Pharming Group N.V. 1Q 2023 Results Call

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Sijmen de Vries, MD – Chief Executive Officer:
Good morning, or good afternoon, ladies, and gentlemen, welcome to our first quarter 2023 results call. And before I go into the call, I would like to have the next slide and show you the forward-looking statements right as we will be making some forward-looking statements that are based upon our future expectations, and our current expectations and assumptions. And as you know, they may change in the future. And with that having said that next slide, please.

I'm here with my three colleagues, Anurag Relan, our Chief Medical Officer, Stephen Toor, our Chief Commercial Officer, and Jeroen Wakkerman, our Chief Financial Officer, and they will be speaking after me. And I will start with a brief introduction. So, next slide, please. And then the next one as well.

So, basically, what Pharming is all about is that we are building a sustainable rare business. And that rare business will be able to be funded from the positive cash flows that we continue to generate from RUCONEST®. And you have seen over the last quarters, that these cash flows from RUCONEST® have helped us to prepare and fund the launch of Joenja® in the United States. And the further pipeline development that we have been doing. So, we're in a very favorable position, and today marks the first quarter in which we have been able to actually get Joenja® approved, and where -- and the last quarter where we will be reporting on sales from our single product, because going forward, as you have seen from the press release, we will start recording sales for Joenja® in the United States as well. So, it's an important demarcation, I would say, in the history of our company, that we are now going to work to get revenues from two products, albeit for the time being on only one major geography, the United States of America, but that will soon change as well.

So, therefore, you can see what we're up to, we're up to successful commercialization of Joenja® for APDS and Anurag Relan will talk about that later and additional rare disease indications where we cannot give you any specifics yet, but we will do that in the second half of the year. And, of course, we still are looking for additional projects that are in mid to late-stage development in rare diseases, to actually further fill our pipeline, and use and leverage further our commercialization infrastructures that we have in the U.S. and Europe. And as we are building up in additional markets as well.

And if you look at the next slide at the pipeline, we see that it is. We have now specified it in terms of the various leniolisib activities that are taking place outside the United States, you see that we have a lot of stuff on our plate and are things to look forward to. And first and foremost, of course, the leniolisib approval in the European Union and the U.K., but also the pediatric project, the Japan

projects, and the Canadian and Australian projects. And last but not least, the additional indications for leniolisib. But it means that we can actually, further down the line, have space in the capacity and our commercialization capacity to launch additional products. Hence why we are very -- continue to be very active to look for additional in-licensing opportunities and other rare diseases and/or mergers and acquisition opportunities in the market.

Let me just go back to, for a moment go back to RUCONEST® and that is why you know we are so very proud -- Next slide please -- of the product like RUCONEST® because it helps us getting a $200 million business. If you look back at the last 12 months with an outlook for single digit revenue growth. And it continues to take a unique place in the market because it's the only recombinant treatment that sees the root cause of hereditary angioedema by replacing that missing protein, that dysfunctional or missing C1 esterase inhibitor and it has proven over the years to be well tolerated and effective. And it is the second most prescribed product detail for these attacks and you can't come much closer to 100 percent efficacy and reliability and that's important because patients are relying on RUCONEST® where they have either a very severe form of the disease and have very high frequency attacks and cannot get by with the significantly improved prophylactic therapies or which increasingly is the case They actually rely on RUCONEST® for being able to treat their breakthrough attacks which you know, almost half of the patients still suffer from in very varying frequencies. They rely on RUCONEST® as their breakthrough medication. That's the actually increasing -- there we see an increasing use of RUCONEST® although that thank God for the patients the strongly improved prophylactic therapies.

And we can do all these successes here because we have, as I was already alluding to, very strong commercialization infrastructure -- next slide, please -- that is consisting of all these functions that you need. And you see on the slide here, all the functions that you need to be successful in commercializing, you know, rare disease assets. And that is where, you know, today, we look forward to is very much confidence to further success of growing the company. And with the -- next slide, please.

With the approval of leniolisib recently, we now are embarking on a growth trajectory going forward. Because as I said earlier, we will now be able to report on two products that are generating revenue for our company. Our, you know, our strong RUCONEST® franchise that continues to show single digit revenue as we expect for this year. And on top of that, we are expecting joined sales from Q2 onwards to be increasing going forward.

