

# Pharming Group N.V. 4Q/FY 2024 Results Call

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Jeff Jones – Oppenheimer Ben Jackson - Jefferies Joseph Pantginis – H.C. Wainwright Alistair Campbell - RBC Simon Scholes – First Berlin

# **Fabrice Chouraqui – Chief Executive Officer:**

Thank you very much. Hello, everyone. And welcome to the Pharming full year and Q4 2024 financial results call.

I'm Fabrice Chouraqui, CEO of Pharming. And I'll be joined on this call today by Stephen Toor, our Chief Commercial Officer; Anurag Relan, our Chief Medical Officer; and Jeroen Wakkerman, our Chief Financial Officer. We will be making forward-looking statements in this call that are based upon our current insights and plans. As you know this may differ from future results.

First of all, let me say that I'm really excited to be joining Pharming, and it's an honor to succeed Sijmen de Vries. I'm passionate about progressing medical sciences and bringing innovation to patients. And naturally, I feel deeply connected with Pharming's mission to serve the unserved rare disease patients.

Over the past 25 years, I've developed experience across the business spectrum from preclinical research and clinical development to commercial leadership, business development and capital formation. I've been able to experience the best of the two worlds, the rigor and sophistication of big pharma and the value creation mindset and agility of venture capital and biotech.

In light of this experience, I'm impressed with the development of Pharming over the past 15 years and its significant growth prospects. RUCONEST® has become one of the cornerstone ondemand treatments for HAE.

Joenja® is already approved and launched in the U.S. for the treatment of APDS with a significant number of patients already on treatment, and it is due to be launched in other key markets around the world. And last but not least, the recent completion of the acquisition of Abliva is another stepping stone in the development of the company.

We have a clear vision for Pharming, which is to develop a leading global rare disease company with a diverse portfolio and presence in large markets, leveraging a proven and efficient clinical development, supply chain and commercial infrastructure.

Our results in 2024 are a good illustration of the solid foundation that we have built to realize this vision. Full year 2024 revenues increased by 21% to US\$297 million, above our guidance range,

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including a strong fourth quarter and with operating profit and positive operating cash flow in the last two quarters of the year. RUCONEST® grew 11% to US\$252 million and 9% in the last quarter of the year, driven by a continued increase of new patient enrollment and the sustained expansion of our prescriber base.

I believe that given its unique profile and positive experience in difficult-to-treat patients, RUCONEST® will continue to grow and could even benefit from the potential increase of the ondemand segment driven by the entry of new entrants.

Joenja® revenue increased by 147% to US\$45 million in 2024. The drug is only in its very early stage of its life cycle, with continued growth to be seen with the enrollment of new APDS patients in the U.S., the launch in key markets including the U.K. in the coming months and several well-defined opportunities to expand the addressable patient population including the pediatric label expansion and the development for much larger primary immunodeficiency indications. Let me now hand over to Stephen Toor, our Chief Commercial Officer, who will give you a more granular perspective on the strong dynamics of RUCONEST® and Joenja®.

# **Stephen Toor – Chief Commercial Officer:**

Thank you, Fabrice. Good morning, everybody. As Fabrice just alluded to, we've delivered another strong performance in 2024. On RUCONEST®, we increased the prescriber base by 11% and new patient enrollments by 24%. This translated to a strong Q4, growing 9% over prior year and hitting almost US\$80 million for the quarter. We ended 2024 with sales of US\$252 million, 11% up on 2023.

In the next two slides, I'll review why RUCONEST® continues to show such strength in growth and why we're confident it will continue to grow in the years to come even as the market becomes more competitive.

On Joenja®, we continue to build our patient pipeline and transition eligible patients to paid therapy. And as you would expect, our team delivered significant growth over the first year of launch, ending Q4 65% up on prior year at US\$13.1 million, and for 2024, plus 147% of US\$45 million.

Of note, in addition to 96 patients plus five pending on paid therapy in the U.S., we have an additional 188 patients on therapy globally under various access programs and in clinical trials that can all move to commercial therapy when the necessary registrations are received.

