Pharming Group N.V. 4Q/FY 2025 Results Call

March 12, 2026

Rewatch the webcast
Download presentation slides
View press release

CORPORATE PARTICIPANTS
Fabrice Chouraqui – Chief Executive Officer
Leverne Marsh – Chief Commercial Officer
Anurag Relan – Chief Medical Officer
Kenneth Lynard – Chief Financial Officer

CONFERENCE CALL PARTICIPANTS
Lucy Codrington – Jefferies
Joseph Pantginis – H.C. Wainwright
Jeffrey Jones – Oppenheimer
Sushila Hernandez – Van Lanschot Kempen
Natalia Webster – RBC
Simon Scholes – First Berlin

Fabrice Chouraqui – Chief Executive Officer:
Thank you, Operator. Good morning and good afternoon everyone. And welcome to our Q4 and full year 2025 earnings call.

I'll be joined on this call today by Leverne Marsh, our new Chief Commercial Officer; Anurag Relan, our Chief Medical Officer; and Kenneth Lynard, our Chief Financial Officer.

Slide 4:
On this call we will be making forward-looking statements that are based upon our current insights and plans. As you know these may differ from future results.

Slide 5:
As you saw in our press release, Pharming ended 2025 on a strong note, operationally and financially.

Total revenues grew by 15% in the fourth quarter of 2025 and by 27% for the full year. Thanks to disciplined cost management, we delivered US$26 million of operating profit in 2025, compared to a loss in 2024.

We also significantly increased our cash position. Operating cash flow came in at US$55 million in 2025, putting our cash position at year-end above that of the end-of-2024 level, before the acquisition of Abliva.

2025 was marked by significant growth of our two commercial assets, RUCONEST® and Joenja®.

RUCONEST® grew 26% year-on-year and by 9% in the fourth quarter. With its efficacy, with its reliability and its rapid onset of action, RUCONEST® is poised to remain an established on-demand treatment option for difficult-to-treat patients in an evolving HAE treatment landscape. Leverne will talk more about RUCONEST® performance and its unique value proposition.

Joenja® grew 29% year-on-year and by 53% in the fourth quarter, fueled by the acceleration of new patients on drug in the U.S., but also increased demand in international markets including in the

U.K., where the drug was launched last spring and in other countries through purchases under government-supported access programs.

Slide 6:
These results underscore Pharming's transformation from a single-asset company into a highly profitable, high-growth biotech with two commercial products and a late-stage pipeline with two programs offering billion-dollar sales potential. We highlighted these programs at our Investor Day last month, offering investors unique insights to our high-value pipeline.

RUCONEST® is the foundation of our portfolio and a reliable cash engine for the future, even in a more crowded HAE on-demand market, given its efficacy profile in the difficult-to-treat patient sub-population.

Joenja® is just at the beginning of its life cycle, with multiple growth catalysts in APDS, international geographic expansion and potential expansion into much larger indications for PIDs/CVID.

Napazimone, which we used to call KL1333, for primary mitochondrial disease is another billion dollar plus opportunity, with the registrational study now well underway. This combination of durable revenue, first-in-disease innovation and an advancing late-stage pipeline positions Pharming well for substantial near-term and long-term value creation.

Let me now turn to our outlook for the remainder of 2026.

Slide 7:
As announced at our Investor Day in February, we expect 2026 revenues between US$405 million and US$425 million this year, representing an 8% to 13% growth, with operating expenses increasing at a slower overall pace even with substantially higher R&D investment in our pipeline to fuel future growth.

We expect continued RUCONEST® growth for the reason mentioned earlier and accelerating Joenja® growth, fueled by the uptake in APDS patients above 12 years in the U.S. and potential future regulatory approval internationally.

Regarding the U.S. pediatric label extension, we've been working to address the FDA request, and we expect to have greater clarity on the resubmission requirements and timeline following the FDA Type A meeting which is now scheduled at the end of March.

2026 is an important year for our pipeline with the materialization of potential value inflection points.

We have now completed the enrollment of two leniolisib Phase II trials for larger prevalence PIDs, and we expect top line data readouts in the second half of this year.