And it's now time, I think, to hand it over to my colleague, Dr. Anurag Relan, our Chief Medical Officer, to dive into APDS and into Joenja®. Anurag, over to you.

Anurag Relan, MD – Chief Medical Officer:
Thanks, Sijmen.

Before we look at the launch of Joenja®, let's review a little bit about APDS. And we can see that on the next slide that APDS is a rare primary immune deficiency, also known as an inborn error of immunity, that was only first characterized just a little over 10 years ago, in 2013. We estimate that it affects approximately 1500 patients in some of the countries that you see listed there. And that's based on a prevalence of 1.5 per million. To date, we have already identified more than 500 of these patients across these areas. The treatment options until recently have been quite limited for

APDS patients. We'll talk a little bit about that. But this is really the treatment has been limited because it's only been focused on the symptoms of the disease. These symptoms begin early in childhood, but do not address the root cause of APDS. And the signs and symptoms vary. And we'll talk a little bit about what those look like. But they vary even within a family. And this results in significant delays. And especially due to the delayed onset of the diagnosis. The diagnosis itself is actually quite simple to make when a clinician thinks of it and that's really through this genetic test that can provide a definitive diagnosis. Next slide.

But as we see here, APDS really impacts patients in many ways. There are of course the physical manifestations. And you see that really in the top left with the recurrent infections that these patients have enlarged lymph nodes and glands. They have numerous infections that lead to damage to their lungs, and a whole slew of symptoms there that you can see, due to these, to this progressive serious disease that develops early in childhood. On top of the physical manifestations, there's real impact on these patients' quality of life. Of course, there's the social aspects where they can't work or can't go to school or do their normal daily activities. There's a mental aspect of being afflicted with a chronic disease with you know, where the treatment up till now has really been focused on the symptomatic management. And then lastly, the treatment burden, frequent hospitalizations, unnecessary surgeries, many times numerous doctor visits, just to get a diagnosis and then really being limited in terms of what can be offered.

On the next slide, we can see what causes APDS. And this is due to a genetic defect that leads to this hyperactivity of this pathway, the PI3K pathway and that when that pathway is overactive, that results in this dysregulated environment for the B and T cell to develop properly. When these cells of the immune system don't develop properly, you see a chain reaction set off and on the right side you can see all of the symptoms that result because of this hyperactive pathway leading to this immune imbalance. Of course, it's a primary immune deficiency so you see recurrent infections. These can be in the lungs in the upper respiratory tract, the lower respiratory tract. It can also be commonly associated with herpes viruses, especially EBV and CMV viruses. One of the hallmarks of the disease is lymphoproliferation. So, what we see that as in these patients is swollen lymph nodes and enlarged spleen and also disruption of their lymphoid tissue across their body. They also can have gastrointestinal manifestations and commonly because the immune systems aren't -- the immune system is not functioning properly; they have autoimmune issues including cytopenia and other autoimmune disorders. And as I mentioned earlier, bronchial stasis, which is a complication in their lung, where this is irreversible also commonly develops in these patients The most severe manifestation and what often takes these patients lives is the development of lymphoma due to this unchecked lymphoproliferative process and this immune imbalance that occurs as a result of this disrupted PI3K activity.

On the next slide, we can see sort of the treatment options that were available to manage these patients prior to the approval of Joenja® in the U.S. On the one hand, on the left, you can see it was limited to trying to address the immune deficiency. We're using antibiotics to prevent infections to treat infections, but also using immunoglobulin replacement therapy as a way to augment their own immune system. On the right side, you see the issues that they were faced with, these patients, in terms of immune dysregulation to try to control the immune system with steroids or other immune suppressants, including drugs like mTOR inhibitors. None of these therapies, however, were approved for APDS treatment. And in the rare cases, some of these patients were given a stem cell transplant, although transplantation itself is a high-risk procedure in these patients.

On the next slide, we can see what Joenja® now offers. And it's an immune modulator that addresses the root cause of this hyperactive pathway, excuse me, in these patients, and is designed to treat that cause by normalizing this pathway, this PI3K Delta pathway. As a result, what we see is a normal balance of the development of the immune system cells. And we can see that when we measure the immature cells and the functional cells, now they progress normally through their development path.

