Pharming Group N.V. 2Q/1H 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 second quarter and first half 2023 financial results call. Next slide please.

I'm here with my three colleagues, Dr. Anurag Relan, our Chief Medical Officer, Stephen Toor, our Chief Commercial Officer, and Jeroen Wakkerman, our Chief Financial Officer, to take you through the highlights of these results and to answer all your questions that you might have afterwards. But before I do that, I would like to point you to the next slide that says something about forward-looking statements, because we will be making some forward-looking statements today that are based upon current plans, beliefs, market circumstances, et cetera that may change towards the future.

And then without further ado, I'd like to move on to two slides ahead and share with you some of the excitement over the last six months. We had a very busy six months, and indeed, made a lot of progress. But first and foremost, we were very, very pleased to see that RUCONEST® did it again. 20% growth over the second quarter results versus the first quarter is a very spectacular recovery from what was a one-off weakness in the first quarter. And it means that we're very confident that we can continue to be on track for the low single digit growth for the entire year. And we say that based upon, the leading indicators that all point in the right direction. Of course, Stephen will talk a little bit more about that later in his part of the presentation.

The significant cash flows that RUCONEST® has been generating over the years not only helped us back in 2019 to in-license Joenja® from Novartis, but also helped us to finance the development of Joenja® and prepare for the successful U.S. launch. And that's exactly what happened: the FDA approved the product a couple of days before the PDUFA date, and we were very quickly off to a strong start in the market. And you've seen and I dare to say that in an ultra-rare business, having 43 patients already on paid therapy in the first quarter that you report results, out of 60 that are already in the pipeline as of June 30 is no mean feat, and is a testament to the preparation of our U.S. colleagues. And it beats analyst expectations insofar as I've seen analyst reports predicting first quarter sales for Joenja®.

We've also made a lot of progress in the regulatory reviews: we've submitted the file to a number of other territories – Canada, Australia and Israel. And we made strides forward with a pediatric clinical program, and that's important because although the current license for Joenja® already incorporates those 12 years and upwards, there is a big unmet medical need to be fulfilled in those

children younger than 12 years. And we're working very hard to get that pediatric program done so that we can submit the file for the extension to the younger children.

And then the third pillar on this slide is as important, if not more, for the future significant inflection point that we believe that we can achieve with leniolisib. That is the second indication. And we, as you may have read already, have already been submitting our plans and have initiated discussions with the FDA on our proposed development program for the second indication, and Anurag will come back to that a little bit later.

And last but not least, we continue to look intensively for additional in-licensing opportunities for rare disease assets that can further leverage our commercialization structure that we have in place. Next slide, please.

Here you see a visual of our pipeline. We're still under review in the European Union and the U.K. The pediatric trial has started. In Japan we're on the brink of starting that trial that we agreed with the Japanese authorities to do. Anurag will comment a little bit later on Japan. And you see here that we made progress, as I just alluded to, by submitting the files to the Canadian, Australian, and Israeli authorities, to increase the geographical footprint for leniolisib or Joenja®.

And then of course, we also made great strides forward - next slide please - with the strengthening of our leadership. We're very pleased to have found Dr. Richard Peters as the new Chairman elect to succeed Paul Sekhri, who came to his maximum term as allowed under Dutch law as chairman of our company. As you can see from the outline on Dr. Peters, he's got an impressive track record, a lot of experience in the healthcare industry, but also in academia, but especially in the rare diseases business with very successful companies such as Genzyme, or Sanofi Genzyme as it is called nowadays. He has been CEO of two NASDAQ-listed biotech companies. As you can see, he held positions in successful companies such as Amgen and Sanofi. And of course, he is a medical doctor by education and has also served as an editor for a very prestigious journal. So, we're very pleased that Richard was happy to join us as our Chairman elect. And of course, we will organize an Extraordinary General Meeting of Shareholders in the not-too-distant future, to actually get him elected.

And then we're very pleased as well to announce, especially in the context of our growth strategy and our ambition to in-license or acquire additional late-stage assets for rare diseases, that Alexandre Breidenbach has agreed to join us as our Chief Business Officer, who will be tasked with the development and execution of our growth strategy and our future plans. And he's a very experienced individual as well, has had several senior positions. Most recently, Chief Business Officer and Chief Development Officer at ACM Biosciences, a Swiss, Basel-based company, and also had a very distinguished and long career in Roche in Basel in the Business Development group.