We also expect to complete enrollment in the napazimone (KL1333) pivotal study this year, to be in a position for a data readout at the end of 2027.

We are clearly determined to maintain strong financial discipline to optimize capital allocation on our growth drivers. This is critical as we strive to build an efficient and scalable organization and make Pharming a leading rare, ultra-rare disease company and deliver sustainable value for our shareholders.

Let me now turn to Leverne Marsh for deeper insights on the performance of our commercial portfolio.

Leverne Marsh – Chief Commercial Officer:

Slide 9:

Good day everybody. Let me start with RUCONEST®, where the U.S. business delivered another year of strong and resilient performance.

In 2025, despite the first new wave of treatment options in the HAE market in almost five years, RUCONEST® continued to grow as an essential therapy for patients living with severe, high-frequency HAE attacks.

Our strategy remains consistent, focused on high-attack patients who require fast, reliable on-demand treatment and ensure that we continue to execute against that differentiated value proposition even as new agents enter the market.

For the full year, RUCONEST® delivered 26% global revenue growth and volume growth in the U.S. of 20%, a clear indicator of the strong and durable demand for RUCONEST® in the acute segment.

In the fourth quarter, we delivered 9% global revenue growth versus the prior-year quarter and a 2% volume growth in the core U.S. market, continuing the upward trajectory of RUCONEST®.

As expected, through Q4, we began to observe impact of newly launched therapies for HAE in the U.S. market with some patients trialing and some already returning to RUCONEST®. Despite these competitive dynamics, we welcomed over 60 new enrollments in the U.S. in the fourth quarter, slightly above Q3. RUCONEST® added both new patients and new prescribers in the quarter, underscoring the resilience of our position in the difficult-to-treat patient segment and the clinical trust placed in RUCONEST® in real-world settings.

Slide 10:

That said, the high burden of HAE matters for patients who have high-frequency attacks. Attacks are often unpredictable and potentially life-threatening and symptom severity often escalates within hours.

Importantly, for patients experiencing multiple attacks per month or patients who experience suboptimal responses to other options, dependable rapid relief is not optional, it is essential. And this high-frequency attack segment is precisely where we have seen consistent, durable use of RUCONEST® over time, and where we expect RUCONEST® to remain highly relevant even as new treatments enter the market.

To that end, a meaningful proportion of new patient enrollments in the U.S. are switching to RUCONEST® from other on-demand therapies, further emphasizing the continued need that high attack patients have for an effective, reliable one-and-done therapy like RUCONEST®.

And so when you put this together, the unpredictability of the disease, the high burden carried by certain patient segments, the limitations of some other acute therapies and the consistently strong clinical performance associated with RUCONEST®, the unique value proposition for RUCONEST® remains clear.

Looking ahead, we foresee some pressure on our growth early in the year, but with no change in the need for an effective, rapid-onset, reliable one-dose treatment like RUCONEST®.

With that in mind, let me turn to Joenja®.

Slide 11:
For Joenja®, we delivered a strong fourth quarter, building on the momentum we established throughout the year. Revenue grew 53% compared to the fourth quarter in 2024, reaching US$19.8 million globally.

For the full year, Joenja® generated US$58 million, representing 29% growth year-on-year and demonstrating both sustained utilization from patients and the expanding clinical recognition and treatment of APDS.

In the United States, patient growth remained a central driver of performance. By the end of 2025, we had 120 patients on paid therapy, representing a 25% increase over year-end 2024.

This steady expansion of our treated population reflects strong physician confidence, consistent engagement with patient communities, and a team that executes with discipline and with urgency. Equally important, we made significant progress in broadening the pool of identified APDS patients, one of the most critical leading indicators in an ultra-rare disease.

In 2025, the number of U.S. patients we identified diagnosed with APDS increased by 40, more than double the increase of 18 we saw in 2024. This growth in identified patients with APDS shows that our educational efforts, our diagnostic partnerships and our medical engagement in the U.S. are working.

Outside the U.S., we saw strengthening demand across international markets including a solid first year uptake in the United Kingdom following the launch in April 2025.

We also benefited from government-supported access programs, which allowed us to reach more patients who currently have limited therapeutic options. Taken together, these results give us a strong platform for Joenja® in the years ahead.