And on the next slide, we can see the summary of what this Joenja® approval now offers to patients. It is indicated for patients who are 12 years of age and older who have APDS. I'll be reviewing with you some of the randomized data, but it met -- the randomized study met both primary endpoints. And we are also going to review some of the secondary endpoints and exploratory measures that we're seeing in this study. The drug was generally well tolerated and there were study withdrawals due to drug related adverse events. And on the right side, you can see some of the other data that we generated with Joenja® specifically that would have long term data showing reductions including discontinuations in the use of immunoglobulin replacement therapy, as well as reductions in infection rates. These study results were consistent with what was observed in the double-blind placebo-controlled study, including long term data on lymphadenopathy, as well as some of the immune phenotype. And as Steve will report in a few minutes, we are well positioned to hit the ground running with Joenja®. Next slide.

Here is a depiction of a of the label as well as the packaging.

And on the next slide, we can see some of the data from the randomized control study. As I mentioned, Joenja® met both co primary endpoints, which saw a reduction in the lymph nodes size on the left, as well as improvement in the naive B cell count compared to placebo. This strongly indicated a correction of the underlying immune defects. And you can see that when you see the size of the lymph nodes decrease relative to placebo, as well as on the right side, you can see how the naive B cell proportion increased in these patients, again, relative to placebo. Both measures were statistically significant, and these were both -- these were the two co-primary endpoints in the study. Next slide.

And when we looked at the open labeled data, what we saw is, over time, a reduction in the number of days that these patients had infections over the course of the year, and we saw that reduce the longer that they were on Joenja®. At the same time, what was observed, and this was, again, spontaneous, really, in the study where these -- were physicians and patients were able to stop using, in many cases, IRT therapy, and many of them also reduced the use of IRT therapies as the study progressed. Next slide.

Now, looking ahead, we have a number of other milestones later this year. Steve will report on the launch that's been started, just last month and as Sijmen mentioned we are under review under Europe. And we're continuing to expect a CHMP opinion later this year with an approval approximately two months later. We also expect to file in the U.K. later this year with an approval soon thereafter. We're also expecting to start the Japanese clinical study, which was a small study and up to five patients to support a regulatory submission there. And we'll be doing that in the first half of this year still. We, of course, had started earlier this year, a pediatric study in children ages four to 11. And that is going on. And we expect to start our second pediatric study in the third quarter of this year in even younger children. Next slide.

Turning now toward another program that we have as an earlier stage program, and this is a partnership with Orchard Therapeutics to develop an ex vivo autologous stem cell gene therapy for HAE. We're continuing to make progress on developing the vector here to enhance expression levels. And that vector is now being tested in a number of HAE disease models in animals, and we anticipate being able to provide further updates as we move toward preparing an IND filing later this year. Next slide.

And I'll turn it over here to Steve to give you a commercial update.

Stephen Toor – Chief Commercial Officer:
Thank you, Anurag. Good morning, everybody.

Over the next four slides, I'm going to provide you with an overview of the Q1 RUCONEST® performance and some early insights as to the progress of the Joenja® launch just six weeks after approval.

As you're aware, there were HAE market wide issues that impacted some government issue patients resulting in delayed product shipments. Our internal data and external audit data showed significant declines for all acute prophylactic products and -- sorry, all acute and prophylactic products, and that impacted all companies serving HAE patients. The issues are resolved late in the quarter, and as affected patients started shipping RUCONEST® sales accelerated, and the product stays the highest positive return or bounced back of all the acute products in the market. With the Q1 market wide issues and disruption behind us, we saw good sales in March and also strong sales in April as the recovery continued. And we also of course, as the slide alludes to, see strength in the underlying business. More especially high volumes of new patient enrollments and growth in prescribing physicians. We therefore expect sales to strengthen through Q2 and we continue to forecast low single digit revenue growth for RUCONEST® in 2023.