In the forthcoming slides, I'll also outline the opportunities we see in the coming months and years that will both build the Joenja® business for APDS and with new potential indications for the molecule create a strong high-growth franchise.

Looking first at RUCONEST®. As I just stated, RUCONEST® is and will continue to be a growth driver for Pharming and an important treatment option for U.S. patients and their doctors, which is why it's already the second most prescribed acute product in the U.S.

And one of the key reasons for this is its mode of action. As you can see in the graphic, there are three inflammatory cascades involved in the development of an HAE attack. C1 esterase inhibition

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represented in the graphic by the red C1-INH markers blocks numerous enzymes across all three pathways.

So, while many patients are effectively treated by blocking a single point in these cascades, patients who don't respond to the targeted therapies available may benefit from RUCONEST® since it works comprehensively across all these systems.

C1 esterase inhibition ultimately stops bradykinin production via multiple points in the contact cascade as well as other systems that may lead to attacks, which in turn, and this is important, leads to the 97% attack resolution in a single dose and a sustained response with 93% of patients attacks stopped for at least three days.

So, let's look more specifically at RUCONEST® patients and what this means for them. The first thing to note is that RUCONEST® serves all patient types, those being type 1, type 2, and normal C1 patients. All three of these RUCONEST® patient groups have one thing in common though.

They all suffer from moderate to severe debilitating HAE attacks, and they have them frequently. They've also typically failed other targeted acute therapies such as (inaudible) or are having to re-dose to stop their HAE attack.

In the photos on the slide, you can see an actual RUCONEST® patient at the start of an attack and then her recovery as it resolves at the 4-hour mark and the 24-hour mark.

For patients like this one suffering with a more severe course of disease attacking frequently and having to re-dose on other therapies, knowing, as I just stated, that 97% of patients will stop their attack with a single dose and almost all of them will be attack-free for at least three days is a very big deal. RUCONEST® efficacy and reliability allows our patients to better control and plan their lives, and that's why RUCONEST® will continue to have a strong position in the U.S. acute market and remain an important product for our company in the years to come.

I'll transition now to Joenja®, which, as you're aware, was launched in the U.S. in March 2023 and is available outside the U.S. through various access programs. We see a number of opportunities for Joenja®, which I'll walk you through now. Pharming's patient-finding efforts are continuing as we build our patient pipeline in the U.S. and globally.

In fact, we've already identified over 240 patients in the U.S., of whom 40% are already on paid therapy, and we've identified hundreds more in other key markets.

So, while we work hard to continue to pull through those identified patients and put them on therapy, we also have some important opportunities to drive growth in the near-term and in the medium-term including efforts to expand the addressable population.

So, what are they? Looking at the second block on the slide, the first is the outputs from the VUS resolution program, which Anurag will discuss. That will deliver another bolus of APDS patients available for treatment this year and beyond. The second will be the pediatric indication launched in the U.S., which is expected in 2026.

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We currently have over 60 patients in our U.S. pipeline and growing, and they will begin transitioning to Joenja® as soon as the indication is approved. And the third is our geographic expansion program. Which is the key markets around the world. And this begins this year with the U.K. launch.

In fact, just today, NICE have published draft guidance in which they recommend the use of Joenja® for NHS England and Wales. And then we have further anticipated launches in other important markets including Japan, Germany, France, Italy, Spain, Canada, and Australia. That means Joenja® will soon be available in most of the industry's top 10 markets.

In addition to that, you can see in the final two blocks on this slide, leniolisib for APDS is only part of the story. As you know Phase II trials have been initiated for two bigger indications, genetic PIDs and CVID.

In fact, CVID, while still rare with a prevalence of 40 patients per million, transitions leniolisib from a small ultra-rare disease molecule to one with blockbuster sales potential, thereby creating the leniolisib franchise delivering a significantly greater value for all stakeholders in the coming years.

With that said, I'd now like to hand over to our Chief Medical Officer, Anurag Relan, whose team are, of course, critical to driving these programs forward and realizing these opportunities, to provide us with a research and development update.

