase II studies in both PIDs with immune dysregulation linked to altered PI3Kδ signaling, and CVID with immune dysregulation. Both studies are fully enrolled and we expect top line data in the second half of 2026.



Not to scale with population sizes

Primary mitochondrial disease (PMD)

Primary mitochondrial diseases are rare, multisystem disorders caused by impaired cellular energy generation, leading to chronic fatigue, muscle weakness, neurological manifestations, and substantial functional limitations. Affecting both children and adults, PMD encompasses a broad and heterogenous spectrum of symptoms reflecting the essential role of mitochondria in cellular metabolism. With no approved therapies that directly address the underlying bioenergetic defect, current care remains largely supportive, underscoring the significant unmet need across this patient population.

Within this broader landscape, mitochondrial DNA (mtDNA)–driven mitochondrial disease represents a genetically defined subgroup with a well-characterized pathophysiology and considerable disease burden in adults. These patients commonly experience severe fatigue and myopathy that impair daily functioning and quality of life, making this population particularly suited to targeted therapeutic approaches.

Across the U.S., EU4 (France, Germany, Italy, Spain), and the U.K., more than 30,000 diagnosed patients fall into this subgroup, highlighting both the scale of unmet need and the opportunity for a therapy that directly improves mitochondrial energy production. Napazimone (KL1333), designed to modulate cellular NAD^+/NADH balance, aims to address this core bioenergetic dysfunction and potentially deliver the first disease-targeted option for mtDNA-driven mitochondrial disease.

"I was raised in a scientific household, yet they could only treat my symptoms... until I was diagnosed with APDS. Getting this diagnosis has shown me how much early recognition and awareness matters because rare diseases like mine are frequently overlooked or missed."

***Liam,** Living with APDS*

Liam living with APDS

Our strategy

Our vision is to 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.

We continue to build on strong foundations, anchored by our established U.S. commercial platform, expanding access to our therapies in key markets, and progressing a high-value pipeline that we expect will power future growth.

We are strengthening and diversifying our portfolio with a goal to deliver long-term, sustainable growth, with anticipated continued growth and durability for RUCONEST®, midterm momentum driven by Joenja® and disciplined advancement of key pipeline assets leniolisib and napazimone (KL1333). We expect our robust infrastructure and deep collaborations across the rare disease ecosystem to support reliable execution and the cash flow required to fund future innovation and expand access in priority geographies.

Our culture is central to how we execute. We are united by a commitment to improving outcomes for people living with rare and life-threatening conditions. We collaborate across disciplines, combine our expertise, and encourage each other to go the extra mile. Our core values 'We put patients at the heart', 'We make it simple', 'We get it done' and 'We act with urgency' guide how we work, strengthen our ability to execute with discipline, and empower our teams to make a meaningful impact on patients and their families.

2025 Strategic progress

In 2025, we advanced our strategic agenda with discipline and focus. We reinforced the resilience and growth potential of our rare disease therapies in the U.S. and prepared for further geographic expansion. We expanded access and progressed regulatory pathways to broaden the reach of Joenja® in priority markets and continued to strengthen patient identification to enable appropriate treatment.

We advanced our innovation ambitions through disciplined late-stage development and targeted investments in programs aligned to our scientific expertise, while deepening collaboration with clinical experts and rare disease communities. Across the business, we sharpened capital allocation and enhanced cross-functional capabilities to support future portfolio growth and scalable execution. These actions strengthened our foundations for long term, sustainable growth and set clear momentum into 2026.

Resources

Insights from patients, healthcare experts and partners

Proprietary assets
Diverse talent
Financial resources

Scalable, high-performing organization

Operational, Commercial excellence

Differentiated commercial assets

High-value, de-risked pipeline

Strong financial discipline, sustainable growth

Value for patients & other stakeholders

Our competitive advantage

Our competitive advantage is built on a focused rare disease strategy, a patient-focused commercial platform, and a high-value pipeline addressing significant unmet needs. We combine deep scientific expertise with disciplined execution to identify, develop, and commercialize therapies in areas where biology is well understood and patient needs remain substantial.

Our commercial portfolio supports both resilience and growth momentum. RUCONEST® continues to play an important role in the acute HAE market, supported by its differentiated profile and established position in U.S. care.

Joenja® is the first and only approved targeted therapy for APDS and represents a clinically validated proof-of-concept for PI3Kδ inhibition in immune dysregulation. With ongoing development in PIDs with immune dysregulation beyond APDS, Joenja® has the potential to address a substantially larger patient population with limited treatment options.

Looking ahead, our late-stage pipeline further strengthens our competitive positioning. Napazimone (KL1333) is being developed for mitochondrial DNA-driven primary mitochondrial disease, an area of significant unmet medical need with no approved disease-modifying therapies. If successful, napazimone (KL1333) has the potential to become the first treatment targeting the underlying biology of this condition.

These commercial assets and pipeline programs are supported by our rare disease expertise, scalable infrastructure, and the strength of our balance sheet and operating cash flows. Together, we expect these strengths to create a solid foundation for growth and long-term impact for people living with rare diseases.

People and capabilities

Our ability to execute our strategy relies on the expertise and commitment of our people. Across the organization we continued to strengthen specialist capabilities, develop leadership, and embed cross-functional collaboration that support rare disease excellence. This focus on skills, culture and agility supports us in scaling effectively, sustaining quality and compliance, and delivering better outcomes for patients and other stakeholders.

Strategic priorities

Strengthen and sustain our marketed rare disease therapies

In 2025, we focused on enhancing the performance and long-term relevance of our marketed therapies. This included reinforcing the clinical and commercial positioning of our products, maintaining reliable supply, supporting appropriate use, and optimizing our geographic footprint.

A key priority during the year was expanding the addressable APDS population and driving uptake of Joenja®, while shaping an efficient, scalable organization to support sustained anticipated future growth.

During the year, we reinforced our franchise through sustained commercial execution. RUCONEST® continued to demonstrate resilient and growing U.S. demand, while Joenja® uptake accelerated, supported by increased patient identification and ongoing efforts to broaden the diagnosed APDS population. Following a strategic review, we made the decision to withdraw RUCONEST® from commercialization in non-U.S. markets, enabling a more focused operating model centered on the United States. This action strengthened capital allocation and sharpened our focus on key growth drivers and pipeline advancement.

Julia living with APDS

Expand global access for Joenja® in APDS

In 2025, we continued executing on our strategy to broaden global access to Joenja® by advancing regulatory submissions, enabling launches in priority markets, and expanding the eligible patient population through pediatric label expansion. We also maintained focus on improving diagnosis and identification of APDS patients, including continued efforts to resolve variants of uncertain significance (VUSs), and convert identified patients to commercial therapy in the U.S.

During the year, we achieved important regulatory and commercial milestones. We filed for approval in Japan for adult and pediatric patients, and successfully launched Joenja® in the United Kingdom. In the United States, we submitted a supplemental New Drug Application (sNDA) for pediatric patients aged 4–11 years and received Priority Review designation. We also progressed the clinical program evaluating Joenja® in younger patients (1–6 years), supporting future regulatory submissions and potential label expansion.

Advance our clinical-stage rare disease pipeline

In 2025, we advanced high-value clinical programs in rare diseases with significant unmet need through rigorous science, operational excellence, and discipline. Our priorities were to broaden the long-term potential of leniolisib beyond APDS, into larger PID populations impacted by immune dysregulation, and to progress the pivotal development of napazimone (KL1333) in primary mitochondrial disease.

We advanced two Phase II proof-of-concept clinical trials evaluating leniolisib in genetically defined PIDs linked to PI3K signaling and in CVID with immune dysregulation, with both studies on track for top-line data readouts in the second half of 2026. We also started and progressed Wave 2 of the pivotal FALCON clinical study evaluating napazimone (KL1333) in mtDNA-driven primary mitochondrial disease, following the completion of the Abliva acquisition. Wave 2 enrollment continued during the year, supporting a planned 2027 readout.

These advancements reflect disciplined execution across our late-stage programs and reinforce our commitment to building a diversified, high-value rare disease pipeline.

Pursue value-accretive business development

We maintained a disciplined approach to business development in 2025, focusing on rare disease assets that align with our scientific expertise and leverage our proven clinical development, regulatory, and commercial infrastructure.

During the year, we strengthened our rare disease pipeline through the acquisition of Abliva AB, adding KL1333 — now named napazimone (KL1333) — a late-stage asset in mtDNA-driven primary mitochondrial disease.

The transaction, initiated in late 2024, culminated in Abliva's delisting from Nasdaq Stockholm in March 2025 and our subsequent acquisition of the remaining minority shares, resulting in 100% ownership by June 18, 2025. This acquisition expanded our presence into mitochondrial disease, added a pivotal-stage program with significant long-term value potential, and reinforced our strategy of acquiring differentiated, science-driven assets in areas of high unmet need.

We also continued evaluating additional external innovation opportunities aligned with our strategic priorities, applying disciplined financial and strategic criteria to all opportunities assessed.

Shape an efficient and scalable organization

In 2025, we focused on developing an operating model that supports anticipated sustained portfolio growth, disciplined capital allocation, and future launches. We strengthened cross-functional capabilities, reinforced our core values, and worked to foster a culture of accountability and operational excellence.

During the year 2025, the first year under Fabrice Chouraqui's leadership, the organization was further aligned around clear strategic priorities and a performance-driven culture focused on execution. The October appointment of Kenneth Lynard as Chief Financial Officer further strengthened financial oversight and discipline as we continue to scale the business.

We advanced initiatives to optimize our cost structure and enhance organizational efficiency, including measures to reduce general & administrative expenses by US$9 million annually. This plan included a 20% net reduction in non-commercial and non-medical headcount. These actions enabled a more streamlined operating model, enhanced financial discipline and prioritized investments in commercial growth and pipeline advancement.

Operate responsibly and sustainably

In 2025, we continued our sustainability journey, embedding quality, compliance, and responsible business practices across our operations. We delivered new initiatives across Environmental, Social and Governance themes, from 'Compliance Day' that communicated updates to policies, broadening and deepening our culture of business integrity, to renewable energy contracts implemented at 2 more sites, reducing our greenhouse gas emissions. Such actions have strengthened our risk management processes and support the sustainable growth of Pharming.

Further detail on progress across our commercial portfolio, pipeline programs, and sustainability initiatives is provided in the Commercial portfolio review, Pipeline review, and Sustainability chapters.

Translating strategy into impact

We translate strategy into impact by aligning commercial portfolio growth with disciplined development, focused market expansion, and an enabling culture. We progress our pipeline where our capabilities create the most value — broadening leniolisib into additional primary immunodeficiencies and advancing late-stage assets like napazimone (KL1333) — while strengthening access for APDS through regulatory progress, targeted launches, and improved diagnosis.

We maintain RUCONEST® as a reliable, growing, and cash-generating U.S. franchise, supported by reliable supply. Evidence generation, from clinical programs to real-world data and health economic insights, underpins regulatory interactions and market access in large geographies.

Across development, supply chain, and commercial operations, we embed quality, compliance, and capital discipline, and we work collaboratively with clinicians, patient organizations, and genetic laboratories.

Our core values guide our teams every day, helping us execute on our strategy and enable earlier diagnosis, broader access and better outcomes for patients, and durable, re-investable cash flow.

Risks and opportunities

Our strategy is designed to deliver sustainable growth while managing the inherent complexities of rare disease markets. We continuously evaluate the risks and opportunities that shape our ability to execute, invest and grow.

Key risks

- **Evolving competitive landscapes:** New treatment options in HAE and emerging innovation in immunology has intensified competition and may require continued diversification of our portfolio.
- **Regulatory and reimbursement uncertainty:** Shifts in national and regional Health Technology Assessment (HTA) requirements, pricing pressure and reimbursement constraints could impact patient access and commercial performance.
- **Clinical and development risk:** As with all R&D-driven organizations, clinical results, regulatory reviews or development timelines may differ from expectations.
- **Supply and operational continuity:** Biologic manufacturing complexity and a global supply chain require ongoing risk mitigation to ensure reliable product availability for patients.
- **Macroeconomic volatility:** Changes in exchange rates, inflationary pressures and geopolitical instability may affect operational costs and financial performance.

Strategic opportunities

- **Expansion into new indications and geographies:** Growing awareness of rare diseases and unmet needs in PIDs create opportunities for label expansions and entry into additional markets.
- **Pipeline acceleration:** Our focused innovation model and advancements in clinical programs provide future growth opportunities beyond our current commercial portfolio.
- **Portfolio expansion through business development:** Disciplined, value-accretive in-licensing and acquisition opportunities that complement our rare disease focus, leverage our commercial and development capabilities, and strengthen our long-term growth trajectory.
- **Leverage AI in the identification of rare disease patients:** The fast evolution of technology and computation opens opportunity to analyze vast amounts of data to find patients who most likely suffer from a disease.
- **Operational leverage**: Continued financial and capital allocation discipline, digital enablement and commercial excellence support sustainable margin expansion over time.

Near-term focus for 2026

In 2026, we intend to grow Joenja® in APDS through intensified patient-finding and ex-U.S. approvals and launches while pursuing pediatric label expansion; reinforce RUCONEST®'s distinctive value proposition in a more competitive HAE market; advance leniolisib beyond APDS via Phase II proof-of-concept trial execution in additional PIDs with immune dysregulation and alignment on registrational routes; progress napazimone (KL1333) for primary mitochondrial disease through pivotal trial execution and integrated CMC readiness; and strengthen platform enablers — quality, supply reliability, data and analytics — while maintaining financial discipline.

Our strategy is supported by a responsible and sustainable operating model that prioritizes patient impact, ethical innovation and long-term value creation, which we explore further in our Sustainability section.

From Hospital Rooms to Hope for the Future

Frequent infections. Repeated hospital stays. More than 40 surgeries before adolescence. For Tyler, childhood was shaped by a rare and progressive immune disorder that went undiagnosed for years.

When genetic testing finally identified Activated PI3K Delta Syndrome (APDS), it brought long-awaited answers. Participation in a clinical study targeting the underlying immune dysregulation later marked a turning point — bringing greater stability and a renewed sense of possibility.

“ The biggest lesson I've learned is that this is bigger than just me. APDS manifests differently for each person. I used to believe my future was uncertain — now I look forward to it”

***Tyler,** Living with APDS*

11 known APDS patients
worldwide at the time of Tyler's diagnosis in 2012

1000+ today

Tyler living with APDS

Moments that matter

Advancing therapies, expanding possibilities

Numbers tell part of the story.
Real life shows the impact.

Every advance we make ultimately connects back to a person. A child who wants to attend school without interruption. An adult who wants the energy to work and care for family. A parent who wants stability instead of uncertainty.

At Pharming, everything begins and ends with the people we serve. Their experiences shape our priorities, guide our decisions and define how we measure success.

In 2025, continued growth of RUCONEST® and Joenja® reflected rising patient demand and expanding access. Behind that growth are individuals and families finding greater stability, confidence and possibility. When patient impact grows, performance follows. This is how we turn care into lasting change.

Living with HAE

Regaining control in unpredictable moments

For people living with hereditary angioedema (HAE), life can change in an instant. Swelling attacks can arrive without warning. They are sudden, unpredictable, and often painful - and sometimes life-threatening, disrupting work, school, travel, and family life. Access to reliable treatment can mean the difference between panic and preparedness.