So, now let's turn to the Joenja® launch. As can be seen in this slide, Pharming is bringing all of this rare disease commercialization experience to bear in the U.S. Our first of many launches and a must win market. We have 54 salespeople and sales leaders and that's comprised of the RUCONEST® sales team, where we think 30 percent of patients are treated by customers already very well served by Pharming, and the new Joenja® institutional team. And this team focuses on central locations or centers of excellence, to which we expect the other 70 percent of APDS patients to either currently be treated or be referred to. And between these two teams, we have the vast majority of the APDS market covered in the U.S. And importantly for you to know, our sales colleagues are comprised of experienced rare disease specialists and hospital representatives and sales leaders with launch experience and importantly, patient finding experience. And as with the RUCONEST® team that successfully turned around the brand or re-acquisition from Valiant, we've stocked that team with award winning salespeople to drive a successful launch. So, there are feet on the street, they're out there identifying patients. Importantly, as you see here, we also have clinical educators to drive family mapping and family testing. And this is critical because this is an autosomal dominant disease. So, other members of the family are highly likely to have APDS and it's important for them that they get access to Joenja® as quickly as they can. And of course, it's an important source of new patients for Pharming. We also have a dedicated full-service concierge Patient Services program that ensures once a patient is diagnosed there is zero distractions and

challenges to addressing or to get Joenja® into a patient's hands. And I think that's important in what is a complex market to navigate. The program covers all of the basics of the filling of prescriptions, financial aid, and ongoing support to ensure adherence and continued access to medication. And in terms of staffing, this is where we believe we've really differentiated ourselves from many of our competitors in the rest of the space. We have care coordinators providing a single point of contact, often the same person delivering consistent service and care and providing reassurance to patients and their families. And that's versus the more traditional commoditized call center model. We have the clinical educators I've mentioned already there to support and educate patients and caregivers. And importantly, we have clinical pharmacists that will be available 24 hours a day, to process Joenja® prescriptions, answer any questions patients might have and speed up approval rates, which having these guys on team really allows to happen. Importantly, we've also partnered with Panther RX, which many of you familiar with the U.S. market will -- who you'll know, they're an excellent value partner, specializing in rare and ultra-rare conditions. And that gives them unique insights and really helps them to deliver for our patients in the way that they expect, and Pharming expects. So, this dedicated program and staff should speed access to medication, minimize bureaucracy and mistakes, and cater, as I said, to the very specific needs of these patients. Next slide, please.

Before I get to the early results, I just want to talk briefly about the value proposition that for Joenja® which Anurag articulated very clearly earlier. So, we should remember that Joenja® is the only indicated treatment for APDS. It's a precision medication, so when the patient tests positive, the ACP and the payer know they're prescribing and approving the right treatment for -- the right treatment option for the patient. And Joenja® disease modifying so it's working on the root cause of APDS as Anurag said. For immune deficiency and dysregulation. So, Pharming therefore is launching a part of the physicians and their underserved patients need. And we have, as I've outlined, hopefully, the right infrastructure and services to get products into the patient's hands as quickly as possible. So, let's look quickly at the progress so far. Next slide, please.

So, I think as you all know, the launch and market preparation were rigorous and thorough. And as expected, we're off to a very good start. So, our first fully reimbursed commercial shipments of Joenja® occurred just two weeks after FDA approval. To date, we've shifted 23 patients all on payer approved products, about half are from the early access or open label extension programs. And we continue to make good progress transitioning these 25 patients to pay product. The other half of those patients are patients that are new to Joenja®. So, mostly EAP patients are enrolled on pay therapy and we're steadily working through the early patients and all this while simultaneously building a new patient caseload. Importantly, for the U.S. in the area of market access or managed care, we continue to make good progress with national regional payers, including state Medicaid programs to prepare for clinical review of coverage policy development. And we expect to see those developed in the next 90 to 100 days. In the meantime, patients are being approved pretty quickly through the medical exemption process. Looking at Medicaid specifically, our teams have done an excellent job getting Joenja® covered for APDS patients with already two thirds of the states listed the product in just six weeks. So, as you can see, we're prepared, we're off to a fast and impressive start, only six weeks since we launched Joenja®. And I greatly look forward to updating you on Joenja® launch progress later in the year when we can share the Q2 results for both RUCONEST® and Joenja®.