## **Anurag Relan – Chief Medical Officer:**

Thanks, Steve. And here, we can see our growing pipeline. For someone who's been at Pharming for many years, it is incredibly impressive to see how this has expanded from only RUCONEST® for HAE to now include multiple products and indications, which can support the vision you heard Fabrice lay out that we have for Pharming.

Since APDS is a primary immune deficiency with immune dysregulation and there are other more prevalent PIDs with similar features, we're especially excited for the work that we have started here with two Phase II studies underway including a new program in CVID or common variable immune deficiency. And last but not least, we have on this slide, KL1333 in a pivotal study with the recent acquisition of Abliva.

Before turning to development, as you know the VUS project has been a focus of our patient finding work. A significant challenge in diagnosing APDS patients occurs when a patient's genetic test result shows a VUS or a variant of unknown significance. This happens because the variant is novel, and there isn't enough information to determine if the variant is disease-causing.

In fact, there are over 1,200 patients in the U.S. alone who have received the VUS result in the genes associated with APDS.

Over the past year, we have been supporting a project to gain additional insights into these VUSs. The recently concluded study, which will be published soon, has shown there are many new variants that lead to hyperactivity in this pathway. The next step in the process is for genetic testing labs to review these data and determine which variants can now be reclassified as causing APDS.

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We expect by midyear, these efforts will lead to the identification of many new APDS patients. Now let's turn to the work going on in pediatrics, where we have an active clinical program with recent data to support regulatory filings.

APDS symptoms begin at a young age, and more than 25% of the patients we found are below the age of 12. Since the disease is progressive, it is important to be able to treat the condition earlier in its course.

In December, we were excited to report the top-line results from the first clinical study in children with APDS ages four to 11. The study demonstrated that leniolisib was generally safe and well-tolerated, and we saw benefits across the two co-primary endpoints, which were consistent with what we have observed in older APDS patients. These data will be presented at a conference in May. And in the second half of this year, we will begin regulatory filings starting with FDA to expand the label to be able to treat younger patients with APDS.

In addition to our work in APDS, we are developing leniolisib for primary immune deficiencies with immune dysregulation, which you see in the diagram are a subset of all PIDs. APDS, in fact, is one such example of a PID with immune dysregulation, and we have started two more programs in this area.

The first program is a genetically defined group of PIDs, which we started a study in October, and we are continuing to enroll patients and FDA recently granted Fast Track designation for the program. We are announcing today the second program in CVID, which is a clinically defined group and represents an even larger group of PID patients.

The study is now open for enrollment, and we expect the first patient to be dosed later this month. The patients in both of these studies lack effective therapies to manage the immune dysregulation that leads to disease progression and early mortality.

And you can see in the prevalence estimates how the new study we announced today in CVID significantly increases the patient population that could potentially benefit from leniolisib. Across APDS and these new programs, the central role of PI3K delta in lymphocytes is clear in driving the immune dysregulation, which supports the investigation of leniolisib.

I look forward to updating you further as we progress with these exciting new programs. To recap, we have a number of regulatory and clinical activities to bring leniolisib to more APDS patients in several key markets across the world and expand the addressable population.

As you heard from Steve, bringing leniolisib to additional APDS patients represents a significant near-term opportunity. And in Europe, we have already concluded the clinical benefit and safety of leniolisib where we have a single CMC issue remaining as part of our review with EMA, and we expect to be able to address this in January of 2026.

We also have marketing authorization in the U.K., and you heard from Steve already, NICE published their final draft guidance today, which will eventually enable reimbursement in the U.K.

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In Japan, we have completed an interim analysis of a small trial there, and this will now enable our filing with PMDA in the middle of this year.

And you heard me already talk about the pediatric study in the four to 11 age group, but we also have an additional trial in children as young as one year of age, which is nearing completion of enrollment. In all, there are quite a number of projects to be able to expand the addressable population including these new PID indications, which support the long-term growth of leniolisib.

And now turning to our third program in our portfolio via the just completed Abliva acquisition, KL1333. This is being developed for primary mitochondrial diseases, which are a group of rare disorders where mutations in mitochondrial DNA lead to impaired energy production. These disorders can have array of diverse clinical features, but a common element is the severe fatigue and muscle weakness seen in these patients, which, of course, leads to a poor quality of life given the degree of symptoms.