Victoria's story

Victoria never knew when it would hit. Swelling could come out of nowhere – and the worst part wasn't only the pain. It was the uncertainty.

For years, she tried to keep going as if nothing was wrong. But living with HAE meant navigating a body that could change in a matter of hours, or even overnight — and a world that didn't always understand what it was seeing.

Even at home, the swelling wasn't always recognized for what it was. Her mother assumed she was simply putting on weight. It wasn't until her brother — a surgeon — witnessed a throat swelling that required emergency intubation that the family realized something far more serious was happening. Even then, they still had no answers.

In Barbados, she went back to doctors repeatedly, looking for an explanation. None came. She moved to the United States hoping someone would finally connect the dots — and that she could find a way to feel safe in her own body.

“ I know what I'm fighting now, and I have the drugs I need to treat it. It may not be perfect 100% of the time, but I know how to treat it. It’s no longer the hopelessness that you feel when you don’t know what is wrong or how to make things better.”

Instead, the years stretched on. Before she received the right diagnosis, Victoria went through more than eight years of chemotherapy for a condition she didn't have. She was intubated multiple times during severe attacks. When swelling closed in on her airway, there was no waiting it out, no “see if it gets better.” It was emergency care or nothing.

It took 15 years after moving to the U.S. for Victoria to be accurately diagnosed with HAE with normal C1-inhibitor (Type 3 HAE). She describes the diagnosis as an explanation that finally made her experience real.

By then, the unpredictability had already taken its toll. Attacks became so disruptive she had to stop working outside the home, stepping away from a career and routines she once loved.

Even after diagnosis, treatment wasn't straightforward. Some therapies triggered severe systemic reactions because Victoria is allergic to human plasma-derived C1 esterase inhibitor proteins. It meant that even with a name for her condition, she still didn't have a dependable way to respond when swelling began.

Working with her specialist, she eventually gained access to a treatment option that fit her medical needs — something she could use when it mattered most.

The change was practical, and it was emotional.
"Having access to a treatment I can rely on means I can plan ahead without constant fear. I finally feel prepared and confident, instead of anxious about what might happen next."

Today, she describes her experience simply as "living life again" — getting outdoors, hiking and gardening, and caring for the animals on her farm.

"Things that once made me anxious because of the uncertainty of whether I may swell or not are now possible because I have access to a medication that truly works."

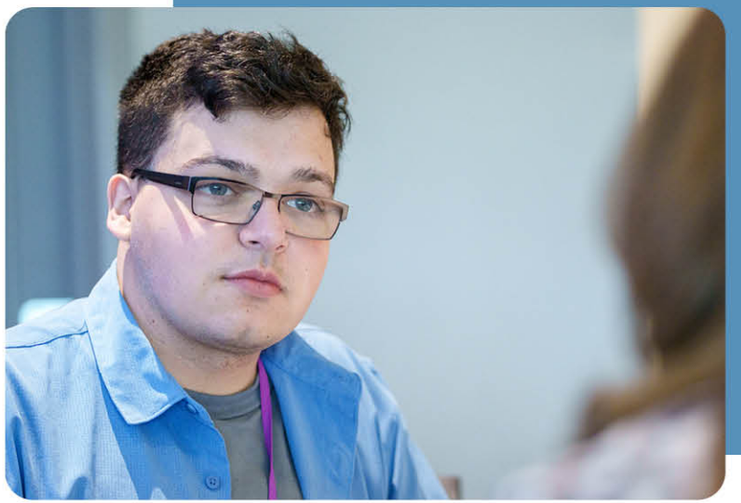
Victoria's experience reflects what reliable, targeted treatment can mean for patients living with unpredictable rare diseases — restoring control, confidence and daily independence.

Living with APDS

From constant infections to new possibilities

For many patients and families living with activated PI3Kδ syndrome (APDS), childhood can be marked by frequent infections, hospital stays, and immune-related complications. Diagnosis often follows years of uncertainty — and even after answers are found, the journey toward effective management can take time.

Tyler's story



" As a kid, I spent most of my time in the hospital. Instead of running around on the playground, I was getting prepped for surgery. I had over 40 surgeries as a child. Constantly being sick made me feel stripped of what some would call a 'normal' childhood."

Tyler is now a teacher, helping kids learn to code and build their own video games. He loves getting lost in a good book, and he finally gets to travel — milestones that once felt out of reach — because for most of his childhood, life was measured in infections and hospital days. He missed about a third of every school year, caught in a loop of getting sick, falling behind, catching up, and then getting sick again. While other kids learned routines and friendships, Tyler learned hospital corridors.

By age 10, Tyler had spent almost an entire year hospitalized. Chronic pneumonia led to repeated month-long hospital stays. His lymph nodes swelled to the size of golf balls and remained enlarged for extended periods. He experienced a wide range of complex symptoms that left his doctors and family searching for answers. Finding relief meant endless trial and error.
At one point, he was on 11 medications — each with its own side effects and challenges.

In 2012, researchers at the U.S. National Institutes of Health sequenced Tyler's entire genome and identified activated PI3K delta syndrome (APDS) as the cause. Finally, there was a name for what he'd been fighting, and while a diagnosis brought clarity, it didn't immediately mean having control. The years that followed were spent navigating treatment options and managing ongoing complications, and for a long time, Tyler felt uncertain about what his future might hold.

Up to that point, Tyler thought he was the only person living with the disorder. He convinced himself it would “end” with him — he didn't want anyone else to go through what he had lived through, and he didn't think he would live to be twenty. When he was offered the chance to join a clinical study, he didn't hesitate.

“I wanted to move beyond my previous treatments and find something that could truly help manage my symptoms. I also felt a responsibility to help others who might be diagnosed after me — to help create options for them.”

Over time, meaningful changes began to emerge. Symptoms that had persisted for years — including chronic vomiting, sinus infections, and ear complications — became less severe. He found he could go on walks without feeling as exhausted, and his breathing improved. Daily treatment also became more manageable.

“Before, I had to take multiple pills throughout the day. Now I take two — one at 11 a.m. and one at 11 p.m. It's such a relief. It's less of a constant reminder that I have APDS.”

“I have fewer infections now and feel more stable than I ever have. For the first time, I'm not just managing crises — I'm planning for the future.”

Today, Tyler's life is no longer defined by hospital stays and uncertainty. With greater stability he's making room for routine, independence, and long-term plans — milestones that once felt out of reach. His journey reflects what progress in rare immune disorders can make possible — shifting life from constant crisis to real stability.

Advancing Possibility Through Innovation
For individuals living with HAE and APDS, access to effective treatment can redefine what daily life looks like. Whether restoring control during unpredictable swelling attacks or helping stabilize immune dysregulation, scientific progress translates into tangible change.

By continuing to invest in research and global access, and by spending time to deepen our understanding of real-world patient experience, we aim to help more patients move beyond uncertainty toward greater stability, confidence and independence.

Patients at the Heart. Performance with Purpose.
When more patients gain access to effective treatment, impact grows — for families, for communities and for Pharming. That impact is reflected in our performance.

Our continued commercial momentum in 2025, including US$376.1 million total revenues, reflects increasing trust from physicians and patients. That trust enables us to invest in innovation, expand access and strengthen our global rare disease platform.

We measure progress not only in revenue growth, but in:
- Greater confidence in managing sudden attacks
- More stability in immune health
- More independence in daily life

This is how we create value — by putting patients first and delivering consistently. This is what performance with purpose looks like. Because ultimately, rare disease breakthroughs aren't just discovered. They're created together.

Approximately

95%

of rare diseases lack an approved treatment option

In rare diseases, one dedicated clinician can redefine a patient's path

Dr. Patel cares for patients living with rare conditions such as APDS, primary immunodeficiencies, hereditary angioedema and primary mitochondrial diseases- part of a landscape of more than 10,000 rare diseases worldwide, with approved treatments available for only about 5% of them.

Each day, he navigates limited evidence, complex presentations and diagnostic journeys that can span years. His work is defined not only by scientific rigor, but by a steadfast commitment to patients who often arrive without clear answers or established options. By listening closely and translating clinical insight into real-world evidence, Dr. Patel helps expand understanding and advance care.

In rare disease, progress often begins with a clinician willing to stand with patients at the edge of what is known — and move the field forward.

“ In rare diseases, understanding the lived experience and filling the evidence gaps are essential. Every new insight brings us closer to delivering options patients have been waiting for.”

Dr. Niraj Patel, Clinical immunologist & research partner

Commercial portfolio review

Our portfolio is designed to deliver meaningful impact for people living with rare and potentially life-threatening conditions while generating durable cash flow that enables continued investment in innovation. In 2025, we focused on disciplined execution across our marketed therapies, emphasizing appropriate use, strong operational performance, and high-quality evidence generation in support of patients and their families, clinicians, payers, and regulators.

Throughout the year, we strengthened commercial excellence and patient identification initiatives, maintained dependable product quality and supply assurance, and expanded real world evidence and health economic insights in preparation for key regulatory and access engagements. We also deepened our collaboration with clinicians, patient organizations, and genetic laboratories to help support earlier diagnosis and more informed treatment decisions.

This section reviews performance across our marketed portfolio, including RUCONEST®, a recombinant C1 esterase inhibitor for acute hereditary angioedema (HAE), and Joenja® (leniolisib), the first approved targeted treatment for activated PI3Kδ syndrome (APDS). It highlights how consistent execution, reliable supply, strengthened diagnostic and patient identification efforts, and effective payer engagement enable timely treatment for eligible patients while reinforcing the Company's long-term strategic and financial objectives.

MacKenna living with APDS

RUCONEST® for the treatment of HAE

RUCONEST® remains an established on-demand treatment for adults and adolescents experiencing acute HAE attacks. As the first and only recombinant C1 esterase inhibitor (rhC1-INH) protein replacement therapy, RUCONEST® restores functional C1-INH activity and addresses the underlying deficiency driving bradykinin-mediated swelling. In a landscape increasingly shaped by long-term prophylaxis — where breakthrough attacks remain clinically meaningful — RUCONEST® continued to serve as a cornerstone acute therapy in 2025, particularly for patients requiring dependable, rapid control.

With more than a decade of U.S. availability and a longstanding prescriber base, RUCONEST® serves a clearly defined patient population and generates reliable cash flow to support Joenja® (leniolisib) commercialization and broader pipeline investment.

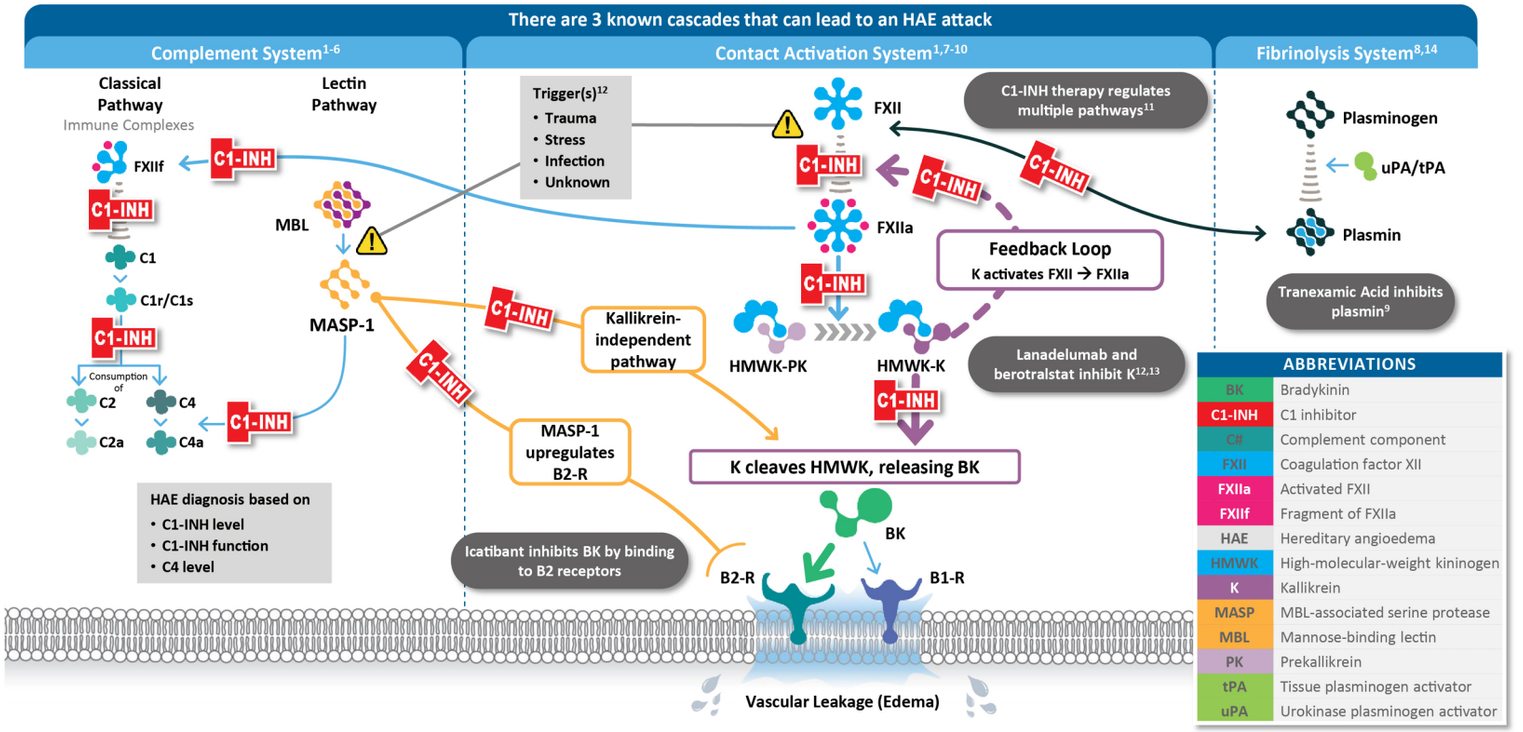
Product overview

RUCONEST® is a rhC1-INH approved for the treatment of acute HAE attacks in adults and adolescents. Produced using our transgenic technology platform, it provides a recombinant source of functional C1-INH to help control the inflammatory pathways that drive acute HAE symptoms. The therapy is administered intravenously and is used at the onset of an attack to help halt its progression.

With more than a decade of availability in the United States, RUCONEST® has an established role in acute HAE care, supported by consistent real-world use, dependable supply, and a mature safety profile.

The therapy has a long-standing position with physicians and patients in this rare disease community.

The scientific illustration below highlights the role of C1-INH across multiple inflammatory cascades relevant to HAE. This schematic reflects current understanding of the biological pathways involved; clinical implications beyond the approved indication are unknown.



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.

2025 performance overview

RUCONEST® delivered another year of strong performance in 2025, reflecting the product's stable role in the U.S. acute HAE landscape. Revenue for the full year 2025 reached a record US$317.9 million, representing a 26% increase compared to 2024.

In the U.S. market, we continued to expand our patient and prescriber base throughout the year. Revenue growth over the prior year reflects the benefit of a larger patient base, including patients with HAE with normal C1-INH.

With its efficacy, reliability and rapid onset of action via IV administration, RUCONEST® remains an established on-demand treatment option for patients experiencing more severe or frequent attacks who have failed other on-demand medications. During the year, we increased the RUCONEST® physician prescriber base by 6%. Unit sales volume in the U.S. increased by 20% for the full year.

“ [APDS] really does affect every aspect of your life.”