And with that I'd like now to hand it over to Jeroen who will cover the financials.

Jeroen Wakkerman – Chief Executive Officer:
Yeah, thank you very much, Steve. And good morning, good afternoon. As -- next slide please.

As Steve mentioned earlier, as you can see on this slide, the first quarter results were lower. And that was due to the HAE market factors which impacted on the entire industry. And those industry-wide factors have since resolved and we can confirm that we have truly recovered. And if we look at the quarter January was in line with last year, and February is where we faced headwinds. March had a strong recovery, and so had April. We've almost made up all of the shortfall and expect to recover the remainder. We therefore continue to expect single digit growth in RUCONEST® revenues for 2023.

And on the slide, you see that the revenues in Q1 were 42.5 million, that's 9 percent down on last year for the reasons I mentioned. Gross Profit developed in line with that it went down by 8 percent to 38.5 million and the operating costs increased from 40 million to almost 53 million and that was on the back of leniolisib both in R&D investments and sales and marketing costs. Operating profit and net profit reduced, and the operating loss was 13.7 million and a net loss of 12.2 million. So, the short of this quarter is that the sales shifted from Q1 to, Q2 And we've seen that in April. And with regards to costs, we're investing in leniolisib. And obviously, we haven't recorded any revenues in Q1 yet for leniolisib or Joenja®. But that will change in Q2. But then go to the next slide, please.

On the cost development, you'll see that we are continuing to invest in a launch of Joenja®. If you looked at the longer-term trends over the quarters that are shown here starting with the R&D brackets at the bottom, we see a reduction in quarterly R&D costs in Q2, and that is because of reduced investment in a transgenic platform. And you see an uptick again in Q1 this year, because of leniolisib. Looking at the G&A general and admin costs and developments, we've seen a slight growth per quarter of the last quarters, which basically means investment ahead of company growth. Q1 2023 was higher than the last year, but lower than previous years. And the big number in Q4 by the way to 17.6 that you see is because of an impairment cost of a building. So, that's not a repetitive cost. The marketing and sales cost is the biggest bracket we've seen a quarterly growth of marketing and sales cost in 2022 With more investments in the Joenja® launch, especially obviously in Q4 last year. And I should note that the marketing and sales expenses for your U.S. launch are high in this period, also in Q3. And going forward, we will see an increase in marketing and sales costs in other key markets, namely Europe and the U.K., in the trading quarters as we prepare for launch. To get an indication of OPEX levels for the remainder of the year and therefore for full year the Q4 of 2022 and the Q1 2023 OPEX levels are good indicators, albeit it may increase moderately. If we then go to the next slide.

It's about the cash flow. The cash went down from 207 million to 185 million at the end of Q1. And the key reason is the net cash flows used in operating activities. The cash loss was 10.2 million from operations, working capital increased by 12 million. And that was mainly because of an increase in Q2 and that is -- the latter was because of phasing. The cash flow used in financing activities is due to irregular interest and lease costs and we have some positive foreign exchange effects, bringing the cash to the $185 million. Then the outlook on the next slides.

We continue to expect low single digit growth in RUCONEST® revenues for the full year. Joenja® was approved in the first quarter on the 24th of March by the FDA and we have been commercializing in the U.S. since early April 2023. We are expecting Europe a positive CHMP

opinion in the second half of this year, and the marketing authorization to follow approximately two months later. Subject to the positive outcome of the CHMP review we will file for U.K. approval with the MHRA. We will continue to invest in future growth and to accelerate it and that will obviously be focused on the Joenja® launch. And we will provide further details of our plans to develop leniolisib in additional indications in the second half of this year. And to finish off with we will continue to look for late-stage opportunities and rare diseases be it in in-licensing or in potential acquisitions. So, we're still open for investments very much in that area.

With that, this concludes the presentation, and I would like to go to the next slide and open up for questions and answers to any of them of the people attending the call from the Pharming side. Thank you very much.

Operator:
Thank you. If you'd like to ask a question, please press star followed by one on your telephone keypad now. If you change your mind, please press star followed by two. When preparing to ask a question, please ensure your line is unmuted locally.

Our first question comes from Alistair Campbell of RBC. Alistair your line is now open, please go ahead.