Slide 12:
Finally, we expect geographic expansion and the anticipated 4- to 11-year approval in the United States to be meaningful contributors to growth. These two catalysts remain materially important

to increase the number of patients who can benefit from Joenja® and expand the global footprint of the APDS business.

Internationally, we've already demonstrated our ability to execute. In the U.K., where Joenja® launched in April 2025, we have seen solid early uptake and strong engagement from the clinical community. That success reinforces that our teams have the capability, the infrastructure and the strategic focus needed to deliver in new markets.

In the United States, our commercial teams are fully prepared to launch Joenja® for children ages four to 11, pending FDA approval. And importantly, we already have 52 eligible patients identified, and one-third of them are currently on therapy through our early access program ready to transition at approval. This will give us a running start and positions us for early momentum once the label is approved.

Beyond the U.S., our international organization is deeply engaged in progressing regulatory submissions and ensuring that reimbursement discussions can start when approvals are granted across Europe, Japan and Canada. Across these three regions, we have over 80 patients already receiving Joenja® through early access mechanisms, awaiting full regulatory approval and commercial availability. This represents a significant built-in foundation for launch acceleration once those approvals are secured.

And as we're stepping into 2026, we do so with confidence. For Joenja®, we have the right growth catalysts in front of us, and we have an organization that has demonstrated that it can execute launches with precision and continuity. This gives us confidence not just in the next quarter, but in the sustained global expansion of Joenja® over the coming years.

And with that, I'll now hand over to Dr. Anurag Relan, our Chief Medical Officer, who will walk you through our progress across the pipeline and the upcoming development and regulatory milestones.

Anurag Relan – Chief Medical Officer:

Slide 14:

Thank you, Leverne. As we discussed at our Investor Day in February, PI3Kδ is a master regulator of the immune system and imbalances here contribute to immune dysregulation in a number of primary immune deficiencies or PIDs. This understanding serves as the foundation and rationale for our Joenja® (leniolisib) development efforts.

APDS, where Joenja® (leniolisib) is currently approved, is a primary immune deficiency caused by a genetic defect that leads to PI3Kδ-hyperactivation. This results in the dysfunction of the immune system and is characterized by frequent and severe infections and a wide array of immune dysregulation consequences, as you see here.

APDS is a progressive disease and leads to early mortality due to these complications, with unfortunately about 25% of patients dying by the age of 30.

Based on this understanding of APDS, we have two ongoing Phase II proof-of-concept clinical trials evaluating leniolisib in more prevalent PIDs which share unmet medical needs, mechanisms and

disease pathology with APDS. These include the genetically identifiable primary immune deficiencies with immune dysregulation linked to altered PI3K-delta signaling and Common Variable Immune Deficiency, or CVID, with immune dysregulation which is identified independently of genetics. And as Fabrice mentioned, both of these studies have now completed enrollment.

Slide 15:
And as you see here, APDS falls under the broader CVID umbrella diagnosis and Joenja®, in fact, serves as a proof-of-concept for the work we are doing now in these much more prevalent PIDs.

Slide 16:
This slide highlights the opportunity now to broaden the use of Joenja®. The prevalence figures here are for the U.S., but they illustrate the larger opportunity to serve patients and underpin peak sales potential above US$1 billion.

We're very excited about the work that I just discussed to study Joenja® (leniolisib) in these additional PIDs with immune dysregulation beyond APDS. These address significantly larger patient populations which are five to 26 times the prevalence of APDS.

In APDS, Leverne already discussed progress with patient identification and our commercial preparations for geographic and pediatric expansion.

But let me update you now on the variants of uncertain significance, or VUS, project and the opportunity there. Following various discussions over many months involving Columbia and genetic testing labs, it became clear that the labs require additional evidence to reclassify VUSs. Understanding the consequences of VUSs remains a significant unmet need and actually a public health problem for clinicians and patients. And as you see, the number of patients with VUS continues to grow.

To complement the significant first batch of data, which were published in *Cell*, we are now planning new experiments to generate the data needed to allow genetic testing labs to evaluate VUSs. Following completion of these experiments, we plan to provide an estimate of how many VUSs may be reclassified, and how many patients may be ultimately diagnosed with APDS.