There are a large number of these patients already diagnosed across the U.S. and large European countries where they're treated at centers of excellence and part of a strong advocacy group. KL1333 addresses the underlying disorder by normalizing the NAD+ to NADH ratio, which is abnormally low in these patients.

There is a pivotal study underway with endpoints agreed upon with FDA, and there was also a blinded interim analysis in which both endpoints passed futility. Having just completed the acquisition, we plan to begin enrollment in the second wave of the study as soon as possible with sites that are already open.

As with the rest of our portfolio, we see this program as one where we can use our rare disease expertise and infrastructure to bring much needed products to patients where there is significant unmet medical need. Now I'll turn it over to Jeroen to review our financial performance and outlook.

### Jeroen Wakkerman - Chief Financial Officer:

Thank you very much, Anurag. Q4 was a very robust quarter for Pharming. Revenues grew by 14%, versus a very strong fourth quarter in 2023. RUCONEST® growth was 9% and Joenja® 66%. Gross profit grew by 9%, and that is lower than the revenue growth, and that was driven by a one-off inventory impairment following a RUCONEST® production issue at one of our CMOs.

The OpEx was then stable and largely as expected in the quarter. The small increase was caused by one-off costs including a full impairment of a lease contract and just over US\$1 million of Abliva acquisition costs.

Operating profit increased by US\$5.6 million, driven by higher gross profit as a consequence of strong sales and active OpEx management. This was the second quarter in a row that we generated operating profit. And the net results went from a loss in Q4 2023 to a net profit in Q4 2024.

We had a positive operating cash flow in the quarter and in fact, for the second quarter in a row. The cash and marketable securities reduced slightly due to interest costs and currency effects.

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Then looking at the full year results. Full year results in 2024 were good. Revenue was up 21%, which was driven by both RUCONEST® with a strong growth of 11% and leniolisib that grew by 147%.

OpEx increased by 10% due to investments in Joenja®, which is at a rate well below the revenue increase. Operating profit on a like-for-like basis improved, and that's when taking out the big one-offs that we had in 2023.

Our cash position reduced, and that was driven by the refinancing of our convertible bonds earlier in 2024 for a lower amount than the previous bond. A quick update on the Abliva acquisition process and timeline. The US\$66.1 million acquisition of Abliva is now completed and Abliva shares approved for delisting next week.

We initiated the compulsory acquisition procedure for the remaining Abliva shares. And the acquisition of the shares was funded with available cash. And the transaction really illustrates our strategy of developing a high-value pipeline.

Moving to the financial guidance for this year 2025. We expect the total revenues to land between US\$315 million and US\$335 million, which means a growth rate of 6% to 13%.

And the assumption underlying the guidance midpoint is a high single-digit RUCONEST® growth and continued strong growth of Joenja® with an acceleration in the second half of the year from the positive impact from VUSs, as Anurag mentioned before.

For Joenja®, we expect continued growth of patients on paid therapy and the U.S. pricing at an annual WAC of US\$594,000. Regarding operating expenses, we expect them to be flat on the previous year on 2024 prior to the impact of Abliva. We have not yet integrated Abliva, and that process should start next week.

And our preliminary estimate for Abliva-related OpEx is US\$30 million for 2025 including US\$17 million R&D costs and the remainder consists of nonrecurring transaction and integration costs and we will provide an update of these costs in our Q1 call in May. With that, I would like to hand over back to Fabrice.

# **Fabrice Chouraqui – Chief Executive Officer:**

Thank you, Jeroen. As you've heard, our in-line portfolio has generated strong growth in 2024, and we are exiting Q4 with a solid momentum. With its unique profile and strong patient experience, RUCONEST® is well positioned to remain one of the treatments of choice for HAE attacks.

In APDS, after an initial strong Joenja® uptake, we are now working to identify and enroll new patients before capturing two well-defined opportunities with the VUS and the expected pediatric indication. We continue to invest in our long-term growth with the objective to generate two blockbuster assets.