Alistair Campbell – RBC
Thanks very much. Yeah, a couple of questions, please. First of all, on RUCONEST®, just so I understand what's going on here. Is this a feature of basically sales? Which would have happened in Q1, and we caught up in Q2? Or is there actually genuinely a shortfall in sales? I'm just trying to understand, you know, if the disruption hadn't happened in Q1, would you be more likely to get maybe something like mid-single digit growth this year as RUCONEST® rather than low single digits? So, that's question one.

Question two is I know, it's very, very early. But just to get a bit of insight into the patients you've put on to Joenja®. At this stage, do you have a sense of severity? There are patients from across the spectrum of severity, or do they tend to come from the most severe end?

And if I can push my luck, you've got 23 patients on therapy now? What do you think a good number would be to be sort of exiting 2023? Thank you.

Sijmen de Vries, MD – Chief Executive Officer:
You were breaking up. Your third question, Alistair?

Alistair Campbell – RBC
Sorry, third question was, given you've got 23 patients on Joenja® now, what do you think would be a good exit number for the end of the year?

Sijmen de Vries, MD – Chief Executive Officer:
Thanks. Let me answer the last one and I'll go back to Stephen on the first two questions. We don't give any sort of, you know, forward looking statements on what we think is a reasonable number, I think it's too early. But you obviously agree with us that already having 23 patients on paid therapy, and many more in the enrollment process gives you a good indication that we prepared

to launch very carefully, but he also was, Stephen was talking about how well we're progressing with getting the reimbursement sorted for Joenja®. So, you know, let's keep it at this. And obviously, over the coming quarters, we will continue to report on those patient numbers. And going forward, we will give some more indications as and when we see a clear trend arising. So, that's the answer to question number three, I'm sorry, I can't go into any further detail. And I would like to go back to Stephen about your question with regards to RUCONEST® sales in the first quarter. And then the -- if there's any sort of differentiation in the severity of your Joenja® patients. Steve, over to you.

Stephen Toor – Chief Commercial Officer:
Thank you, Sijmen.

In terms of the RUCONEST® sales, I really think our guidance wouldn't have changed, there was disruption in the first quarter, which pushed patients out a little in terms of delivery of product. So, at this point, we're playing catch up. So, I think the guidance would remain the same as it was in mid to late March when we last gave it, which is expect low single digit revenue growth.

In terms of severity of patients, I don't have deep insights. What I would hypothesize and perhaps invite Anurag, if he's got anything to add, is that these are patients largely already identified, at least half of them are in EAP, or in the open label extension. So, they were identified, therefore, they are exhibiting symptoms, and were at least at the moderate end of the scale. Does that answer your question?

Alistair Campbell – RBC
Yeah. That's great. Thank you.

Anurag Relan, MD – Chief Medical Officer:
I'm sorry, I was just going to add. I think the key point is that there was a mix of patients here. So, we have patients who were in the study that were -- have now started a Joenja® commercial paid product. We have patients who were in the expanded access program. And we also have naive patients. So, these are patients who are not in the extended access program or in the study, who are now receiving Joenja® leniolisib for the first time.

Alistair Campbell – RBC
Okay, thank you.

Sijmen de Vries, MD – Chief Executive Officer:
Okay. Does that answer your question Alistair?

Alistair Campbell – RBC
Yes, it's good. Thank you.

Sijmen de Vries, MD – Chief Executive Officer:
All right. Thank you.

Operator:
Our next question comes from Sushila Hernandez from VLK. Sushila, your line is now open. Please go ahead.

Sushila Hernandez - Van Lanschot Kempen
Yes, thank you for taking my question. On RUCONEST®, could you expand on the reimbursement disruptions affecting the HAE market? Since Takeda showed an increase in sales this quarter. What sort of circumstances lead to a more pronounced disruption on your end?

And a second question. You mentioned that you expect quarterly fluctuations for RUCONEST® still. So, what are the drivers behind these fluctuations that you're anticipating? Thank you.

Sijmen de Vries, MD – Chief Executive Officer:
I hand these questions back to Stephen.