Patient living with APDS

Throughout 2025, RUCONEST® maintained steady utilization within the acute segment, supported by new patient enrollments and consistent clinical engagement. Operational execution remained strong, with continued supply, enabled by stable manufacturing throughput and robust quality oversight. Product quality and safety indicators remained consistent with historical performance.

RUCONEST® continued to be a dependable source of cash flow, supporting ongoing investment in Joenja® (leniolisib) commercialization and our broader development pipeline. Our priorities remain focused on maintaining reliable supply, responsible lifecycle management, and operational efficiency.

RUCONEST® maintained steady utilization among prescribers and patients who rely on rapid, reliable control of acute attacks and it is commercialized exclusively in the United States through our direct commercial organization.

In November 2025, following a strategic review, we announced the withdrawal of RUCONEST® from commercialization in all non-U.S. markets. These markets represented less than 2% of RUCONEST® revenue and were not financially sustainable long-term.

Throughout this transition, our highest priority has been ensuring continuity of care for affected patients. To support this, RUCONEST® remains accessible in certain countries outside the United States through the HAEi Global Access Program (HAEi GAP), subject to local regulatory frameworks and individual eligibility assessments.

With this transition complete, RUCONEST®'s commercial footprint is now fully focused on the U.S. market, supported by dedicated commercial, medical, supply, and patient-services teams.

Intellectual property and exclusivity

RUCONEST® has patent protection in the United States and European Union until October 7, 2026, and biologics reference product exclusivity in the United States through July 16, 2026.

Beyond formal exclusivity, RUCONEST® benefits from a proprietary recombinant production platform and specialized manufacturing processes that are complex and capital-intensive.

As a complex biologic produced using transgenic technology, the product requires highly specific technical capabilities, validated purification processes and regulatory oversight that may present meaningful barriers to entry.

Development of a biosimilar would require substantial investment, validated manufacturing capabilities and regulatory approval and would carry significant execution risk.

To date, we are not aware of any pending biosimilar applications referencing RUCONEST® in the United States or European Union.

While competition cannot be ruled out following expiry of exclusivity periods, we believe the scientific complexity of recombinant biologics, combined with manufacturing know-how and established physician familiarity, may limit near-term biosimilar risk.

“ With the right plan, I've learned that I can live beyond HAE.”

***Noah,** Living with HAE*

Noah living with APDS

Joenja® (leniolisib) for the treatment of APDS

Our second commercialized product, Joenja®, is an oral small molecule PI3Kδ inhibitor approved in the United States, United Kingdom, Australia and Israel, as the first and only targeted treatment indicated for activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS), in adult and pediatric patients 12 years and older, and in Japan for adult and pediatric patients aged 4 years and older. This represents the first global approval of Joenja® for children aged 4 to 11.

Product overview

As a disease modifying therapy, Joenja® targets the root cause of APDS, by selectively inhibiting PI3Kδ, supporting a more balanced pathway and addressing immune dysregulation characteristic of the disease. Joenja® inhibits production of phosphatidylinositol-3-4-5-trisphosphate, a cellular messenger that regulates processes including proliferation, differentiation, cytokine production, survival, metabolism, and cell migration/ trafficking.

Joenja®'s efficacy and safety in APDS were established in a Phase II/III randomized, placebo-controlled trial and an open-label extension (OLE) study.[14] The OLE with data from APDS patients 12 years and older taking Joenja® for up to 6 years was published in 2024 and completed in 2025, reported improvements in health-related quality of life (HRQoL) and a reduction in the severity of certain clinical manifestations over time. The study also noted decreases in prescribed medications for several patients, reflecting sustained clinical benefit throughout long-term follow-up. A full description of study designs, endpoints, outcomes, and safety is available in the Clinical Studies section of our 2024 Annual Report and referenced peer-reviewed publications.[15,16,17,18]

2025 performance overview

Joenja® continued to expand its presence in 2025 with approvals in Australia and the UK alongside securing reimbursement in the UK. During the year, we focused on strengthening the foundations needed for long-term, sustainable adoption: deepening patient identification, supporting appropriate initiation, and advancing reimbursement and regulatory submissions to broaden access.

Financial performance

Joenja® delivered solid financial performance in 2025, with uptake accelerating in the U.S. and a strong start to the UK launch, as rising patient identification continued to expand the diagnosed APDS population. Revenue for the full year 2025 increased to US$58.2 million, a 29% increase compared to 2024. Revenue growth in the fourth quarter of 2025 was driven by a significant increase in patients on paid therapy in the U.S. and increased demand in international markets, including strong patient uptake in the U.K. following the April 2025 launch and purchases under government-supported access programs.

The United States contributed 86% of 2025 Joenja® revenues, generating US$50.1 million in sales compared to US$40.5 million in 2024.

International markets outside the United States contributed 14% of 2025 Joenja® revenues, largely driven by initial uptake in the United Kingdom following positive reimbursement decisions by the National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC) and implementation through the National Health Service (NHS), and by early access in additional regions.

Across all markets, performance benefited from uninterrupted supply, reliable patient services and continued diagnostic testing support, which together contributed to sustained patient identification and treatment initiation. Inventory levels, manufacturing schedules, and logistics remained aligned with increasing global demand, and no material supply constraints or inventory build affected revenue in 2025.

Looking ahead, revenue growth is expected to be influenced by the pace of new patient identification, country specific reimbursement timelines, and ongoing regulatory progress, including activities related to the supplemental U.S. submission for patients aged 4–11 years. We remain focused on scaling access in approved markets, advancing new market launches, and supporting appropriate use among eligible APDS patients.

Patients on therapy

As of December 31, 2025, 120 patients in the United States were receiving Joenja® through commercial channels, representing a 25% increase from the 96 patients at the end of 2024. The number of patients on paid therapy in the U.S. increased by 24 during 2025, compared to an increase of 16 in 2024.

Patient identification

Globally, 998 diagnosed APDS patients of all ages had been identified by year-end, including 274 patients in the U.S. and 382 in core markets outside of the U.S. The number of U.S. patients diagnosed with APDS that we have identified increased by 40 in 2025 compared to an increase of 18 in 2024. Of these identified patients in the U.S., 181 patients are 12 years of age or older and currently eligible for treatment with Joenja® under the approved label, while 52 are between 4 and 11 years of age.

As of year-end 2025, there are 175 APDS patients in either a leniolisib Expanded Access Program (compassionate use), an ongoing clinical study, or a paid access program. Many of these patients may transition to commercial therapy following regulatory approvals in their respective markets.

APDS patient diagnostic support

Launched on March 2, 2021, navigateAPDS (U.S./Canada) is our sponsored program that provides no-charge, panel-based genetic testing and third-party genetic counseling to eligible individuals with suspected primary immunodeficiency, as well as familial variant testing for blood relatives after a positive molecular diagnosis. The program is designed to reduce barriers to definitive diagnosis for APDS by combining broad immunodeficiency gene panels, expert pre- and post-test counseling, and family cascade testing. In Europe, we continue to intensify patient-finding through collaborations with immunology centers of excellence and national networks focused on rare immune disorders.

Earlier and accurate diagnosis can help clinicians distinguish APDS from other immunodeficiencies and immune dysregulation syndromes and inform appropriate management, including consideration of Joenja® where approved and indicated. By supporting family cascade testing, the program also helps identify previously undiagnosed relatives who may be affected.

We continued to advance multiple initiatives in 2025 to support earlier and more accurate diagnosis of APDS. Alongside our sponsored genetic testing program in the U.S. and Canada, we expanded collaborations with genetic testing companies, clinicians, and patient communities to reduce barriers to testing and increase appropriate family cascade evaluation. As APDS is an inherited condition, we believe that many of the more than 270 identified U.S. patients may have undiagnosed relatives, and as such family cascade testing is being offered to help identify related individuals who may also be affected.

VUS patient reclassification and APDS prevalence

APDS diagnosis requires integration of clinical presentation, immune function, and genetic testing, with a pathogenic or likely pathogenic (P/LP) variant in the *PIK3CD* or *PIK3R1* gene needed to confirm the diagnosis. However, many patients with clinical features consistent with APDS receive a variant of uncertain significance (VUS) genetic finding, where the disease relevance is not yet known.

Data from our navigateAPDS program show that VUSs in *PIK3CD*/*PIK3R1* occur roughly four times more frequently than currently known P/LP variants. This underscores both the scale of diagnostic uncertainty and the potential impact of resolving VUSs on the number of patients who may eventually receive a confirmed diagnosis.

As of December 31, 2025, approximately 1,800 individuals in the U.S. are known to carry a VUS in one of the APDS implicated genes. When a VUS is reclassified as P/LP, clinicians can establish a definitive APDS diagnosis, allowing patients to be considered for Joenja® treatment if they meet the approved label criteria.

In June 2025, leading peer-reviewed journal *Cell* published results from a high-throughput functional screening study conducted by Columbia University, evaluating portions of *PIK3CD* and *PIK3R1*. The research demonstrated that numerous previously uncharacterized variants, including existing VUSs, exhibited PI3Kδ hyperactivity, a hallmark of APDS biology. Functional data of this type is recognized by expert bodies such as American College of Medical Genetics and Genomics and ClinGen as one component that can contribute to variant classification, alongside clinical, genetic, and computational evidence.

Based on evaluation of the data from this study by genetic testing laboratories, additional complementary evidence will be required to enable variant interpretation by genetic testing laboratories. We are planning new experiments to generate the data needed for genetic testing laboratories to evaluate VUSs identified in patients who have undergone genetic testing for APDS or other immunodeficiencies. We expect to provide an estimate of how many of these patients may be diagnosed with APDS following completion of these experiments. To generate this data, we have initiated a broader collaboration with Columbia University to extend functional testing to the remaining regions of both APDS genes using next-generation screening technologies. This expanded effort is designed to evaluate many more VUSs than the initial study, enabling a more complete understanding of the mutational landscape associated with PI3Kδ hyperactivation.

A second conclusion of the research conducted by Columbia University was that APDS may have a broader clinical presentation and significantly higher prevalence than previously assumed, an important consideration for long-term market growth. Further research is on-going on this topic.

We remain committed to supporting the full diagnostic journey for individuals and families affected by APDS and will continue to work with laboratories, clinicians, and academic partners to generate and interpret the evidence needed to resolve both novel variants and existing VUS.

These efforts are expected to enable the identification of additional patients with APDS, helping to close the diagnostic gap created by uncertainty in current genetic testing.

Operational performance
Operational readiness remained a key driver of Joenja® performance during 2025. Supply remained uninterrupted across all commercial markets.

Access and regulatory progress

In 2025, we made significant progress advancing global access to Joenja® (leniolisib) for people living with APDS.

United States

Joenja® is approved in the United states as the first and only targeted treatment for APDS patients 12 years of age and older. During 2025, we continued to expand patient identification, supporting increasing uptake in the U.S.

United Kingdom

On September 25, 2024, the U.K. Medicines and Healthcare products Regulatory Agency (MHRA) granted marketing authorization for Joenja® for the treatment of APDS in adult and adolescent patients 12 years of age and older. Joenja® was the first new medicine approved by the MHRA via the International Recognition Procedure (IRP) using the U.S. FDA as reference regulator. We launched Joenja® in the U.K. in April 2025.

England & Wales
On April 23, 2025, the National Institute for Health and Care Excellence (NICE) issued positive final guidance recommending Joenja® for routine reimbursement and use within the National Health Service (NHS) in England and Wales for the treatment of APDS in adult and pediatric patients aged 12 years and older. This followed NICE's positive final draft guidance published on March 13, 2025.

In England, Joenja® is funded through the Innovative Medicines Fund, enabling immediate access for eligible patients, while in Wales it is funded through the NHS in designated specialist centers.

Scotland
In Scotland, on December 8, 2025, the Scottish Medicines Consortium (SMC) published its initial ultra-orphan assessment for Joenja®. From March 5, 2026, Joenja® can be prescribed within the ultra-orphan pathway while further evidence on its effectiveness is generated over a period of up to three years. The first patient in Scotland is now receiving treatment, with data collection ongoing to support a future reassessment and a decision on routine use within NHS Scotland.

Australia

In March 2025, we received positive feedback from the Australian Advisory Committee on Medicines, and we received approval for Joenja® from the Australian Therapeutic Goods Administration (TGA) for the treatment of APDS in adult and adolescent patients 12 years of age and older.

In July 2025, the Australian Pharmaceutical Benefits Advisory Committee (PBAC) recommended the listing of Joenja® on the Pharmaceutical Benefits Scheme (PBS) and reimbursement negotiations are ongoing.

Israel

In April 2024, the Israeli Ministry of Health granted marketing authorization for Joenja® for the treatment of APDS in adult and pediatric patients 12 years of age and older.

We continue to work with Kamada Ltd. as our commercial partner for Joenja® in Israel; leniolisib is listed on Kamada's product portfolio for adults and adolescents ≥12 years (weight-based per local label). Discussions with health authorities on reimbursement have been progressing.

Japan

In March 2026, the Japanese Ministry of Health, Labour and Welfare (MHLW) granted marketing authorization for Joenja® for the treatment of APDS in adult and pediatric patients aged 4 years and older, following a positive recommendation from the Japanese Pharmaceutical Affairs Council. Joenja® is the first approved treatment for APDS in Japan and this approval of Joenja® is the first anywhere globally for children aged 4 to 11.

Japan remains a strategically important market, supported by its advanced rare disease infrastructure and strong clinical engagement. The Japanese Pharmaceuticals and Medical Devices Agency (PMDA) reviewed the application under the Priority Review pathway, following Orphan Drug Designation granted by the MHLW in May 2023. In August 2023, the first patient was enrolled in a local Phase III clinical trial to support regulatory approval in Japan. An interim analysis completed in 2025 showed safety and efficacy findings consistent with the global Phase II/III program, supporting the regulatory submission, which was submitted in June 2025. Eligible patients from the clinical study will continue to receive leniolisib through an open-label extension period to further assess long-term safety and tolerability.

Under an agreement with Pharming, OrphanPacific, Inc. serves as the Marketing Authorization Holder for Joenja® in Japan and, in collaboration with Pharming, is responsible for product supply and distribution. Commercial launch is expected following agreement with the MHLW on the National Health Insurance drug price.

For more information our regulatory activities in other regions, please refer to the section Leniolisib for APDS: global regulatory filings within the Pipeline review of this Annual Report.

Named-patient and early-access programs
In countries where leniolisib is not yet commercially available, physicians can request access for eligible patients through named-patient or early-access routes, subject to local health authority regulations and approvals.

Intellectual property and exclusivity

Joenja® (leniolisib) is protected worldwide by the Novartis composition-of-matter patent family (e.g., U.S. Patent No. 8,653,092, European patent EP2590974B1, which provides patent protection for Joenja®, such as treatment of APDS in the United States and the European Union through July 2036). A six-month pediatric extension, if granted, could extend U.S. patent protection to January 2037.

In the United States, the patent term was adjusted by the USPTO at grant to account for regulatory review delays, and we have applied for a patent term extension following FDA approval of Joenja®.

In the European Union, we intend to seek a Supplementary Protection Certificate (SPC) following EU marketing authorization. Together, these adjustments and extensions are expected to support patent protection through July 2036 (or January 2037 with a pediatric extension).