So, we're very pleased to have strengthened our team with these two extremely experienced and talented individuals. Next slide, please.

Let's just look at - and then the next one, please - what it actually means and what it brings, what we need, and what is our bread and butter. That's our very strong rare disease, commercial infrastructure. We have what it takes to actually be successful at this, we've already proven that, and we're about to prove that again with Joenja® going forward. So, we have these dedicated sales forces in the U.S., in the European Union, and also, we have people in the Middle East and North

Africa calling on these immunologists and academic hospitals, we have our medical affairs team. So that's, of course, very important, especially in rare diseases, that you have a very strong medical affairs team that include medical directors, medical science liaisons, molecular geneticist and publication specialists. Very big medical teams that are absolutely essential for your success in commercialization of rare diseases.

And then as market access becomes more and more important, we have built up our market access teams over the last few years on both sides of the ocean. They include national account directors in the U.S., Health Economic Research Specialists and Directors, on both sides of the ocean, to work with the authorities to get the product to reimbursement. And you've seen how quickly we got the product reimbursed in the U.S. So, I think we have an excellent team in place there.

And then there's Patient Support Teams that help services in the U.S., reimbursement managers, patient care managers, we have a third-party nursing force to take care of our patients and clinical educators. And then last but not least, we're extremely active with everybody, including our salesforce teams to be at conferences, and there's a lot of disease education ongoing, also directly to patients that we actually facilitate, we support the patient organizations. So, basically, it's an extremely, well-oiled team. And that has shown that for instance, with the launch of leniolisib, they know what they have to do, given the fact that we got it immediately reimbursed and found and identified those patients in a new disease. A disease not even discovered 10 years ago.

And as you can see on the next slide, we're really ready to leverage all of this for Joenja®. As and when Joenja® gets approved, of course, and as you heard earlier, we've also now decided to expand to Australia, Japan and Canada that are not yet on this map because it's just the established business.

Okay then, one more slide from my end here about the durable commercial asset that RUCONEST® represents. It's quite rare, I would say, that a product that's already nine years in the U.S. market is still growing and is still getting more and more meaningful.

Why is it getting more meaningful and why is it growing? Because it has a very special place. It's the only recombinant protein-replacement therapy that is available in the hereditary angioedema market. And that's very important, because although there is a big trend that has been going towards prophylactic treatments for hereditary angioedema. Those prophylactic treatments have become better and there's also the fact that almost half of these patients suffer from so-called breakthrough attacks under the prophylactic therapy. That's where it becomes important to have your breakthrough medication at hand at all times.

RUCONEST® is now beginning to make inroads and being used for breakthrough medication. And that is a very important aspect, because it means that more and more patients are discovering that it is the right drug to have for your breakthrough medication and that more and more physicians are actually discovering that RUCONEST® is the right choice for that patient's breakthrough medication.

And yes, RUCONEST® is an IV drug that is self-administered, but we also know that the absolute vast majority, almost all patients, are very, very confident and very well-trained to actually do the self-injections. They are perfectly happy with that because they can rely on the product and you can see it on the slide here that it has an incredibly good track record with regards to efficacy, and

that they can basically count on it to stop that breakthrough attack as it becomes clear that they're getting a breakthrough attack. That's why we know that RUCONEST® has this very special place in the market, and will continue to have this very special place in the market going forward.

Then, I would like to switch over to APDS, and I'll switch over to Anurag to present the Joenja® story. Anurag, over to you.

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

And on the next slide, we can see a little bit of information about APDS, which is a rare, serious, and progressive primary immunodeficiency. And as you heard from Sijmen, it was first described about 10 years ago. We think that APDS affects about 1.5 patients per million, based on literature estimates and our own patient-finding efforts, and I'll talk a little bit about those patient-finding efforts in a moment.