Stephen Toor – Chief Commercial Officer:
Certainly, I mean, the disruption was primarily in the government sector and some of those patients saw disruption to their co pays and the cost of accessing medication, which is what resolved itself as we went through the quarter. I can't comment to the company, to the sales, obviously, of other companies. What I will say is if you look at Symphony data, for example, and specifically their metrics database, you see that every company saw significant disruption in Q1. So, I can't comment on that. But I can say that the disruption was specifically within the government insured patient sector. And it resolved satisfactory, and all those patients, certainly on the RUCONEST® side, are receiving their medications.

Sijmen de Vries, MD – Chief Executive Officer:
And the other question, Sushila?

Sushila Hernandez - Van Lanschot Kempen
Yes, you mentioned that you expect quarterly fluctuations in RUCONEST® sales. So, what are these drivers behind these fluctuations that you anticipate?

Stephen Toor – Chief Commercial Officer:
Apologies, I missed that. So, it's mostly tending to be obviously more a combination of seasonal and just the length of the selling month. So, for example, we tend to see some dips during the significant U.S. holidays, so Independence Day Thanksgiving around Christmas. So, it's not -- and also in during the holiday season as well, where patients will stock in ahead of going on vacation, and then not order as much during the periods when they're away. So, it's -- and I think it's the fact we're not, we're not driven by the law of large numbers, right, we have a certain amount of patients and when the change in their order and patterns changes, then you see changes in our order rates and therefore quarterly fluctuations. So, it's nothing of real significance beyond that.

Sushila Hernandez - Van Lanschot Kempen
Okay, thank you.

Stephen Toor – Chief Commercial Officer:
Thank you.

Operator:
As a reminder, if you would like to ask a question, please press star followed by one on your telephone keypad now, and if you change your mind, please press star followed by two.

Sijmen de Vries, MD – Chief Executive Officer:
Does this mean there's no more questions?

Operator:
Our next question comes from Simon Scholes of First Berlin. Simon, your line is now open. Please go ahead.

Simon Scholes – First Berlin
Yes. Hello. I see you've taken the decision to discontinue Pompe. I remember a few years ago, you expected your product and the development to have quite a benign side effect profile compared with the current market leader. I was wondering if you could comment on your decision to discontinue given your expectation of those -- of that positive side effect profile or -- and also, whether you took the decision to discontinue maybe because it would have would have taken too long to bring the product to market if that played a role?

Sijmen de Vries, MD – Chief Executive Officer:
Yeah, that's a good question, Simon. I think there's still significant unmet medical needs in Pompe. So, that's not necessarily the case. We did not simply see the differentiating features that we felt confident enough to go forward with investing in the project. So, therefore, we basically, you know, decided to stop, and abandon this project. And there's also, of course, new developments on the horizon. Where other than protein replacement therapies for instance, the GYS1 receptor antagonists are being developed as we speak. So, we thought it was appropriate to stop this bearing, you know, but not seeing any of the differentiating features, that was the main reason to make the decision.

Simon Scholes – First Berlin
Okay, thanks very much.

Operator:
If you'd like to ask a question, please press star followed by one on your telephone keypad now.

And there are no further questions. I'll hand it back to the management team for any closing remarks.

Sijmen de Vries, MD – Chief Executive Officer:
Okay, thank you very much.

Yeah, ladies and gentlemen, thanks for attending this, this first quarter results conference call. As I was saying in the beginning of the call, this first quarter marks a clear demarcation. We've got our second product approved here. We have an established commercialization infrastructure in the U.S. and we're building that up in Europe. So, we're preparing very rigorously for the launch of leniolisib outside of the U.S. I hope you will agree that we are off to a very good launch with regards to Joenja® in the United States, because of the fact that we already have these 23 patients on paid therapy, you know, within six weeks after launch, which is not a given in rare diseases, and there

are many more already in the process. So, we look forward to -- very much forward, I would say, to coming back to you next quarter and reporting on not one, but two products that will drive our revenue. And of course, last but not least, we remain confident, I would like to again say that we remain confident in the robustness of our RUCONEST® business going forward.

So, thank you very much for being here again, and we look forward to updating you again on the next quarter's results in the beginning of August. Thank you. Goodbye.

[end of transcript]