In addition to patent protection, Joenja® benefits from regulatory exclusivity in Europe. Upon EU marketing authorization, Joenja® would be eligible for ten years of orphan medicinal product market exclusivity. Following successful completion of the agreed Pediatric Investigation Plan (PIP), this period may be extended by up to two additional years. We therefore anticipate that patent protection is expected to extend beyond the period of orphan market exclusivity in Europe, supporting long-term protection for Joenja®.

“ It's about the future and what it holds, and what else is going to pop up… A lot of it's just about what comes for them, and what life choices they're going to make now that they have the knowledge…. To be able to say, ‘We have this mutation. Do we want to have children? And what does all that mean?’”

Family living with APDS

~44
per million patients with immune dysregulation

Every breakthrough begins with understanding what one patient needs

Matt works at the intersection of deep scientific understanding and disciplined execution, helping drive future value for patients and for Pharming. He leads cross-functional teams across development and commercialization to ensure Joenja® reaches eligible APDS patients while advancing Phase II programs in broader primary immunodeficiencies (PIDs) with immune dysregulation.

Inspired by a passion to develop transformative therapies for patients with immune dysregulation — many of whom have limited or no approved treatment options — Matt is driven by the impact a fully integrated, cross-functional team can deliver. His inclusive and curious leadership reflects Pharming's determination to find new solutions, translating insight and knowledge into action and delivering meaningful, lasting progress for patients who deserve better.

“ Understanding our science is where everything begins. What drives me is leading cross-functional teams to translate that science into sustainable value - for patients, the healthcare ecosystem and the business. By working closely with the scientific and patient communities, we move with urgency to ensure patients feel the impact as quickly as possible.”

Matt Cohen, Vice President Program Lead Leniolisib

Pipeline review

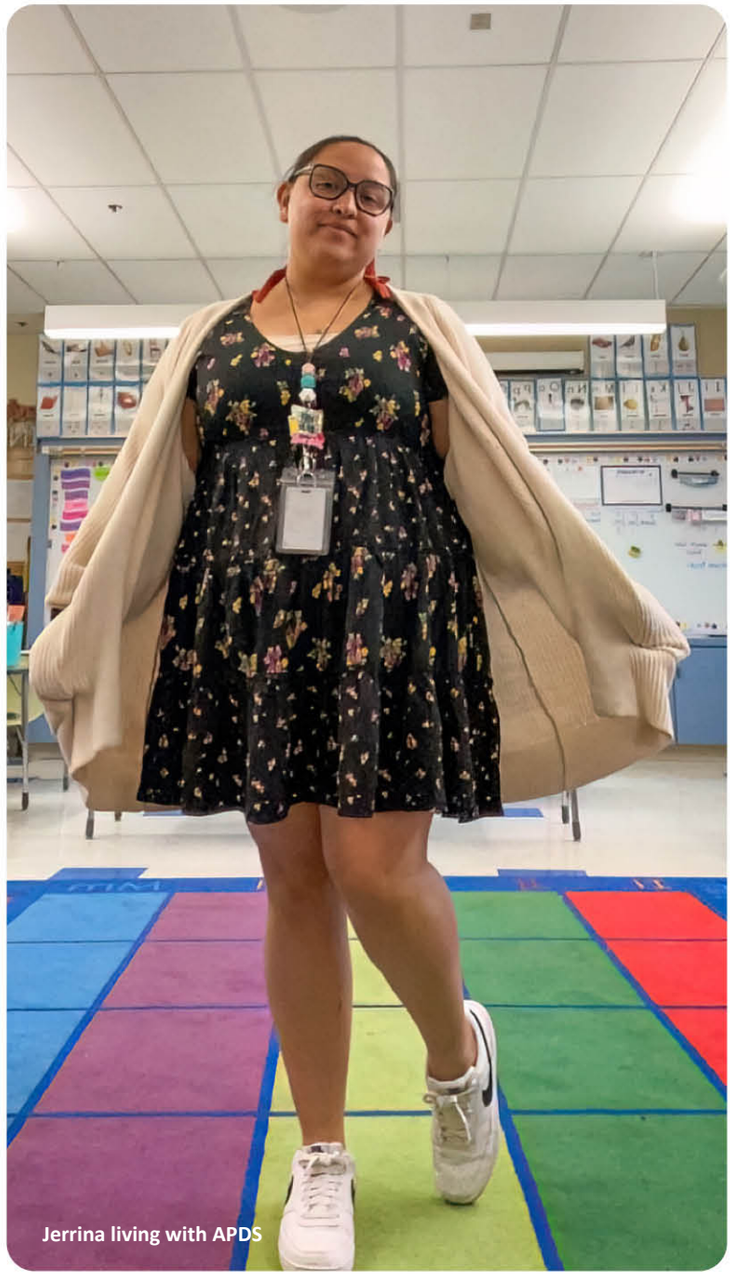
Advancing purposeful innovation for rare disease communities worldwide

In 2025, we continued to advance our high-value rare disease pipeline, progressing clinical programs designed to address serious and underserved immune and mitochondrial conditions. Our progress reflected disciplined execution across global development and regulatory pathways, strong scientific foundations, and sustained investment in areas of significant unmet medical need.

Across the portfolio, we worked to advance leniolisib beyond its approved indication in APDS. During the year, we supported global regulatory submissions for APDS in additional markets and progressed two Phase II proof-of-concept studies evaluating leniolisib in broader PIDs with immune dysregulation, including CVID. These studies build on the established mechanism of action and clinical experience in APDS and represent the next phase of development aimed at addressing substantially larger patient populations.

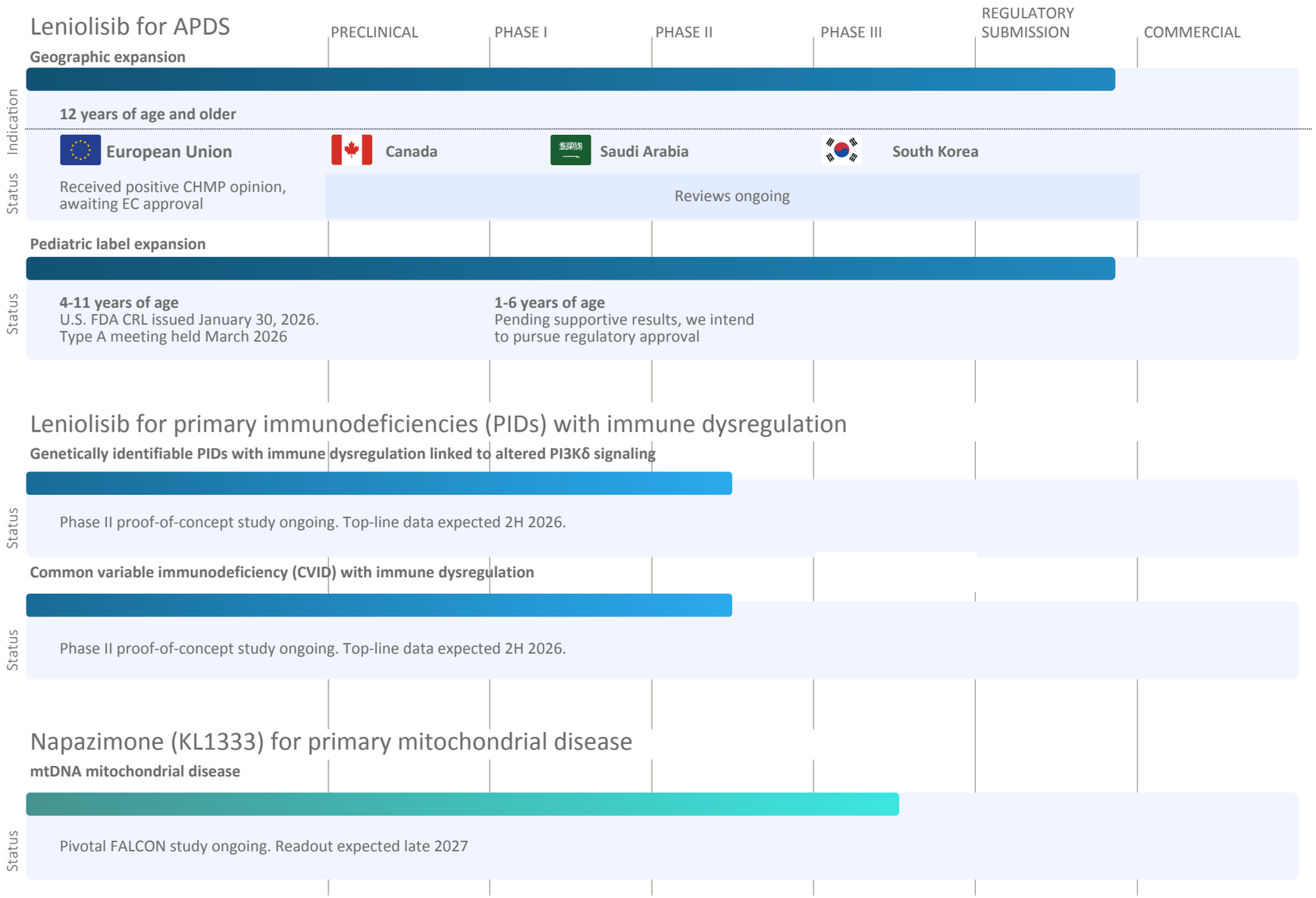
In parallel, we continued advancing napazimone (KL1333) in the pivotal FALCON study for mtDNA-driven primary mitochondrial disease (PMD). Following the integration of Abliva AB, we progressed global site expansion and patient recruitment throughout 2025. The program builds on the positive interim futility analysis completed in 2024 and remains on track for a 2027 readout.

Together, these programs form the core of our high-value clinical-stage pipeline and position the Company for a series of important potential clinical and regulatory milestones in 2026, including anticipated regulatory decisions for APDS in key markets and topline results from the two Phase II leniolisib studies in PIDs with immune dysregulation.



Jerrina living with APDS

Pipeline



All investigational programs above have not been approved for the indications under investigation. Safety and efficacy have not been established.

Leniolisib for APDS: global regulatory filings

Expanding patient access to the first targeted therapy for APDS

Joenja® (leniolisib) is the first and only targeted therapy approved for the treatment of APDS in patients 12 years of age and older. Its approval in multiple jurisdictions is supported by robust clinical data demonstrating meaningful improvements in lymphoproliferation and immunophenotype correction. During 2025, we advanced regulatory filings to expand access for patients across key regions.

European Economic Area

In May 2024, the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) affirmed the positive clinical benefit and safety profile of leniolisib, consistent with the assessment provided by the EMA's Ad-hoc Expert Group and identified one outstanding chemistry, manufacturing and controls (CMC) item related to the definition of regulatory starting materials for leniolisib's manufacturing process.

In 2025, the CHMP maintained this position and we completed all required manufacturing activities and quality controls and, within the extended deadline of January 2026, submitted a comprehensive response including supporting data to the EMA.

On March 26, 2026, the CHMP adopted a positive opinion recommending marketing authorization for leniolisib in adult and pediatric patients aged 12 years and older. A final decision by the European Commission (EC) on the marketing authorization for Joenja® (leniolisb) under exceptional circumstances is expected within approximately two months. If approved, Joenja® (leniolisib) would become the first approved treatment for APDS in the European Union.

The centralized marketing authorization would be valid in all 27 European Union Member States, as well as Norway, Iceland and Liechtenstein.

Current status: Received CHMP positive opinion, awaiting EC approval decision in the second quarter of 2026.

Canada

Our regulatory pathway in Canada continued to progress in 2025:

- A regulatory submission for APDS (12+) was filed in the third quarter of 2023.
- In July 2024, we submitted a response to a Notice of Deficiency.
- In January 2025, Health Canada issued a Notice of Non-Compliance requesting additional CMC data.
- Health Canada granted us an extension to February 2026 to submit the required information.
- We submitted a response with additional CMC data to Health Canada at the end of January 2026.
- A decision on the regulatory submission is expected by mid-year 2026.

Current status: Review ongoing; response submitted and decision expected by mid-year 2026.

Additional markets

Saudi Arabia

We submitted an NDA to the Saudi Food & Drug Authority (SFDA) in November 2024 for patients aged 12 years and older. The review is aligned with the SFDA reliance pathway, which leverages FDA decisions. A regulatory decision is expected following completion of the reliance-based assessment.

Current status: Review ongoing.

South Korea

In May 2024, South Korea granted Orphan Drug Designation for leniolisib in APDS. In March 2025, we submitted an NDA for patients aged 12 years and older to the Ministry of Food and Drug Safety (MFDS).

Current status: Review ongoing.

Pediatric label expansion — Driving toward earlier intervention for children living with APDS

We are committed to ensuring that children with APDS have equitable access to targeted treatment as early as possible in their disease journey. In 2025, substantial progress was made across our two pediatric clinical development programs, covering children 4–11 years and 1–6 years of age.

Our pediatric strategy builds on the robust efficacy and safety profile demonstrated in adolescents and adults, as well as regulatory recognition of the significant unmet medical need in younger APDS patients. Global regulatory authorities, including the EMA and MHRA, have already endorsed our Pediatric Investigation Plans (PIPs), supporting the structured expansion of leniolisib into younger age groups.

APDS in children 4 to 11 years of age

Clinical Development Progress

In 2024, we completed a global Phase III study evaluating leniolisib in children aged 4 to 11 years of age with confirmed APDS, supporting the potential expansion into this younger patient population. Twenty-one patients were enrolled and completed the 12-week treatment period.

The study applied a weight-based dosing strategy to ensure appropriate pediatric exposure and utilized endpoints consistent with the pivotal adolescent and adult program, enabling meaningful cross-study comparison.

The trial demonstrated improvements across the co-primary endpoints, including reduction in index lymph node size and an increase in the percentage of naïve B cells, reflecting modulation of the underlying immune dysregulation characteristic of APDS.

Health-related quality of life measures were also assessed to capture broader functional outcomes in this pediatric population.

The safety profile was consistent with prior experience; all treatment-emergent adverse events were mild to moderate in severity, and no drug-related serious adverse events were observed.

Collectively, we believe these data support the clinical rationale for extending leniolisib to younger APDS patients and formed the basis for regulatory submissions.

Regulatory Progress

United States

In July 2025, we submitted a supplemental New Drug Application (sNDA) to the U.S. FDA seeking approval of Joenja® (leniolisib) for children aged 4 to 11 years of age. The application was accepted for review in October 2025 and granted Priority Review designation, reflecting the significant unmet need in the pediatric APDS population.

On January 30, 2026, the FDA issued a Complete Response Letter (CRL). The CRL requested additional pediatric pharmacokinetic (PK) data to further support dosing in lower-weight pediatric patients, as well as clarification related to an analytical method used in production batch testing. The FDA did not identify any new safety concerns, and the currently approved indication for patients aged 12 years and older remains unaffected.

We have engaged with the FDA to address the clinical pharmacology and batch testing methodology issues outlined in the letter. We held a Type A meeting with the FDA on March 26, 2026, to discuss the Agency's feedback and align on a path forward for resubmission. We expect to receive written feedback from the FDA in the form of meeting minutes, which will inform our next steps, including the timing of a resubmission.

Current status: Type A meeting held on March 26, 2026, awaiting written feedback in form of meeting minutes.

Japan

In March 2026, the MHLW granted marketing authorization for Joenja® (leniolisib) for the treatment of APDS in adult and pediatric patients aged 4 years and older. This approval of Joenja® is the first anywhere globally for children aged 4 to 11.

Current status: Approved in March 2026.