In addition, the work published in *Cell* also suggested that the prevalence of APDS could be significantly higher than we currently estimate.

We convened a global advisory board and are now initiating work to explore and better understand the prevalence of APDS as well as the spectrum of disease.

I'll now cover the progress we are making in APDS including some of our near-term regulatory milestones.

Slide 17:
Overall, we made good progress in APDS during 2025 and early this year. While we are disappointed in the Complete Response Letter (CRL) we received from FDA in January regarding our regulatory submission for the pediatric label expansion for Joenja® for the treatment of APDS in children aged

four to 11, we believe we can address both the clinical pharmacology and analytical batch testing methodology issues outlined in the letter. A Type A meeting has now been scheduled with FDA for later this month, and we expect to discuss the Agency's feedback and align on the path forward for resubmission.

In Europe, we have filed a marketing authorization application for leniolisib for patients 12 years and older and now responded to CHMP's questions on manufacturing activities and quality controls and believe we have addressed their concerns. We now expect a CHMP opinion on the MAA to take place at their meeting later this month, with potential EC approval in the first half of this year.

Regarding the Japan NDA for leniolisib for the treatment of APDS in patients four years of age and older, the Pharmaceutical Affairs Council meeting has recommended approval. This news was covered in the Pink Sheet, who importantly noted that this would represent the first approval for children with APDS aged four to 11. We now await the formal decision by the PMDA by the end of March.

I'll now turn to our next pipeline asset, napazimone or formerly known as KL1333.

Slide 18:
Napazimone has also progressed significantly in the past year. This is being developed for primary mitochondrial diseases, which is a group of rare disorders where mutations in mitochondrial DNA lead to significant fatigue and muscle weakness. Napazimone addresses the underlying disorder, by normalizing the NAD+ to NADH ratio which is abnormally low in these patients.

There are a large number of these patients already diagnosed across the U.S. and large European countries, where they are treated at centers of excellence and part of strong advocacy groups. And here, we have a registration-enabling study underway with endpoints agreed upon with FDA. And importantly, there was a blinded interim analysis in which both endpoints passed utility.

Since completing the acquisition of Abliva last year, we are making good progress in the second wave of the study and are on track to complete enrollment later this year with readout in 2027 and potential approval later in 2028.

And with that, I'll turn it over to Kenneth now to discuss our financial results.

Kenneth Lynard – Chief Financial Officer:
Slide 20:
Thank you very much, Anurag. I'm pleased to now provide some color on our strong 2025 financial performance and our outlook for 2026.

The fourth quarter was a strong finish with revenues of US$106.5 million, being 15% growth versus Q4 of 2024. This was driven by continued momentum across our portfolio, including a 9% growth for RUCONEST® and a 53% growth for Joenja®. Notably, Joenja® annual revenues exceeded US$50 million for the first time, triggering our first US$5 million sales milestone payment in the quarter. As a reminder, this milestone is recorded in cost of goods sold and therefore affected gross margin for the fourth quarter.

Adjusted operating profit was broadly stable year-over-year, after several offsetting one-off items. Fourth quarter 2025 expenses include US$9.3 million in expenses related to Abliva and napazimone, following the completion of the Abliva acquisition this year, as well as the US$5 million Joenja® sales milestone payment just mentioned. Excluding these items to compare operating profit on a like-for-like basis to the fourth quarter of 2024, operating profit in the fourth quarter of '25 would have been US$14 million higher.

Finally, cash and marketable securities increased by US$12.2 million from US$168.9 million at the end of Q3 to US$181.1 million at year-end, primarily driven by positive operating cash flow, reflecting the strength of our commercial performance.

Slide 21:
Turning to our full year 2025 results, our financials show the continued strong execution of our strategy. Total revenues increased 27% to US$376.1 million, driven by robust double-digit growth from both RUCONEST® and Joenja®. Gross margin remained stable at approximately 88%, despite the US$5 million Joenja® sales milestone recorded in the fourth quarter.

Operating expenses rose to US$311.3 million. Excluding US$4.1 million of one-off restructuring costs related to our G&A reduction program announced in October, operating expenses were US$307.2 million and within our previously communicated guidance range of US$304 million to US$308 million.