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First, the potential new indication for PIDs with immune dysregulation is a great opportunity to continue to expand Joenja's sales potential. Second, the acquisition of Abliva's pivotal stage program in mitochondrial disease brings another asset with significant revenue potential.

Our 2025 revenue guidance of US\$315 million to US\$335 million illustrates our momentum and obviously we look forward to updating you on our progress. Let me now open the line for questions.

### **QUESTIONS AND ANSWERS**

**Operator:** And your first question comes from the line of Jeff Jones from Oppenheimer.

**Jeff Jones (Oppenheimer):** Fabrice, welcome to the team, and congratulations on a fantastic first earnings update here. I guess two questions for us. First, with respect to KL1333 in the target of primary mitochondrial disease.

Could you speak a little bit to how this patient population breaks down? And what portion of that population you think would be treatable or eligible for treatment based on the likely label? There are a lot of sort of mutations within that group.

And then just a clarification for a second question. For the 188 patients you mentioned that are on the expanded access program, which includes clinical trials, are any of those patients paid? And perhaps could you provide some breakdown in terms of the territories where those patients are?

**Fabrice Chouraqui:** Thank you, Jeff. Let us elaborate on your question. So, I'll hand over to Anurag first to tell you more about the addressable population with the ongoing trial on KL1333.

**Anurag Relan:** Jeff, so with respect to primary mitochondrial diseases, when we talk about the 30,000 number of patients that are in the U.S. and the large European markets, we've already limited it to the group of patients that have the mutations that are going to be enrolled in this study.

So specifically, the mitochondrial DNA mutations, which already represent 80% of all PMDs. And then we broke that down further and looked at the mutations that are specifically being enrolled in this study.

So that's how we came up with this estimate of 30,000. So, in essence, all of those 30,000 are the addressable population. And I'll turn it to Steve now to answer your question about the access program.

**Stephen Toor:** So, of the 188, we actually have those patients in multiple countries around the world including many of the key markets that I listed earlier. Those patients are predominantly in either in the early access program or compassionate use and also in clinical trials. And we do have a number on paid therapy through named patient programs. But as you can appreciate, that's not a specific number or a revenue line that we would discuss publicly.

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**Operator:** Your next question comes from the line of Ben Jackson from Jefferies.

**Ben Jackson (Jefferies):** Just two for me. If we first start on one that's not too exciting. So, the US\$30 million anticipated additional OpEx from the Abliva's acquisition, are you able just to touch on how much of that is recurring? Apologies if you've noted that already in the call.

And then the second one, just interestingly, do you see any theoretical exposure to potential U.S. tariffs if they were to apply to drugs? I get at this point that it's very unclear what's going to happen, and we don't necessarily know where it's going. And then I guess, as a result of that, have you noticed any kind of level of changing inventories or stocking or patient interest in a measure to hedge against a potential short-lived trade war or anything like that? Your thoughts or color around that would be great.

**Fabrice Chouraqui:** Thank you, Ben. So let me take your first question about the US\$30 million of OpEx spend on Abliva in 2025 as announced by Jeroen. About US\$17 million will be R&D and the rest will be nonrecurring transaction and integration costs.

Now when it comes to the tariff, obviously we are monitoring the situation, and we're doing some homework in the background, looking how we can minimize the impact of potential tariffs if they were decided by the U.S. administration.

We're looking at some adaptations that we could make to our supply chain. This is obviously ongoing. I cannot share any details, and we can tell you more in due time. But this is obviously something on which we are proactive and are monitoring very, very closely.

**Anurag Relan:** I think there was a question on whether there's any stocking or inventory buildup as in anticipation of the tariffs.

**Stephen Toor:** No. We have enough stock already for our needs within the U.S., and there's no planned inventory buildup at this point in time until we have a clearer picture of the situation.

Operator: Your next question comes from the line of Alistair Campbell from RBC.

**Alistair Campbell (RBC):** Let me start with RUCONEST®. It's great to see a level of confidence that this product will continue to grow. Just very briefly, in the Q4 number, just for modeling purposes, were there any stocking effects in there? Or is that largely driven by underlying demand? And then thinking about the outlook for 2025, I mean clearly, you feel very confident that you will continue to grow despite new competition.