APDS in children 1 to 6 years of age

Clinical Development Progress

Children aged 1-6 years represent the youngest segment of the pediatric APDS population and are often those who stand to benefit most from early intervention. In 2025:

- Enrollment in the global Phase III trial was **completed in April 2025**.
- This trial utilizes a **bespoke pediatric granulated formulation** of leniolisib designed to ensure age-appropriate administration and optimized tolerability.
- The trial evaluates safety, tolerability, and efficacy in **15 patients**, using the same core endpoints leveraged in earlier studies:
  - **Lymphoproliferation** measured by imaging
  - **Immunophenotype correction** through naïve B-cell assessment; and
  - Secondary measures including patient- and caregiver-reported quality-of-life metrics
- As in the 4–11 cohort, this trial includes an **open-label extension period** enabling continued treatment for a minimum of one additional year following the initial 12-week assessment period.

Topline results from this trial are anticipated following database lock and analysis beyond 2025.

Current status: Pending supportive results, we intend to pursue regulatory approvals for this younger age group.

Regulatory Foundations & Global Framework

Both pediatric programs are conducted under EMA- and MHRA-approved Pediatric Investigation Plans, enabling harmonized global development aligned with regulatory expectations for clinical trial design, dosing, safety monitoring, and benefit-risk assessment.

The unified design across the 1–6 and 4–11 studies is intended to ensure that pediatric data packages are scientifically coherent and directly comparable to the adolescent/adult evidence base. The pediatric expansion also supports our broader mission to ensure equitable access to targeted therapy across all age groups living with APDS.

“ Living with APDS while working in healthcare education gives me a unique perspective. I see how awareness shapes outcomes, which is why I'm passionate about teaching others, advancing understanding, and helping ensure patients are recognized, supported and cared for sooner.”

Annie, *Living with APDS*

Annie living with APDS

Leniolisib for primary immunodeficiencies with immune dysregulation (investigational)*

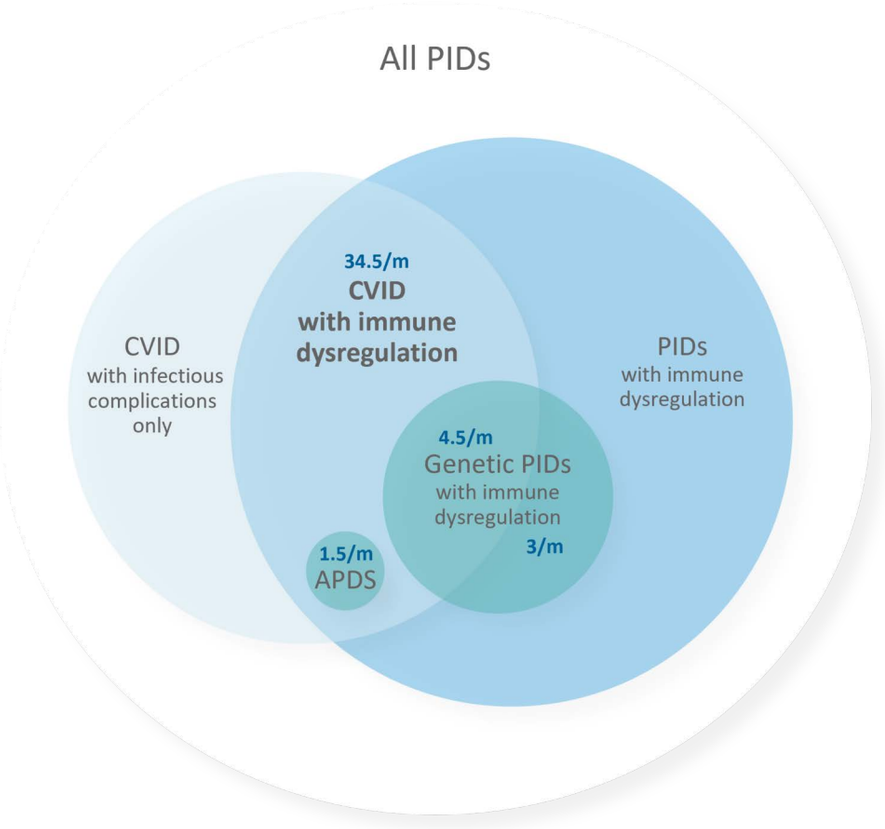
Expanding therapeutic potential to broader immune dysregulation disorders

In 2025, we advanced two Phase II proof-of-concept (PoC) studies evaluating leniolisib beyond APDS:
(i) Genetically identifiable PIDs with immune dysregulation linked to altered PI3Kδ signaling, and
(ii) Common variable immunodeficiency (CVID) with immune dysregulation identified independently of genetics.

Both studies address substantially larger patient populations than APDS and build on the validated PI3Kδ mechanism, a key regulator of immune cell activation and survival. The studies are designed to evaluate safety and tolerability, pharmacokinetics and pharmacodynamics, and exploratory clinical efficacy to inform potential Phase III development beyond APDS. Topline results for both studies are anticipated in the second half of 2026.

Leniolisib has demonstrated clinically meaningful efficacy and a favorable safety and tolerability profile in APDS, with sustained improvements in lymphoproliferation and disease-relevant immunologic biomarkers. We believe the consistent modulation of the PI3Kδ pathway provides a strong scientific rationale for investigating leniolisib in broader primary immunodeficiencies characterized by immune dysregulation.

Expanding into these high-unmet-need immune dysregulation populations represents a central pillar of our pipeline strategy and is supported by regulatory recognition, including Fast Track designation for PI3Kδ-linked PIDs and Orphan Drug Designation for CVID.



Not to scale with population sizes

* Investigational status: Leniolisib is investigational for PIDs and for CVID with immune dysregulation; safety and efficacy have not been established for these uses.

Genetically identifiable PIDs with immune dysregulation linked to altered PI3Kδ signaling

Rationale and patient population

Building on APDS biology and experience, leniolisib is being evaluated as a potential treatment in PIDs where enhanced PI3Kδ pathway signaling drives immune dysregulation. Target conditions include ALPS-FAS[19], CTLA4 haploinsufficiency[20], NFKB1 haploinsufficiency[21], and PTEN deficiency[22], which often present with lymphoproliferation, cytopenias, and/or organ-specific autoimmune/inflammatory manifestations.

Epidemiology context

The combined targeted PID population for this study is estimated at ~7.5 patients per million, compared with ~1.5 patients per million for APDS, reinforcing the opportunity to address larger patient groups if the benefit-risk profile is supportive.

Study design

- **Type:** Phase II, single-arm, open-label, dose-range–finding study (~12 patients).
- **Key eligibility:** one of the following genetic mutations linked to PI3Kd signaling: SOCS1, PTEN, CTLA4, NFKB1-GOF, FAS (germline or somatic) or RALD (somatic NRAS or KRAS) plus at least one clinical symptom of: Cytopenia, splenomegaly, lymphadenopathy or GLILD.
- **Objectives:** Safety/tolerability, PK/PD, exploratory clinical efficacy; to inform a potential Phase III program.
- **Site & leadership:** NIAID/NIH; PI: Gulbu Uzel, M.D.; Co-I: V. Koneti Rao, M.D., FRCPA (ALPS Clinic).
- **Milestones:**
  - First patient dosed October 29, 2024.
  - FDA Fast Track designation granted February 2025, enabling enhanced U.S. regulatory interaction
- **Timing:** Top-line results expected in the second half of 2026.

CVID with immune dysregulation

Rationale and patient population

CVID is the largest symptomatic PID group; an estimated ~50% of patients exhibit immune-dysregulation manifestations — such as splenomegaly/lymphadenopathy, autoimmune cytopenias, interstitial lung disease (ILD), and enteropathy — that are associated with higher morbidity and mortality than primarily infectious phenotypes. The unmet need for these patients is high with an 11 times higher risk of early death than CVID patients who do not have immune dysregulation[23] and there are no approved or effective treatments. Many patients display APDS-like clinical and immunologic features, supporting shared pathophysiology and the rationale for PI3Kδ modulation with leniolisib.

Epidemiology context

The targeted CVID with immune dysregulation prevalence is ~39 per million, representing a substantially larger opportunity to address unmet need if clinical benefit-risk is demonstrated.

Regulatory engagement and study initiation

We engaged with the FDA and EMA prior to launch and initiated the Phase II study in March 2025.

Study design

- **Type:** Phase II, single-arm, open-label, dose-range–finding, multi-center study (~20 patients, ≥12 years).
- **Key eligibility:** CVID diagnosis with lymphoproliferation and ≥1 additional immune-dysregulation manifestation (e.g., ILD, autoimmune cytopenias, enteropathy).
- **Objectives:** Safety/tolerability, PK/PD, exploratory clinical efficacy to guide Phase III.
- **Sites & leadership:** Lead Investigator: Jocelyn Farmer, M.D./Ph.D. (Lahey Hospital & Medical Center / Beth Israel Lahey Health), with additional sites in the U.S., U.K., and EU.
- **Milestones:** First patient dosed in March 2025.
- **Timing:** Top-line results expected in the second half of 2026.

"Trying to get people to understand that you might struggle with one thing at one point, but then it's something else at a different time,...That it still falls under the umbrella of APDS. It's not that you have a million and one different things. It's all caused by the same thing."

Patient living with APDS

Napazimone (KL1333) for primary mitochondrial disease (investigational)*

Advancing a pivotal program in mtDNA-driven primary mitochondrial disease

In 2025, following the completion of the acquisition of Abliva AB, KL1333 (now napazimone) became a core component of our rare disease pipeline. The transaction, initiated in late 2024 and concluded with our full ownership in June 2025, strengthened our presence in mtDNA-driven primary mitochondrial disease (PMD) and added a late-stage clinical asset with the potential to address significant unmet medical need.

During 2025, we advanced the pivotal FALCON study, a global, randomized, placebo-controlled trial, designed to support regulatory approval, evaluating napazimone (KL1333) in adults with genetically confirmed primary mitochondrial disease (PMD) experiencing severe fatigue and myopathy. The study builds on a positive blinded interim futility analysis completed in 2024, prior to the acquisition, in which both FDA-agreed alternative primary endpoints passed futility and the Data Monitoring Committee recommended continuation. In 2025, we initiated Wave 2 of the study, expanded global site activation, and continued patient recruitment, maintaining momentum toward the anticipated late 2027 readout.

Napazimone (KL1333) is an oral modulator of NAD^+/NADH designed to address the impaired cellular energy production characteristic of mtDNA-driven multisystemic PMD. The program targets clinically meaningful, patient-prioritized manifestations of disease, including myopathy and fatigue that significantly affect daily functioning and quality of life.

Study design and endpoints

FALCON is a pivotal study (3:2 napazimone vs placebo) enrolling approximately 180 adults for 48 weeks of twice-daily dosing (total daily dose 50–100 mg). The trial includes two alternative primary efficacy endpoints:

1. PROMIS® Fatigue Mitochondrial Disease Short Form; and

2. 30-second Sit-to-Stand test

of which one must be positive to support a potential marketing application.

A positive blinded interim futility analysis conducted following the first wave of recruitment confirmed acceptable safety to date and demonstrated that both primary endpoints passed futility, indicating potential for benefit at final analysis and supporting progression to the second recruitment wave.

Regulatory designations and addressable population

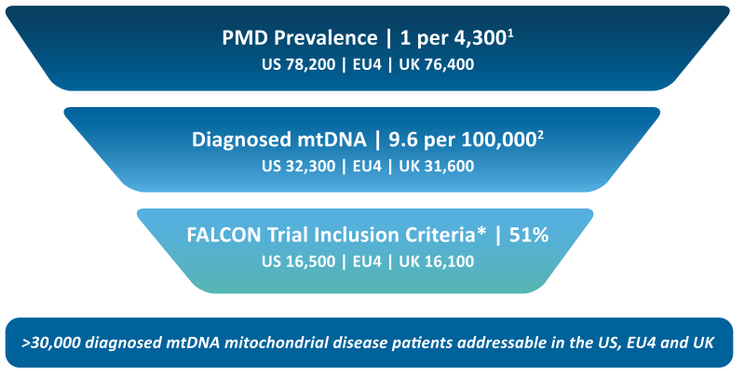
Napazimone (KL1333) has received U.S. Fast Track designation for PMD and Orphan Drug Designation in both the U.S. and EU. Across the U.S., EU4 (France, Germany, Italy, Spain), and the U.K., more than 30,000 diagnosed patients with mtDNA-driven PMD are potentially addressable if development is successful and regulatory approvals are obtained.

Next milestones

Pivotal read-out from FALCON is anticipated in late 2027. Subject to clinical outcomes and health authority interactions, regulatory submissions could follow thereafter.

At-a-glance

- **Indication:** mtDNA-driven primary mitochondrial disease (PMD) (adults) — severe fatigue & myopathy.
- **Mechanism:** NAD^+/NADH modulation (oral).
- **Trial:** FALCON pivotal trial (randomized, placebo controlled; ~180 patients; 48 weeks). Continued global site activation and enrollment-controlled; ~180 patients; 48 weeks).
- **Endpoints:** PROMIS® Fatigue (PMD Short Form); 30-sec Sit-to-Stand (alternative primaries).
- **Interim analysis:** interim futility passed on both endpoints
- Designations: Fast Track (U.S.); Orphan Drug Designation (U.S./EU).
- **Population:** >30,000 diagnosed mtDNA-PMD patients potentially addressable across U.S., EU4, U.K. (if approved).
- **Next milestone:** Readout expected late 2027.



* Investigational status: napazimone (KL1333) is investigational; safety and efficacy have not been established.
*mtDNA mutations including m.3243A>G, large scale mtDNA deletions, m.8344A>G and other pathogenic mtDNA variants causing multisystemic disease
1. Gorman, G.S. et al. Prevalence of nuclear and mitochondrial DNA mutations related to adult mitochondrial disease. Ann Neurol 2015 May;77(5):753-9.
2. Gorman, G.S. et al. Mitochondrial Diseases. Nat. Rev. Vol 2, 1-22 (2016).

Sustainability

Since 2023 we have taken important steps to develop and implement our sustainability program. We remain committed to delivering on all material Environmental, Social and Governance (ESG) topics selected for Pharming, with our goals and progress structured into four sections:

General information
covering our sustainability strategy, double materiality assessment, stakeholder engagement, governance and connection to our corporate strategy.

Environmental
focusing on climate change mitigation and adaptation.

Social
addressing, amongst others, employee well-being and engagement, employee training and skills development, human rights, patient safety and product quality, and access to products and services.

Governance
covering business ethics and animal welfare.

General information

We launched our sustainability program in early 2023, responding to international and national regulatory developments, embarking on a learning journey for ourselves to understand what sustainability means to Pharming. Since 2023, sustainability-related regulation has continued to evolve rapidly, especially relating to climate change. At present Pharming does not meet existing, or proposed, thresholds for mandatory disclosures for international sustainability regulations, such as those of the European Union (EU) or Securities and Exchange Commission in the USA.

Throughout 2025, we continued to leverage the work already invested over the past three years and continued to shape our sustainability program towards delivering the benefits of sustainability, beyond compliance. We remain dedicated to upholding an impactful (materially and financially) sustainability program that is consistent with our company's strategy, vision and mission and size. We also improved our disclosures on multiple material topics and we will continue to investigate opportunities for further improving our reporting to our stakeholders. Our progress over the previous three years is summarized in the table on the right, and follows a maturity journey of ‘definition, integration, and implementation’.