Importantly, when also excluding the full US$29.7 million of Abliva-related expenses, operating expenses increased only 2% on a like-for-like basis. This reflects disciplined cost management.

In total, adjusted operating profit which exclude non-recurring Abliva acquisition-related costs and other offsetting items, was US$36.4 million, compared with a loss of US$8.6 million in 2024. Excluding recurring Abliva expenses and the US$5 million Joenja® sales milestone, operating profit for 2025 would have been US$24.4 million higher.

Cash flow from operating activities totaled US$54.7 million, versus being slightly negative in 2024, showing the improved profitability and cash generation of the business. Cash and marketable securities increased US$11.7 million to US$181.1 million, despite US$68.0 million used for the Abliva acquisition, again highlighting the strength of our underlying operational cash generation.

Slide 22:
Turning to our 2026 outlook, we reaffirm our expectation for total revenues of US$405 million to US$425 million, representing full-year growth of approximately 8% to 13% versus 2025. The growth is expected to be driven by continued growth for RUCONEST® in the U.S., partly offset by declining ex-U.S. revenue as we exit those markets, and accelerated growth for Joenja®.

For RUCONEST®, quarterly revenue typically fluctuates with patient ordering patterns and channel inventory movements, with the first quarter usually being the lowest.

In Q1 2026, inventory drawdowns are expected to impact U.S. RUCONEST® revenue growth by ~7% to 9% year-on-year as market dynamics settle. This is factored into our full year guidance which assumes mid-single digit RUCONEST® growth at the midpoint of the range.

For Joenja®, we expect growth to accelerate, with annual growth approximately 10 percentage points higher than in 2025. The pediatric APDS indication remains an important future growth driver, and we look forward to clarity on the U.S. approval timeline for patients aged four to 11 following the upcoming FDA Type A meeting. In the meantime, U.S. pediatric revenues are excluded from our 2026 guidance.

For operating expenses, we anticipate a range of US$330 million to US$335 million, including more than US$60 million of incremental R&D investment. This guidance also reflects the US$9 million favorable impact from the 20% G&A headcount reduction program announced in October 2025, along with stable marketing and sales spending.

Overall, we remain committed to financial discipline, prioritizing investment that drives near- and long-term value creation for our shareholders.

Because Joenja® revenue is not expected to exceed US$100 million in 2026, we do not assume the US$10 million commercial milestone payments, which otherwise would be recorded in cost of goods sold.

As communicated during our Investor Day in February, and aligned with ex-U.S. rollout plans, no additional milestone payments are expected. We estimate cost of goods sold at 10% of revenues, corresponding to a gross margin of 90%.

Finally, available cash and future operating cash flows are expected to fully support pipeline investments including all pre-launch activities.

With that, I will now hand over to Fabrice for his closing remarks.

Fabrice Chouraqui – Chief Executive Officer:
Slide 24:
Thank you, Kenneth.

So in summary, we are pleased to report another strong quarter and a record year for Pharming in 2025 with US$376 million revenues and a shift to operating profitability and positive operating cash flow.

Looking ahead, RUCONEST® is poised to remain a cornerstone treatment for severe HAE patients, underpinning a strong revenue base.

We see clear revenue catalysts ahead for Joenja®, with the product well positioned to generate a significant proportion of our revenues in the future.

Our upcoming clinical data readouts including the two leniolisib Phase II later this year and the napazimone pivotal study readout next year, each have the potential to unlock significant value and take the company to a whole new level.

And finally, the decisive steps we have taken to improve financial discipline will support driving the positive bottom line.

With our strong commercial and development capabilities, a fit-for-purpose leadership team with strong new additions like Leverne and Kenneth, and a scalable organization, we are committed to making 2026 another steppingstone in achieving our vision of becoming a leading global rare disease company.

Let me now open the line for questions.

QUESTIONS AND ANSWERS

Operator: (Operator Instructions) We are now going to proceed with our first question. And the questions come from the line of Lucy Codrington from Jefferies.

Lucy Codrington (Jefferies): To begin with Joenja® growth this year, how much of that are you expecting to come from the U.S. market versus international markets? And then secondly, looking at the U.S. market, of those 61 eligible patients that are not on treatment in the U.S., how many of these do you think could be converted to paid therapy? Versus how many have been considered and ruled out?