Is that sort of based on your internal thinking? Or I'm just intrigued if you've done any sort of market research to underpin that in terms of trying to assess what physicians think of the new product as it comes to market? And then finally, maybe turning to CVID, just a sense of the timeframe for those trials, should Phase II trials be sufficient for approval? And then how you think those trials are likely be scoped in terms of size and duration?

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**Fabrice Chouraqui:** So, thank you so much for your question. Alastair. I'll start answering some of them and hand over to Steve and Anurag for the last one on the CVID.

I want to reinforce that actually, there's not been much stocking actually in Q4. And the result, the very, very strong results that you saw actually on RUCONEST® were by large, actually driven by strong demand. So that should be very clear.

When it comes to the positioning of RUCONEST®, again, as we've said, I mean because of the unique profile of the drug, and the very strong patient experience that we've been able to generate years after years since the launch of the drug more than 10 years ago, we feel confident that RUCONEST® will remain a treatment of choice for HAE attacks despite new oral entrants.

As we've seen in other categories, there will probably actually be that the new orals will be help to develop the market. They'll be able to position themselves for specific categories of patients. Based on our experience with RUCONEST® and on the feedback we are gathering from doctors and more general market insights, this is our strong belief. And I'll ask Steve, obviously to comment given his experience with the drug.

**Stephen Toor:** Certainly. The only thing I would add to that, Alistair, and you asked about market research is, we've obviously pressure tested that through a number of advisory boards and steering committees over the past year. And the resounding conclusion of those interactions is exactly what Fabrice said, which is this is a relatively unique drug in terms of its mode of action. The patients we serve are severe and they attack frequently, and they have a positive experience. And for those reasons and as I say, validated externally, we believe that RUCONEST® has a place both in the short-term and the long-term and will continue to be a growth driver for Pharming.

**Anurag Relan:** And then, Alistair, on the question about the CVID study, we'll have some more details after we dosed the first patient, but this will be a Phase II study, of course, and we'll talk a little bit more about what those results and the timing of those results would look like once we begin the program formally, and we'll also then be able to talk to you about what the development path might look like.

**Operator:** Your next question comes from the line of Joe Pantginis from HC Wainwright.

**Joseph Pantginis (H.C. Wainwright):** Joe Pantginis here from HC Wainwright. Fabrice, obviously good luck at the helm here, a very successful company. So, I think you're taking over at a great time as well.

So, first question is with regard to RUCONEST® and building the new prescriber base in the U.S., how would you sort of describe the yes versus no dynamic as you're trying to convince physicians to be new prescribers to support the core growth of the asset?

**Fabrice Chouraqui:** All right. Thank you, Joe, for your kind words. When it comes to RUCONEST®, I've actually, over the past few days actually met a few RUCONEST® prescribers. And so got a first-hand experience of what's happening in daily medical practice.

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I think we see an increased number of prescribers with RUCONEST® as they can see that the drug has a unique profile.

And as a consequence, it has a unique value proposition for a specific segment of the patient, those difficult-to-treat patients who are experiencing actually a number of breakthrough attacks. And those doctors, I think, are reinforced by their experience of the drug.

I mean some of them have spoken to me about a drug which is transformative. I'm using again the word, transformative for the life of their patients. And so, I think that's very meaningful.

Given my experience, I mean when you hear a clinician who has actually treated patients with such a debilitating disease, you've seen, I mean the picture actually of the patients suffering from an attack, that means a lot. Steve, do you want to elaborate a bit?

**Stephen Toor:** Sure. Thank you, Fabrice. Joe, I think to answer your question of the yes versus no dynamic, we're 10 years post launch, and obviously this is a well-developed market. And even in year 10, we were able to grow the prescriber base by 11%. And the reason for that is if you get outside of the centers of excellence where they have large numbers of patients and experience, many physicians haven't had to consider RUCONEST® because they've simply not had a patient or they've had their first one.