As we look forward to 2026 and beyond, we endeavor to bring more metrics to our performance measurement in sustainability, more accurate data, further improved collaboration internally and externally and better communicate how we deliver on sustainability topics.

2023	2024	2025
Definition	**Integration**	**Implementation**
• Defined high level plan for developing Sustainability strategy and function • Stakeholder analysis completed • Double Materiality Assessment completed • Gap assessment and organizational readiness analysis completed • Integration approach defined, with prioritization	• Implemented sustainability governance • Deployable roadmap for material topics developed • Alignment with corporate values and mission • Board approved metrics for mandatory topics and target setting for Climate Change • Designed processes and internal controls for reporting	• Material topics maintained from an updated DMA • Monitoring of and adaptation to regulatory requirements • Defined and implemented measurement methodologies for key metrics on Climate Change and Animal Welfare • Emissions data collection continued and Scope 1 and 2 decarbonization actions progressed • Tracking and reporting approach of animal welfare incidents unified internally

Our stakeholders

We recognized five main stakeholders that Pharming should engage with closely within the context of our sustainability program:

- **Patients**
  Patients are the most important stakeholders for Pharming receiving our healthcare services, reflecting Pharming's purpose to serve the unserved rare disease patients.
- **Healthcare professionals**
  Healthcare professionals are also key stakeholders for Pharming achieving optimal healthcare and building trust.
- **Pharming employees**
  Recognizing employees as key stakeholder for any organization is essential for building and further shaping a sustainable organization.
- **Pharming management**
  Pharming management is an important stakeholder because of their decision-making authority and their role in driving innovation and adaptation within Pharming. Their involvement and support are critical for Pharming's success and sustainability.
- **Investors**
  Investors are essential for maintaining financial stability, driving growth, and creating sustainable long-term value for all stakeholders.

These stakeholders have a significant impact on Pharming's sustainability program and strategy, and Pharming has a significant impact on these stakeholders. Further information on our ESG stakeholder dialogue policy can be found on our website.

Our material topics

During 2024, Pharming conducted a double materiality assessment (DMA) relating to our operations and value chain. We identified and prioritized environmental, social, and governance matters that are most relevant for Pharming.

These matters are summarized in the table below and reflect (i) Pharming's most significant impacts on people and the environment, and (ii) the most significant sustainability-related risks and opportunities affecting Pharming.

In line with our commitments last year, we conducted the same process for our operations and value chain in 2025. Some key considerations were our supplier base, our business activities, our stakeholders, and latest policy and scientific developments. Whilst we recognize changes that have occurred, such as the acquisition of Abliva AB, these have not resulted in significant changes to our operations or value chain, across environmental, social and governance topics. Therefore, we identified no significant changes to our material topics or thresholds to include new topics and concluded that the results of the 2024 DMA remain valid, and remain the focus of our sustainability program.

As Pharming continues to grow and evolve, we will likely need to update our double materiality assessment more frequently to reflect more frequent and significant updates to our operations and value chain. However, at this stage in our maturity, we acknowledge that we need to find a balance between identifying or assessing issues and making improvements on the most important topics already identified.

With this in mind, we will update our stakeholder dialogue policy in 2026 to reflect a simplified double materiality assessment process and stakeholder dialogue approach, that we believe will still capture any material changes whilst allowing us to focus on action with the resources available. The simplification will be a shift to biennial frequency for conducting our full double materiality assessment, with alternating years using a simplified approach that uses the expertise of the ESG Steering Committee to identify any significant changes, based upon qualitative assessments.

Theme	Material topics
Environmental	Climate change
Social	People and Culture
	Patient safety and product quality
	Access to products and services
Governance	Business ethics and human rights
	Animal welfare

Connection to our strategy

Pharming's strategy is presented in the section of this annual report titled Our strategy. Our Sustainability goals and objectives are closely related to our company's overall strategy, purpose, vision and mission. We aim to minimize the risk impact of Pharming's activities toward our stakeholders, and to identify and manage sustainability risks that could be an issue for Pharming. Secondly, we aim to make positive impact and capture opportunities that are mutually beneficial to Pharming and our stakeholders.

Our core values are also the foundation of our sustainability program. We put patients at the heart by following through on climate and health, we act with urgency by setting science-based targets, we make it simple in our communication with stakeholders, and we get it done, reporting on our achievements transparently.

It strengthens our company when we manage impacts, risks and opportunities of our material topics effectively. By embedding sustainability into our strategy, we create long-term value for our stakeholders.

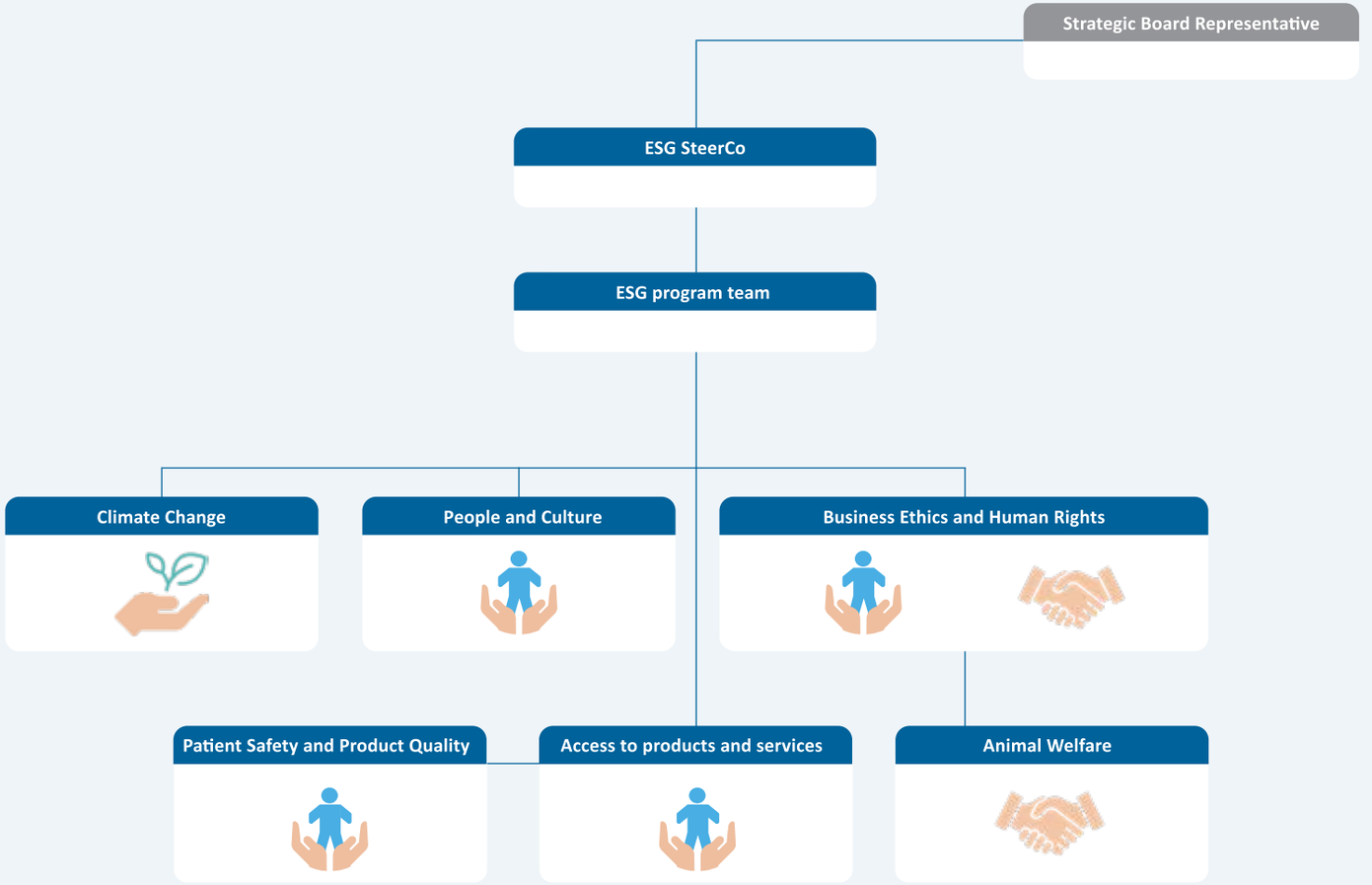
Sustainability governance

Pharming's sustainability program is managed by our sustainability Lead, who reports to the ESG Steering Committee on progress, issues and risks. The ESG Steering Committee, with three members of the Executive Committee (CLCO, CFO and COO), provides advice and oversight in quarterly meetings to the Sustainability Lead to ensure the program achieves its aims in synchronization with other strategic and business goals. The sustainability program team is represented in the ESG Steering Committee by the Sustainability Lead, who monitors and coordinates the activities of the six working groups.
The Board of Directors designated one of its non-executive members as Strategic Board Representative, who acts as the ultimate sponsor of the sustainability program.

Throughout the year 2025 this governance structure was applied and is also intended to be retained for the year 2026. Without Corporate Sustainability Reporting Directive (CSRD) compliance, it is not a regulatory necessity to have such a governance structure implemented, but we see the value in delivering on sustainability beyond compliance, and to ensure we deliver we need strong governance. Whilst we have streamlined the governance process, we do not lessen the cross-functional transparency and accountability that our governance structure affords us, maximizing our potential for improvements and minimizing our and our stakeholders' risks.

Additional information regarding the overall corporate governance at Pharming can be found in the section of this report titled Corporate Governance.

Governance structure for sustainability



Environmental

Climate Change is a global environmental issue, and at Pharming we acknowledge that we have a role to play in abating global greenhouse gas (GHG) emissions and reducing our environmental impact through our direct and indirect operations. We recognize that as a company that intends to continue a growth path, delivering on our vision and commitment to serve the unserved rare disease patients, we will need to ensure we can achieve this with low emission trajectories. It is a challenge that is not unique to our company, but it is nonetheless a challenge that requires commitment and collaboration across Pharming and our value chain, investment in decarbonization and a shared value perspective that leads to effective decision-making for Pharming and our stakeholders.

We have been on a journey since 2023, to understand which activities in our business and value chain generate emissions, and how we can improve the measurement of those emissions and reduce these. In 2025 we have developed a solid understanding of our Scope 1 and 2 emissions, and how we can reduce these emissions in line with the science-based targets that we have adopted.

Our Scope 3 emissions have been measured across all material GHG emissions Scope 3 categories, however, there is still a degree of uncertainty regarding the calculations made, whether that be as a result of spend-based calculations, or emissions factors that are not specific to our operations and value chain.

This is part of our journey, dealing with uncertainty about emissions and climate change. Considering this uncertainty, to ensure we remain aligned to our mission and values, we exercise the precautionary principle, as is best practice, in calculating our emissions. For example, when using spend based emissions factors we use the supply chain emission factors with margins, even if in some cases transport and distribution are included in our purchased goods or services.

Our emission calculation methodology was developed during 2025, in accordance with the principles and guidance of the Greenhouse Gas Protocol (GHGP) and guidance from other standard-setting organizations such as the Science Based Targets initiative (SBTi). We will continue to maintain alignment with these frameworks going forward.

In 2024, Pharming adopted the following Climate targets, which are in alignment with the goals of the Paris agreement. We stand by these targets and can confirm that the baseline year we have established is an average of 2022-2024 emissions inventories. We set a multi-year baseline due to the early stage of our emissions data maturity combined with our growth path as a company, using this approach enables us to reflect our business activity fairly and accurately ensure the scientifically calculated reductions remain possible alongside anticipated business growth.

Short-term targets:

- Reduce absolute scope 1 and 2 GHG emissions 42% by FY2030 from a FY2022-2024 base year
- Reduce absolute scope 3 GHG emissions by 25% by FY2030 from a FY2022-2024 base year

Long-term targets:

- Net Zero emissions across Scope 1, 2 and 3 in 2050

In 2025 we recognized the need to implement emission management software to better understand our scope 3 data and enable us to manage the emissions of our supply base in a cost effective and scalable manner. The new software in 2026 may bring changes to our emissions profile, through improved estimations. If any updates to our baseline are required it will be carried out in accordance with best practice from the Greenhouse Gas Protocol, and in line with our own emissions calculation methodology that can be found on our website. Any changes to our methodology will be clearly and transparently communicated in next year's Annual Report.

Emissions profile

We are proud to be able to share the first publication of our first greenhouse gas emissions inventory, using the Greenhouse Gas Protocol as our reporting framework. The profile covers the previous four years of emissions at Pharming. Like many organizations we have a significant proportion of our emissions in Scope 3. This proportion has stayed relatively stable over the past four years and reflects our historic and current business strategy and operations accurately and fairly.

Action plan and progress to date

In 2025 we conducted a review of historic actions taken to reduce GHG emissions, potential actions and actions being implemented, developing an understanding of how our emissions have changed over time, and where we can reduce our emissions today and in the future.

We intend to build on our action plan, and accelerate our decarbonization in the future, in a cost-effective manner and within regulatory limitations whilst allowing our company to continue on its path for growth. A clear trajectory will be developed with new software-based tooling in 2026.

With these actions we believe we can 'decouple' our emissions profile from our financial performance, enabling a sustainable and prosperous future for Pharming and our value chain that continues to serve our vision and mission. We have evidenced this trend already, with our significant growth in sales, and a year-on-year reduction in emissions.

Scope 1

In scope 1 our emissions originate principally from stationary combustion (natural gas in facilities) and mobile combustion (fleet vehicles) sources, with minimal fugitive and process emissions identified. We are investigating how we can transition to a low carbon fleet across the Company, taking into account local contexts on energy options, public transportation systems and our employees needs.

For our emissions from our facilities, as tenants of the majority of our facilities, we are working with the property owners to implement improved energy data monitoring, via smart metering. Furthermore, we are investigating ways to reduce natural gas usage via energy efficiency measures such as improved insulation and ways to ‘green’ our sites via

Measured in Tons of Carbon Dioxide equivalent (tCO^2e)

Emission categories[†,‡,§]	2025	2024	2023	2022
Total Scope 1 Emissions	925	1.094	1.117	865
Total Scope 2 Emissions (location-based)	461	490	535	547
Total Scope 2 Emissions (market-based)	282	398	436	326
Total Scope 3 Emissions	15.440	16.183	16.442	13.519
Total Emissions (market-based)	**16.647**	**17.675**	**17.995**	**14.710**

[†] Environmental data for the current year is based on actual performance data from January to October, with estimates for November and December, unless indicated otherwise. Any significant deviations from actuals data against these estimates will be restated for 2025 in our sustainability report the following year. 2022, 2023 and 2024 reflect full year actuals data.
[‡] Data from the Pharming acquisition, Abliva, is included from the date of acquisition in February 2025.
[§] Pharming discloses Scope 3 emissions categories that are considered material in 2025. Further information can be found in our methodology documentation.

electrification and renewable energy contracts. Out of six sites we have three sites with natural gas consumption, one site with district heating and two with all energy via electricity, requiring slightly different approaches.

Scope 2

In scope 2, we have a similar aim as in scope 1 with regards to improved energy efficiency, as a key strategic lever for meeting our targets, alongside switching to renewable or low carbon energy sources.