And then finally, in terms of RUCONEST® dynamics, you mentioned that you have heard of patients coming back to treatment and having tried the orals. Do you have any -- I guess it would be anecdotal commentary on how quickly the patients return having tried the orals?

Fabrice Chouraqui: Very good. Thank you so much, Lucy, for those questions.

I'll turn to Leverne in a minute, actually. Let me start with the last one on RUCONEST® and then we can take your two questions on Joenja®. It is true that we've seen some patients trying and coming back.

As you know and as Leverne reinforced, HAE is a very severe disease and patients have got to have it controlled and especially patients who were used to highly reliable treatment. And so when those patients try another treatment and see that their crisis is not properly controlled, they tend to come back to their previous medication very quickly because not controlling a crisis could be life-threatening.

Now when it comes to Joenja®'s growth, as we've said, we see the growth this year in 2026 being fueled both by the continued growth in the U.S. as well as growth increasing in international markets.

Obviously, the growth in international markets will come from the U.K. where the drug has been launched and new launch countries. And this will happen in a staged fashion since once we receive marketing approval, we'll have to negotiate price and reimbursement. So ultimately, a bigger portion of the growth will come from international market, but that's going to be gradual.

When it comes to more specificities on Joenja®'s patient funnel, I'll ask Leverne to comment.

Leverne Marsh: Thank you. Thank you, Lucy, for your question. How we would think about the patients eligible for Joenja® in the U.S. and the pull-through to patients on therapy, that there would be a lag there as patients are going through the patient journey, a fairly complicated patient journey, seeing multiple physicians, the enrollment and then the reimbursement process, before we get to patients on paid therapy.

And so how I would think about the delta there and the opportunity, there's still a substantial proportion of patients both eligible and potentially reimbursable that would drive growth for us in the U.S. in 2026.

Kenneth Lynard: Maybe, Lucy, this is Kenneth speaking. Maybe I can just add, out of the expected Joenja® growth in 2026, it will probably be around about 70%/75% that will come from the U.S.

Lucy Codrington (Jefferies): So 70%/75% from the U.S.

Kenneth Lynard: Of the growth in 2026 for Joenja®, yes.

Operator: We are now going to proceed with our next question. And the questions come from the line of Joseph Pantginis from H.C. Wainwright.

Joseph Pantginis (H.C. Wainwright): So I know you might not be able to give color here ahead of the Type A, but I'm going to ask anyway.

Obviously, the reasons for your discussions, as Anurag said, were the clinical pharmacology and the analytical batches. Is there anything that you would consider sort of the lead rate-limiting step here? That's question number one.

Question number two, regarding RUCONEST®, you touched upon this a little bit, but I guess the RUCONEST® case can be split into two components in my belief.

So first, you have the medical component which continues to make the case. And I'd like to touch upon the investor component and specifically your comments that patients are still seeing switches from the new therapies to RUCONEST®. So I was hoping you can provide some additional color as to why those switches are taking place.

Fabrice Chouraqui: Thank you, Joe, for these questions. So on the Type A meeting, it's very difficult to speculate. I think Anurag has been clear on the questions that were raised by FDA, and we are really looking forward to engaging with the Agency later this month to address their feedback and discuss a path forward.

So too soon to tell. Clearly, given what they've raised, we feel actually that these questions are addressable. And once again we look forward to engaging with them.

When it comes to RUCONEST®, I'll let Leverne answer your question.

Leverne Marsh: So thanks for your question, an important one.
So as we've seen new agents entering the HAE market in the U.S. between June and August last year, as expected, we saw some trialing of both acute agents and new prophylactic agents in the U.S. market.

What we're observing in our data currently, and it's still early, is within about three to six months, some of these patterns may start to shift, and we've seen some return of RUCONEST® patients that have originally adopted or trialed a different product coming back to RUCONEST®.

But again early days, and we're monitoring this closely, and we continue to execute competitively based on RUCONEST®'s very different value proposition for patients, specifically in the high attack segment, where they are increasingly concerned with reliability and a fast on-demand treatment, like RUCONEST®.