So what we find, especially with those physicians is they're very open to the concept of RUCONEST® because they're now having to treat these severe frequently attacking patients that they haven't before.

So as I said, we have a strong base of prescribers. But as the market expands over time and as we go deeper into them, and we are very confident that more and more physicians will need to prescribe, and will use and be open to it.

**Joseph Pantginis:** That's really helpful, Steve. And then I guess, Fabrice, I certainly acknowledge this is a very early time to ask this question. Anything you could share with regard to changes you might or might not envision for the company's growth? Example, is there any further rightsizing of the sales force, the impact of new assets or even further in-licensing of assets to look forward to?

**Fabrice Chouraqui:** It's very early actually to tell you about what my vision and the roadmap for the company will really be. I believe, again, given what you've heard today about the momentum that clearly, we will continue to move forward.

I mean I've tried to share a bit about my vision for the company, which is actually very similar to what you heard from Sijmen in the past.

I think our success will come from great ambition, a relentless focus on execution, rigorous P&L management and OpEx management specifically. And then the continued expansion of our pipeline, looking how we can expand our pipeline with the current assets.

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And you've seen that there are a number of great opportunities with our current assets, but also continue to look at value-accretive deals that could actually drive shareholder value in the midand long-term.

I'd be very happy to tell you more in the coming weeks and months as I'm becoming more and more knowledgeable with the intricacies of our company.

**Operator:** And the question comes from the line of Simon Scholes from First Berlin.

**Simon Scholes (First Berlin):** Thanks for taking my questions, I've got two. Just a follow-up on the Abliva costs. I was wondering if you could give us an indication of how those costs might evolve during 2026 and 2027. And then on CVID, my understanding is that on PI3K delta, you will require a Phase III. I was just wondering if you could give us an indication of why you might not require a Phase III on CVID.

**Fabrice Chouraqui:** All right. So let me quickly answer your question about the spend on the Abliva program. So as I said, and you heard it from Jeroen, about US\$30 million this year, US\$17 million R&D, the rest nonrecurring transaction and integration costs.

When we announced the deal at the end of last year, we've estimated the total cost of the program to be around US\$120 million to US\$125 million, okay? So, you do the deduction with the US\$30 million that we're still in that bulk, obviously we'll refine this as we complete the integration, resume the trial.

But today, we don't have any data that tells us that actually this will be any different. Now when it comes to CVID, I'll let Anurag actually clarify perhaps some misunderstanding.

**Anurag Relan:** Yes. Simon, so we do anticipate and again, this is early days and not having even dosed the first patient yet, but we do anticipate that there would be a need for a Phase III study as we've done with APDS.

These are rare diseases, so these are still relatively small programs. The Phase II program in the first PID with immune dysregulation is 12 patients and the CVID indication Phase II program will be slightly larger, but we still anticipate a Phase III requirement to enable registration. Of course, as the Phase IIs read out, we'll be able to tell you more what those Phase IIIs look like, but that's our current planning.

**Simon Scholes:** Okay. And just one last one. I mean do you also expect CVID to get an FDA Fast Track designation at some stage?

**Anurag Relan:** We will certainly look at all of those types of options. These are severe diseases. They have a similar course as APDS. There's early mortality associated with them. These are sick patients who have these conditions. So, we do anticipate being able to work with the regulators to try to expedite the development.

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**Operator:** There are currently no further questions. I will hand the call back to Fabrice for closing remarks.

**Fabrice Chouraqui:** Thank you very much, Operator. Thank you very much to those of you who attended the call and those of you who were on the webcast. As I said, I'm very excited to be joining Pharming.

I believe that we are exiting 2024 with a very solid momentum that there are very clearly identified growth opportunities ahead of us. And as per my answer to the question, our success will come from our ability to realize them, manage our P&L rigorously and maintain a very high level of ambition given clearly the growth prospects that we can have with our in-line brands and with Abliva.

And also, I think the capabilities and the unique infrastructure that we have created that can really position the company as a leading rare disease company in the future. Thank you very much.

**Operator:** Thank you. This concludes today's conference call. Thank you for participating. You may now disconnect.

[END OF TRANSCRIPT]

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