Regarding energy efficiency, we have been working hard on embracing the principle of sufficiency in our operations. With colleagues in our Research and Development laboratory in Leiden demonstrating progress by achieving our first MyGreenLab certification in 2025, achieving the top grading of 'Green'. The certification covers a broad range of environmental sustainability practices, from waste management to energy usage and the circular economy. We will continue to share these best practices across our other operations sites in the coming months and years, further improving our environmental sustainability performance .

Our progress on scope 2 emission reductions has been significantly strengthened by switching to renewable electricity contracts at one of our sites in 2024 and a further two sites in 2025. We are working hard with our facilities partners to make this transition at the two remaining sites, with the intention of having 100% renewable energy at all of our facilities.

Scope 3

A significant proportion of our emissions exist in Scope 3, including the products and services we purchase. Consequently, we recognize that supplier collaboration will be key to ensuring we can reduce our climate impact in line with our adopted Science Based Targets.

We have refined our emissions calculation methodology to ensure it is consistent, transparent, and scalable. Whilst our sustainability reporting in 2025 is on a voluntary base and not subject to audit, we are committed to maintaining high-quality data that supports credible and future-proof climate disclosures. Alongside these improvements, we continue to enhance the accuracy of the GHG inventory to ensure our targets remain both realistic and achievable.

Lastly, we are working with internal and external stakeholders to develop and further evaluate the impact of our decarbonization initiatives, prioritizing them for their emission abatement potential, financial impact to Pharming, and ease to implement. We engaged several suppliers in 2025, prioritizing our highest emission suppliers, and have begun discussions on how we can better measure and reduce our emissions with them.

With these ongoing improvements we are preparing ourselves for a low carbon and sustainable operation at Pharming and in our value chain, whilst ensuring we can continue our mission of serving the unserved rare disease patient.

Eli living with APDS

Social

Pharming is continuing its work on several material topics in the social pillar, addressing our key stakeholders of our own employees, and workers in our value chain.

People and culture

In 2025, we adapted our approach in line with regulatory developments, and continued to deliver improvements on the social dimension of sustainability at Pharming.

Our approach

Pharming's success depends on attracting and retaining specialized biotech talent who deliver results with impact. Our values — We put patients at the heart, We act with urgency, We make it simple, We get it done — define how we work and what we reward. We foster a high-performance environment where employees from diverse backgrounds contribute meaningfully, supported by a culture of recognition that celebrates strong performance against stretched objectives. The Dutch Works Council, established in 2023 with nine elected members across all departments and locations, formalizes employee voice in company decision-making through structured dialogue with management.

Employee training and skills development

We invest in continuous professional development to build the specialized expertise required in biotech. In 2025, we simplified our performance management approach to create clearer links between corporate objectives and individual goals. Three formal reviews annually, supplemented by ongoing feedback conversations, keep development on track and reward strong performance.

Employee engagement

In 2025, we introduced a "Work, Grow, Thrive" framework built around nine moments that matter in the employee experience: how employees contribute and create value (Work), develop capabilities and advance (Grow), and sustain wellbeing and build connections (Thrive).

We use multiple channels to understand employee experience and drive engagement. Our annual pulse engagement survey provides enterprise-wide insights into what's working and where we need to improve. Performance conversations occur three times annually and serve as structured touchpoints for feedback and development dialogue. Together, these mechanisms create regular opportunities for employees to share their perspectives and for leadership to respond.

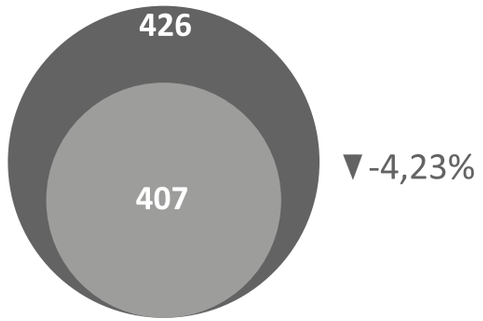
As we strengthen our people practices, we're focused on ensuring these listening channels translate into meaningful action that improves the employee experience across all nine moments.

Employee statistics

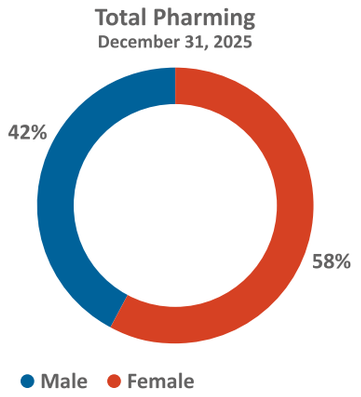
Throughout 2025 we have undergone several organizational changes, reflected in our employee statistics. These changes put us in good stead to continue our mission at Pharming in the coming years.

Employee headcount at the end of the year

407 (2024: 426)



Gender representation



Headcount by region

	2025	2024
The Netherlands	215	231
Australia	4	2
France	14	14
Germany	8	7
Italy	2	2
Spain	1	1
Turkey	1	1
United Kingdom	20	16
United States	140	152
Sweden	1	-
Norway	1	-
Total	**407**	**426**

	2025	2024
Research and development	131	139
General and administrative	115	133
Marketing and sales	112	111
Production	49	43
Total	**407**	**426**

Human rights

Pharming operates today in areas of low risk for human rights issues, but as we grow as an organization, entering new markets we recognize that we need to be prepared to identify and mitigate any risks or issues that could arise.

Pharming recognizes that any potential impact on workers in the supply chain (including contractors and suppliers) and their exposure to forced labor and child labor should be identified and addressed to rectify an issue and prevent it from occurring again. In 2024, we published our Modern Slavery and Human Trafficking Statement, which can be found on our corporate website.

We have also drafted a Human Rights statement that is under internal review. We recognize that these statements are not the end of our improvement path. We are investigating how we can use sustainability software and platforms, for example EcoVadis, to support us with identifying and managing any potential human rights issues in our supply chain today or in the future, to demonstrate how we are enforcing these statements.

Patient safety and product quality

Consistent with our company's purpose and embedded in our core values is the premise that we put patients at the heart of everything we do. This reinforces our commitment to patient safety and product quality. Ensuring the quality of products and patient safety is an objective we commit to by developing robust production processes and delivering high quality products. Risk assessments are inherently part of the regulations, the clinical programs, the regulatory review process, and our internal processes and procedures.

Good Clinical Practices (GCP), Good Pharmacovigilance Practices (GVP), Good Manufacturing Practices (GMP) and Good Distribution Practices (GDP), which, along with the numerous national and regional regulatory laws and standards, are the foundation of our Quality Management System policies and procedures.

Our Quality Assurance department is involved in all quality-related matters, reviews and approval of quality-related documents and conduct of internal audits to monitor the compliance with the principles of GMP, GDP, GVP and GCP and Pharming's policies and procedures. The Health and Youth Care Inspectorate (IGJ) conduct periodic inspections of all medicine manufacturers in the Netherlands to assess whether these comply with GMP guidelines. In June 2025, following an inspection, the IGJ concluded that the processes used by Pharming are compliant with Good Manufacturing Practice (GMP). To ensure we maintain the high standards achieved last year, we ensure continuous improvement of our processes, our products and Pharming as a whole.

We carefully select and manage a supplier and vendor network which includes Contract Manufacturing Organizations (CMOs), Wholesalers and Distributors, Contract Laboratory Organizations (CLOs), Clinical Research Organizations (CROs), Clinical Sites, and Pharmacovigilance Service Providers. Our external audit program, and supplier assessment and (re)qualification processes together with the use of Master Service Agreements, Quality Assurance Agreements and Safety Data Exchange Agreements underpin this and ensure our commitment to patient safety and product quality is maintained when we outsource. As part of our responsibility as a clinical trial sponsor, trial sites and investigators are assessed prior to selection, monitored throughout the trial by our qualified external Clinical Research Organizations, and audited by Pharming's Quality Assurance department. This ensures the ethical and safe conduct of the trial, protecting our patients' rights and safety.

Patient safety

To manage, support and fulfill our obligations and commitment to the safety of our patients, Pharming has a Global Pharmacovigilance department. This is responsible for the global safety surveillance of Pharming's products through the monitoring of safety reports, which are received worldwide from unsolicited and solicited sources. The department works with qualified contract partners who perform delegated pharmacovigilance activities in their territory.

All safety reports, for product complaints and adverse events/ safety concerns, are entered into our global safety databases to ensure we can assess the full safety profile of our products and respond to any safety signals accordingly. We follow all applicable laws for providing reporting routes associated with pharmacovigilance and train our employees and contract partners in the required processes and routes to report safety concerns to us, in our onboarding program and regularly throughout the year through our training program, and with events such as our 'Compliance Day' held in November 2025

Our pharmacovigilance system and its components are fully described in our Pharmacovigilance System Master File (PSMF). To maintain its accuracy and relevance, we mandate a review and update at least once per quarter, and we successfully completed this for 2024.

Further information can be found on our website as to how to report any adverse events or product safety concerns.

Product quality

The processes, from product manufacturing to the delivery to the end user, are established and maintained to ensure product quality.

The manufacture, according to defined specifications, for all relevant materials, intermediates and final products, and the quality control testing are outsourced to qualified and licensed CMOs and CLOs. GMP is applied throughout. Critical process parameters and all analytical measures are validated and re-validated if major changes occur. Production equipment, utilities and instruments are well maintained, and critical equipment and instruments are calibrated and qualified.

The release of finished product is completed by the Pharming Qualified Person (QP). The QP checks that the GMP quality system is adhered to during all production steps and that the manufactured product meets the required specifications.

Transport of the packaged drug product to wholesale license holders and marketing authorization holders is performed by a qualified transporter. The distributors are responsible to ensure that counterfeit control measures are taken and that product recipients are authorized to receive medicinal product.

Pharming has processes in place to record, investigate and resolve product quality complaints, to evaluate and initiate recalls should they be required, to describe the process for returned products and to ensure no falsified product can be introduced in the Pharming supply chain.

Access to products and services

Pharming serves the unserved rare disease patients through our innovative medicines. It is our mission to increase access to patients, amongst others through license extension indications to pediatric populations, and exploring other potential disease indications.

We work with government, payers and regulators to provide as broad and sustainable access to our medicines, across the globe as we can. However, changing regulatory landscapes and policies can heavily impact our ability to provide our medicines to all across the globe. Nonetheless, our ambition to grow our positive impact through providing innovative medicines to rare disease patients globally, remains steadfast. A specific example of how we continue our work on medicine access is by providing our products to eligible patients in the U.S., who qualify for the copay savings program or our Patient Assistance Program in the event coverage cannot be altered.

Patients can also access our products through participation in one of our clinical trials. All Pharming sponsored clinical trials are approved by regulatory authorities and ethics committees and conducted in strict accordance with Good Clinical Practices.

Further information on our authorized medicines can be found in the Commercial portfolio review section of our Annual Report.

Governance

Pharming places governance and business integrity at the core of our culture, as it sets the foundation of trust and is, therefore, an essential part of the way we work in a highly regulated industry. We firmly believe that successful, sustainable business is ethical business. In the following section we share how we are living up to good governance practices, across business ethics and animal welfare in particular.

Business ethics

We expect all Pharming management, employees, officers and contractors to conduct any business related to Pharming according to our principles and ethical standards, as described in our global Code of Conduct that can be found in the Corporate Governance section of our corporate website. The Code of Conduct was reviewed and updated in 2025.

Pharming has a whistleblower policy in place, referred to as Alert Reporting and Investigation Procedure, which can be found in the Corporate Governance section of the Company's website. This Alert Reporting and Investigation Procedure describes the reporting and investigation procedures for suspected breaches of the Pharming Code of Conduct, policies, or any law or regulation applicable to the Pharming organization. The procedure applies to all Pharming entities in all countries. Pharming has a strict non-retaliation policy.

Based on our solid long-term strategy and business integrity framework, we have introduced new and enhanced policies in 2025, accompanied by more operational procedures, covering a variety of corporate and healthcare compliance matters.

The most salient improvements are new policies and standard operating procedures in respect of social media, sanctions and export controls, medical engagement, grants and donations and cross-border meetings and events.

The introduction of these policies and procedures has been accompanied by a training program, targeted at audiences selected according to a risk-based approach, ensuring that those who need to know follow the trainings. Furthermore, we ran our Compliance Day event in 2025, which brought together over 200+ colleagues from across the organization to communicate updates to policies, broadening and deepening our culture of business integrity.

To support the reporting of suspected breaches of the Pharming Code of Conduct, policies, or any law or regulation applicable to the Pharming organization, a Speak Up! framework was launched globally in 2025, providing multiple channels that can be used for reporting and ensuring strict confidentiality, strengthening our ability to identify and respond to any compliance issues.

Regarding improvements on the transparency of how we identify and mitigate any potential bribery and corruption issues, our internal methodology for anti-bribery and anti-corruption related activities was formally endorsed by the Pharming Board of Directors in 2024. Following this, we can share for 2025 the status of reports on bribery and corruption at Pharming below.

Corruption and bribery reported by Pharming	Number of convictions in 2025	Total amount of fines in 2025
	0	0

We take privacy and data protection seriously. Compliance with the General Data Protection Regulation (GDPR) and other privacy regulations remains a priority for Pharming.

In 2025, Pharming maintained a structured and risk-based privacy and data protection program aligned with the GDPR, other privacy regulations and applicable local laws, overseen by an independent Data Protection Officer and supported by a dedicated Privacy Team.

Key focus areas included strengthening governance, updating and maintaining Records of Processing Activities across core functions and conducting Data Protection Impact Assessments for higher-risk processing activities such as Pharmacovigilance, Early Access and Compassionate Use programs, third-party platforms, and emerging technologies.

We actively monitored and managed privacy risks. Seven personal data breaches were recorded in 2025; most were assessed as low risk and therefore notification to the relevant Data Protection Authority and affected data subjects were not required. One high-risk incident was reported to the Dutch Data Protection Authority in accordance with GDPR requirements, and appropriate mitigation measures were implemented.

Privacy awareness and training activities were reinforced in the second half of the year through renewed e-learning and company-wide compliance initiatives. Regulatory developments, including cross-border data transfer mechanisms and evolving EU digital legislation, were continuously monitored to ensure ongoing compliance and future readiness.

Animal welfare

Our proprietary transgenic manufacturing technology platform is the foundation upon which we started our company. We have developed a unique and scalable, current Good Manufacturing Practices (cGMP), validated methodology for the production of c1-esterase inhibitor (recombinant human protein). By law, the use of animals to produce (recombinant) therapeutic proteins is only allowed when production methods that do not make use of an animal model are unavailable, as is the case for Pharming as well.

Our manufacturing process utilizes transgenic animals, specifically rabbits, to produce this human recombinant protein in their milk. This process enables the production of the protein in the milk of the animals without the animals being subjected to unnecessary discomfort or being altered in other aspects of their biology.

We raise the rabbits at specialized and regulator approved facilities with high standards of animal husbandry, welfare and security. These facilities further incorporate protections against contamination from the outside environment.

All institutions using animals for research or production of medicinal products must comply with EU and national regulations regarding experimental animals. Before commencing any activity involving animals, a project license application must be approved by the Dutch regulatory ethics committee for the Netherlands, and by the Ministry of Higher Education & Research and the Ethics Committee in Animal Experimentation in France.

We have a comprehensive Policy on the Use of Animals, which not only enforces strict regulatory control over our transgenic biological materials and animals, with regard to the environment and particularly the continuous well-being of our animals, but also emphasizes our commitment to treat animals respectfully, refining procedures and reducing discomfort and stress as much as possible.