Operator: And the questions come from the line of Jeff Jones from Oppenheimer.

Jeffrey Jones: Congrats on the great year 2025. A couple of questions from us. You spoke a little bit to the cadence of moving patients from early access on to paid therapy. Any notable variations between Europe, Japan, Canada as you look to that? And then as you look at the other primary immune deficiencies for Joenja® and the Phase II readouts that you're anticipating, can you speak a little bit about next steps?

What types of additional trials might be needed and how to think about the path forward there?

Fabrice Chouraqui: Thank you, Jeff, for these questions. When it comes to access to paid therapies for Joenja® in international markets, most of these markets have centrally driven access.

So the dynamic is different compared to the U.S., where in a sense, each patient needs to deal with different payers.

So in a centralized access system, things are slower at the beginning because you need to negotiate, obviously with the authorities for reimbursement but once you get reimbursement, then afterwards, the reimbursement process is extremely efficient.
So we don't expect to see the same type of dynamic international.

When it comes to higher prevalent PIDs, I'll ask Anurag to elaborate.

Anurag Relan: Jeff, so on the question about what happens next.

So as I mentioned, the studies have completed enrollment.

We expect results later this year. The results that we're looking for, again the endpoints that we're evaluating are clinically relevant endpoints similar to the ones that we looked at in APDS as well as other clinically relevant endpoints.

As we look at those endpoints, we'll plan to have a discussion with FDA and other regulators about the path forward.

I think our base case here is that we would expect to do a Phase III randomized type of study.

However, I think you've also seen from FDA some openness and willingness to look at alternative mechanisms and pathways for patients with rare diseases, especially those where there's a plausible mechanism and there's a mechanism that's understood.

So those are the types of discussions that we would have with the FDA, again once we have the data to be able to plan the path forward.

Operator: We are now going to proceed with our next question. And the questions come from the line of Sushila Hernandez from Van Lanschot Kempen.

Sushila Hernandez (Van Lanschot Kempen): On Joenja®, you mentioned different launch dynamics for international markets.

So what kind of timelines are you working with for getting these patients in Japan and Canada on paid therapy? And in the U.K., will you also start reporting the numbers of patients on paid therapy? So currently, how many patients in the U.K. are on paid therapy and how many have you identified?

Fabrice Chouraqui: So when it comes to timelines, so we've elaborated a bit on our expectations when it comes to regulatory timelines in the very near future. When it comes to CHMP opinion from Europe and PMDA approval in Japan.

In Japan, specifically since you asked the questions, we will be submitting a price very shortly for reimbursement, and it takes about three months actually for the price to be granted.

So today we have launch timelines planned for the summer.
We will be reporting more information on the international market on Joenja® in a full fashion as soon as we have launches in more than one country.

So this should happen very soon. It's absolutely essential and you can count on transparency here. For the second part of your question, I'll let Leverne elaborate.

Leverne Marsh: Certainly.

As Fabrice has said prior, as we look at different approvals coming online at different times in this year, every country fundamentally will be a country-by-country process as the approval and reimbursement processes are quite unique for the countries that we discussed.

So how we're thinking about conversion is conversion will depend on physician experience, diagnostic confirmation and testing and access, and we are building those enablers systematically with the international teams to make sure that we are able to execute upon approval.

Operator: We are now going to proceed with our next question. And the questions come from the line of Natalia Webster from RBC Capital Markets.

Natalia Webster (RBC): My first one is a follow-up on Joenja®® and the patients on paid therapy in the U.S. You added 18 in H1 2025 and six in H2.

I appreciate that this takes time but are you expecting for this rate to pick up into 2026?

Then the second question is just on costs. You're guiding to 6% to 8% growth in OpEx in 2026. And you mentioned some phasing considerations on the revenue side. Are there any particular phasing considerations for the costs through the year particularly around the higher R&D costs and G&A cost savings? And then just finally, on M&A.

In the release, you mentioned continued focus on potential acquisitions and in-licensing opportunities. So, any additional color on what your thoughts are there would be helpful.

Fabrice Chouraqui: Very good. Thank you so much, Natalia, for your question.