Pharming ensures that the “3 R principles” (Reduction, Replacement and Refinement) as outlined by EU legislation are considered prior and during the course of an experiment (such as the routine production of therapeutic protein), which includes the care, handling and treatment of animals meeting their species-specific needs as much as possible.

In 2025, we advanced our commitment to animal welfare monitoring by building on the groundwork laid in 2024. Following the establishment of a dedicated working team and the endorsement of an entity-specific metric by the board in 2024, detailed data collection on animal welfare issues commenced in 2025.

Issues that may have affected the welfare of our rabbits were reported to the respective animal welfare body structures at Pharming in each country, in line with European legislation and the Instantie voor Dierenwelzijn (IvD) in the Netherlands and Structures chargées du Bien Être des Animaux (SBEA) in France. Each Animal Welfare Body consists of a minimum of one qualified scientist, two biotechnicians and the designated or the company veterinarian.

In 2026, the baseline data will be evaluated to enable consistent tracking and reporting and if needed the established metric will be refined. As part of a broader effort to prevent any re-occurrence of issues we have brought together the teams across our sites for a lessons-learned session on animal welfare in 2025.

We received one outstanding critical issue from the competent Dutch regulatory authority, the Nederlandse Voedsel- en Warenautoriteit (NVWA) the Dutch Food and Consumer Product Safety Authority. A Corrective and Preventative Action Plan (CAPA) is in place and actions are being executed.

Financial performance

Outlook 2025

At the beginning of 2025, we announced performance guidance for the year and anticipated:

- Total revenues between US$315 million and US$335 million (6% to 13% growth), with quarterly fluctuations expected.
- Total operating expenses not to exceed the prior year pre-Abliva impact, and a preliminary estimate of US$30 million in Abliva-related operating expenses, including research and development and non-recurring transaction and integration expenses.
- Significant progress finding additional APDS patients in the U.S., supported by VUS resolution efforts and subsequently converting patients to paid Joenja® (leniolisib) therapy.
- Increasing ex-U.S. revenues for leniolisib — driven by funded access programs and commercial availability in the U.K.
- Progress towards additional regulatory approvals for leniolisib for APDS patients 12 years of age or older, and submitting regulatory filings in Japan and for pediatric label expansion in key global markets.
- Advancing the two ongoing Phase II clinical trials in PIDs with immune dysregulation to significantly expand the long-term commercial potential of leniolisib.
- Advancing the ongoing pivotal FALCON clinical study for napazimone (KL1333) in mitochondrial DNA-driven primary mitochondrial diseases.
- Continued identification of value-accretive business development and licensing opportunities to develop our portfolio and pipeline.

No further specific financial guidance for 2025 was provided.

Financial review 2025

RUCONEST® growth continued in 2025, with revenue for the full year increasing by 26% to a record US$317.9 million, reflecting the benefit of a larger patient and prescriber base in the U.S. market. Unit sales volume in the U.S. increased by 20% for the full year.

Joenja® revenue for the full year 2025 increased by 29% to US$58.2 million, reflecting a 25% increase in patients on paid therapy in the U.S. and increased demand in international markets. The U.S. market contributed 86% of 2025 revenues, while the EU and Rest of World contributed 14%, driven by strong patient uptake in the U.K. following the April 2025 launch and purchases under government-supported access programs.

We completed the acquisition of Abliva, finalized integration activities, and successfully started the second wave of the pivotal FALCON clinical trial for napazimone (KL1333) in primary mitochondrial disease in 2025.

Also during the year, we significantly advanced our efforts to study leniolisib in primary immunodeficiencies with immune dysregulation beyond APDS and started the Phase II clinical trial for CVID with immune dysregulation.

We demonstrated disciplined cost management, achieving US$25.8 million operating profit in 2025, compared to a loss in 2024 and achieved US$54.7 million net cash flow from operations in 2025, compared to negative cash flow in 2024. These results mark an important inflection point and strengthen our ability to fund growth and long-term investment.

"2025 was a defining year for Pharming and reflects the focus and discipline our teams have brought to executing our strategy. We outperformed revenue guidance and delivered strong financial performance, with total revenues up 27%, driven by continued RUCONEST® growth and rising demand for Joenja® (leniolisib)."

Fabrice Chouraqui,
Chief Executive Officer
and Executive Director

Financial review

Amounts in US$ million except per share data	2025	2024	% Change
Consolidated Income Statement			
Revenues	376.1	297.2	27%
Gross profit	330.6	261.8	26%
Operating profit (loss)	25.8	(8.6)	400%
Profit (loss) for the year	2.5	(11.8)	121%
Consolidated Balance Sheet			
Overall cash & marketable securities	181.1	169.4	7%
Share Information			
Basic earnings per share (US$)	0.004	(0.018)	122%
Fully-diluted earnings per share (US$)	0.004	(0.018)	122%

In 2025, Pharming revenues increased by 27% to US$376.1 million. Operating profit improved from a US$8.6 million loss in 2024 to a US$25.8 million profit in 2025. Similarly, net profit improved from a US$11.8 million loss in 2024 to a US$2.5 million profit in 2025.

This section will further elaborate on Pharming's financial performance in 2025.

Income statement

Revenues and Gross Profit

Total revenues for 2025 grew by 27%, reaching US$376.1 million, compared to US$297.2 million in 2024. Total RUCONEST® revenues were 26% higher at US$317.9 million, compared to revenues of US$252.2 million for 2024. Joenja® revenues amounted to US$58.2 million in 2025, a 29% increase compared to revenues of US$45.0 million for 2024. This increase was primarily driven by a 37% increase in volume.

Cost of sales increased by 29% from US$35.4 million in 2024 to US$45.5 million in 2025. Cost of inventories recognized as expenses in 2025 amounted to US$32.0 million compared to US$25.6 million in 2024, primarily due to the higher unit sales volume. The remainder of the increase in cost of sales in 2025 stems primarily from the higher royalty payments to Novartis on Joenja® sales of US$5.8 million (2024: US$4.9 million) and the first sales milestone payment for Joenja® of US$5.0 million (2024: US$— million), partially offset by lower impairment charges on inventory of US$2.7 million (2024: US$4.8 million).

Gross profit increased by US$68.8 million, or 26%, to US$330.6 million for the year 2025. The primary driver for this increase was higher sales volumes of RUCONEST® and Joenja®.

Other income

Other income increased to US$6.5 million compared to US$2.2 million in 2024. Other income in 2025 was supported by the gain on the early termination of the DSP facility lease at Pivot Park in Oss, the Netherlands of US$3.9 million.

Operating Profit (loss) and Other Operating Costs

The operating profit amounted to US$25.8 million compared to an operating loss of US$8.6 million for the prior year. Adjusted to exclude US$10.3 million of non-recurring Abliva acquisition-related expenses (of which US$8.1 million is included in General and administrative expenses and US$2.2 million is included in Research and development expenses), US$4.1 million in one-off restructuring expenses, and the US$3.9 million gain on the early termination of the DSP facility lease, the operating profit amounted to US$36.4 million. The improved operating result was primarily driven by an increase in revenues, partially offset by higher operating expenses which include a total of US$29.7 million in Abliva-related expenses, and the first sales milestone for Joenja® of US$5.0 million. Excluding the Abliva-related expenses and restructuring expenses, other operating expenses increased by 2% compared to prior year.

Finance result (net) and share of result in associates

The finance result (net) and share of result in associates amounted to a loss of US$13.0 million compared to a gain of US$0.1 million in 2024. The year-on-year decline was primarily driven by foreign currency losses of US$7.2 million, compared to a gain of US$2.0 million in 2024, resulting from the strengthening of the euro against the US dollar. In addition, interest income declined as the Company reduced its investments in marketable securities during the year. These effects were partially offset by a higher share of results in associates of US$2.4 million.

Income tax expense

Income tax expense increased from US$3.3 million for the year ending December 31, 2024, to US$10.3 million for the year ending December 31, 2025. This tax expense mainly results from the profits of Pharming in the U.S. being taxed against a U.S. Federal and State combined tax rate of 27.96%, while the losses in the Netherlands only partly result in an offsetting tax credit, as the share-based compensation expenses and losses in associates are generally non-deductible based on Dutch tax law.

Net result for the year

The Company had a net profit of US$2.5 million in 2025, compared to a net loss of US$11.8 million in 2024.

Balance sheet

Intangible assets

In 2025, intangible assets increased by US$74.5 million, from US$61.0 million in 2024 to US$135.5 million in 2025. The significant year-on-year growth is primarily attributable to the acquisition of Abliva AB, including the recognition of the intellectual property related to the napazimone (KL1333) program. The amortization relates to regular amortization of software, the RUCONEST® licenses (U.S. and EU) and the Joenja® license. The RUCONEST® license has a remaining amortization period of 12 years for the U.S. and 6 years for the EU. The Joenja® license has a remaining amortization period of 11 years.

Property, plant and equipment

The value of property, plant and equipment decreased from US$7.8 million in 2024 to US$7.2 million in 2025. This decline was primarily driven by regular depreciation (US$2.1 million) and positive foreign currency effects (US$0.8 million), partially offset by capital expenditures (US$0.7 million).

Right-of-use assets

The right-of-use assets increased from US$16.4 million in 2024 to US$16.7 million in 2025. This increase was primarily driven by remeasurements (US$2.4 million) and positive foreign currency effects (US$1.6 million), partially offset by regular depreciation (US$3.5 million) and the subsequent impairment of the remeasurement of the DSP facility at Pivot Park in Oss, the Netherlands (US$0.5 million). The 2025 building remeasurements were related to adjustments in the existing right-of-use assets to account for inflation-related higher lease payments.

Investments

Investments increased by US$4.4 million to US$8.6 million as of December 31, 2025. This increase was primarily driven by the capital contributions made to BioConnection of US$0.7 million, Pharming's share in the net result of BioConnection of US$0.6 million and a fair value increase of US$2.3 million in the preference share in BioConnection, carried at fair value through the statement of profit and loss (FVTPL).

Inventories

Inventories increased from US$55.7 million as of December 31, 2024, to US$64.9 million as of December 31, 2025 mainly as a result of foreign currency effects.

Cash and cash equivalents and marketable securities

Cash and cash equivalents alone increased by US$90.4 million to US$145.3 million as of December 31, 2025. Cash and cash equivalents are managed in combination with the marketable securities position.

The combined total of cash and cash equivalents, together with restricted cash and marketable securities increased from US$169.4 million at year-end 2024 to US$181.1 million at year-end 2025. This increase was primarily driven by the positive operating cash flow of US$54.7 million as well as proceeds from exercise of share-based compensation awards during 2025, amounting to US$19.8 million in positive cashflows for 2025. This increase was primarily offset by purchases of Abliva shares totaling US$68.0 million.

Shareholders' equity

Shareholders' equity increased by US$56.0 million from US$221.1 million for the year ended December 31, 2024, to US$277.1 million for the year ended December 31, 2025. This increase was driven by transactions recognized directly in equity relating to share-based compensation and exercised options (totaling US$23.7 million) and the other comprehensive income of US$29.1 million. The other comprehensive income was primarily driven by currency translation differences.

Convertible bond

The convertible bond position has increased by US$15.7 million to US$98.1 million at year-end 2025, from US$82.4 million as of December 31, 2024. This increase was mainly driven by foreign currency effects of US$11.0 million resulting from the strengthening of the euro against the US dollar.

Lease liabilities

Lease liabilities decreased by US$12.2 million, moving from US$29.9 million as of December 31, 2024, to US$17.7 million as of December 31, 2025. This decrease was primarily driven by disposals of lease liabilities, amounting to US$13.7 million of which the main contributor was the early termination of the DSP facility lease at Pivot Park in Oss, the Netherlands.

Trade and other payables

Trade and other payables increased by US$39.3 million, moving from US$66.6 million as of December 2024, to US$105.9 million as of December 31, 2025. This increase was driven by the fee for the early termination of the DSP facility lease at Pivot Park in Oss, the Netherlands of US$12.3 million, the acquisition of Abliva AB resulting in an additional US$7.0 million in Trade and other Payables, as well as the first sales milestone for Joenja® of US$5.0 million.

Going concern

Pharming's 2025 financial statements have been drawn up on the basis of a going concern assumption.

The 2025 year-end combined total of cash and cash equivalents, together with restricted cash and marketable securities of US$181.1 million is expected to provide sufficient liquidity to fund the Company for more than twelve months from the date of this report.

During 2025, operating cash flows improved strongly compared to 2024, reflecting the continued strength of RUCONEST® revenues, sustained Joenja® growth, improved operational efficiency, and disciplined cost management. This improvement in operating cash generation further supports the Company's assessment that it holds adequate liquidity to meet its obligations as they fall due.

Following the completion of the acquisition of Abliva AB in February 2025, the Board of Directors anticipates continued investment in the development of napazimone (KL1333) and related clinical programs, as well as ongoing preparations for the commercial launch of leniolisib (Joenja®) outside the United States, which began in 2024 and is expected to further expand in 2026. These strategic investments are expected to continue to exert pressure on profitability in the near term.

Consequently, Pharming's combined cash, restricted cash, and marketable securities may decline during 2026 as the Company advances its long-term growth strategy.

Revenues from Joenja® are expected to increase from 2026 onwards as additional regulatory approvals are obtained and commercialization expands. The Company remains confident in the robustness of RUCONEST® sales and the strengthening of its pipeline, although no assurances can be given regarding the timing or magnitude of future profitability.

If additional capital is required, financing options may include equity issuance, expansion of the existing convertible debt, new debt financing, or a combination thereof. Any equity raise may dilute existing shareholders' interests. The Company does not currently foresee a need to raise capital to support its ongoing operations; however, it may do so to support acquisitions or in-licensing opportunities if terms are favorable and aligned with shareholder interests.

Based on its review of the financial position, the strong improvement in operating cash flows, cash flow forecasts, and principal risks, the Board of Directors concludes that Pharming has adequate resources to continue as a going concern for the foreseeable future.

“Living with APDS has impacted my finances a lot. Because I get sick so easily, I have to take sick days leave without pay in order to get better so I can return to work. That takes a huge chunk out of my paycheck sometimes.”

Patient living with APDS

Outlook 2026

For 2026, the Company provided performance guidance and anticipates:

- Total revenues between US$405 million and US$425 million (8% to 13% growth), with quarterly fluctuations expected.
- Total operating expenses between US$330 million and US$335 million (6% to 8% growth), including US$60 million incremental R&D expenses to advance the pipeline and US$9 million structural G&A cost reductions based on the plan announced in October 2025.
- Continued RUCONEST® growth, and significant and accelerating Joenja® U.S. and ex-U.S. growth.
- Progress towards additional regulatory approvals and commercial launches for leniolisib for APDS patients 12 years of age or older and for pediatric label expansion in key global markets.
- Top-line data readouts for the two ongoing leniolisib Phase II clinical trials in PIDs with immune dysregulation to expand the asset's addressable patient population.
- Completion of enrollment in the pivotal FALCON clinical study for napazimone (KL1333) in mitochondrial DNA-driven primary mitochondrial diseases.
- Enhancing capital allocation to drive growth and build a leading rare disease company.
- Continued focus on potential acquisitions and in-licensing of clinical stage opportunities in rare diseases. Financing, if required, would come via a combination of our strong balance sheet and access to capital markets.

No further specific financial guidance for 2026 is provided.