So I'll start with the last one when it comes to M&A. We have clearly a number of growth catalysts, both in our commercial portfolio and in our pipeline in the years to come. So obviously there is no urgency to do any transaction hastily to compensate for any sort of weakness.

Yet, we clearly aspire to leveraging proven capabilities and a great growth platform to take that to a whole new level and make Pharming a leading rare disease, ultra-rare disease player.

And so we are constantly looking out for opportunities to expand our pipeline.

Now it is absolutely essential that these opportunities, if they were to materialize, would be value accretive. And that's really our commitment to our shareholders.

So it's not about actually leveraging any external growth opportunities, but making sure that anything that we would consider would be complementary, would fulfill our mission and will be quickly accretive from a value perspective.

From a cost perspective, I'll let Kenneth elaborate.

Kenneth Lynard: Yes. Thanks for the question.

I think the way to think about it is in the 2025 baseline, we obviously had also some one-off costs that were related to the transaction of Abliva, so non-recurring transaction cost of about US$10

million. And we also communicated earlier that we had about US$4 million in costs related to the G&A reduction program.

But then when you're looking into the more than US$60 million incremental investments in 2026, we, of course see the US$9 million of savings in G&A come fully through.

And then we have seen the impact in our planning of the strengthened capital allocation which has allowed us to keep also marketing and sales cost flat.

So there are different dynamics that are playing in. But I think the picture speaks for itself that we are fueling where we're seeing the opportunities to advance in this case, in 2026, the pipeline and are very diligent around spend discipline across all other areas.

Fabrice Chouraqui: And so lastly, coming to your question on Joenja® which is actually a very important question because, as I said, I really see Joenja® taking a larger part of our revenues as the drug continue to grow significantly and realize its full potential.

You heard that last year, we re-accelerated the uptake of the drug. We had more new paid patients on therapy in the U.S. that we had in '24. Also, we've identified more APDS patients in the U.S. in '25 than we did in '24. And so this is really fueling the growth.

So this year, we expect to continue to accelerate patient enrollment on Joenja® and as a consequence, accelerate our revenue growth.

Operator: And the questions come from the line of Simon Scholes from First Berlin.

Simon Scholes (First Berlin): I've just got two.

So, I was wondering if you could give us a timeline on the performance and collation of the results from these additional experiments you need to perform with regard to the variant of uncertain significance. And also, are you still confident that 20% of these VUSs will turn out to be APDS?

And then just on RUCONEST®, you were talking about a possible inventory-related decline or inventory-related effect on sales in Q1.

Is this an unusually large inventory drawdown that you're expecting to see in Q1? I mean that was my impression. And if so, why do you think you saw such a large inventory buildup towards the tail end of last year?

Fabrice Chouraqui: Thank you, Simon. So let's start with the VUSs. Clearly, this is a sizable opportunity, and we are really committed to helping those patients by accessing the right diagnostics. So Anurag?

Anurag Relan: So Simon, we are now planning these new experiments. These experiments, again we're working with Columbia. They're actually going to be using new technology, new base editing

technology, to be able to generate different types of variants, generate more controls as they go through this process.

It's too soon to tell in terms of the timelines as well as the actual number of patients that will actually be reclassified.

But as this work gets underway over the coming months, we should be able to provide more details around it.

Kenneth Lynard: And Simon, on the inventory part, the way we're thinking about it is that there's always this quarterly fluctuation of the RUCONEST® business, given the patient ordering patterns and the general movements. And we have also historically, if you go back in the previous years, seen similar dynamics in the early part of the year.

Now it's a little bit, let's say higher in terms of inventory drawdown and dynamics this year, and we kind of attribute that simply to some of those market dynamics are kind of settling now and that inventory levels are just kind of returning to a little bit more of the normalized level.

So impact-wise, it's a little bit higher, but the mechanics of how the quarters are fluctuating and the fact that the inventory impact in the first quarter are not new to us.

Fabrice Chouraqui: Very good. So listen, I think with this, we are going to close our call. Thank you so much for these additional questions, and we look forward to keeping you closely informed on our plan. There are a number of important milestones coming up. And so we look forward to reconnecting with you very soon. Thank you so much.

[END OF TRANSCRIPT]