But with those efforts, we've identified now more than 640 patients in key global markets already. We think that that represents a portion of the total of 1,500 patients or so that are out there with APDS. And the signs and symptoms of APDS vary widely. As with many rare diseases, there's also a significant delay to diagnosis because of these varying symptoms and the rarity of the disease. These delays result in significant morbidity and mortality for these patients and it's something that we're really trying to address with our own patient-finding efforts and making Joenja® available.

The treatments for APDS up until now have been focused on symptom management. So, really, when we think about the problems that these patients face -- so, with infections, giving them antibiotics or giving them immunoglobulin replacement therapy, is really not addressing the root cause. A genetic test, however, is a very simple way to make the diagnosis of APDS, and we'll talk a little bit about some of the things that we're doing on this front too, in a moment.

On the next slide, we can see that Joenja® was approved by the FDA earlier this year. Joenja® modulates this hyperactive pathway and as a result, allows the immune system now to develop properly. This development of the immune system, when it occurs improperly in these APDS patients, leads to all the problems that we see in these patients, with autoimmune phenomena as well as all of the infection-related problems that these patients face. With Joenja®, we can try to balance this pathway and to support this immune function in a proper manner.

Next slide. And here's a highlight of some of the data on Joenja®. You can see in the top left, the indication statement that Joenja® is approved in the U.S. to treat patients who are 12 years and older with APDS. And this was on the basis of a randomized placebo-controlled study which showed that Joenja® met both co-primary endpoints, as well as meeting several secondary endpoints and exploratory parameters as well.

From a safety standpoint, there were no drug-related serious adverse events or withdrawals due to Joenja® in the study. But more importantly, what we saw was that when patients took Joenja® on a long-term basis, in the open-label study, we saw many other downstream clinical benefits, including reductions and discontinuation in the use of IVIG or immunoglobulin replacement therapy, as well as reduction in infection rates.

And these results were consistent with what we saw in the randomized double-blind placebo-controlled part of the study. We continue to collect further data and we'll report this data on lymphadenopathy, as well as some of the biomarkers that were collected in the study, including markers of abnormal B cell development as well as the production of some of the antibodies that we see are elevated in untreated APDS patients.

As you've seen from our data so far we're off to a good start, with the patients being able to start Joenja® very quickly in the U.S., and Stephen will report on this progress further shortly.

Next slide, please. And what are we doing now to look for APDS patients? When we think about APDS, it's really part of a larger pool of patients with what's called inborn errors of immunity or sometimes also called "primary immunodeficiency." And through those efforts, we've – as I said – identified more than 640 patients worldwide with APDS, among which there are 200 identified in the U.S. Those efforts, you can see in the box on the left, are really focused around education; testing patients; family testing; multiple medical affairs activities, including raising awareness about the disease, raising awareness about the condition itself, and really working with patients and clinicians to help identify patients.

But we also know, as with most rare diseases, there's a large pool of undiagnosed APDS patients. We are out there with our medical and commercial teams, trying to identify these patients. We know that many of these patients are seen by immunologists, but we also know that they are also being seen by other providers, where they don't even have a diagnosis yet of a primary immunodeficiency or an inborn error of immunity. We're making available in the U.S. a comprehensive genetic testing program to help get those undiagnosed patients the proper diagnosis.

As a result of this genetic testing and the wider-spread use of genetic testing, we're also encountering many patients in the third box here on the right. These are patients who have a result that is inconclusive, and this is called a VUS or a Variant of Uncertain Significance. What that means is that they have a variant in one of the two genes that leads to APDS, but that variant has not been described previously. There's not much information in the literature about it or there is no information in the literature about it. So, we're doing a number of efforts now to help those patients – which there are a significant portion of – that have this inconclusive result, that have a primary immunodeficiency, that have already had a genetic test but have a result that doesn't give them a final diagnosis.

We're doing a number of things that you see there, including looking through the literature, going to other clinical laboratories and seeing what testing may have been done. We're also embarking on functional testing, both in the U.S. as well as worldwide and then, importantly, family testing, to try to see if we can figure out if this variant is present in other family members and if those family members also have the condition or not. As we move forward, I expect that we're going to find a significant number of patients in this population of patients who currently are sort of in limbo with this Variant of Uncertain Significance diagnosis, currently.

Next slide. Then thinking ahead, beyond the FDA, as you heard from Sijmen, we are continuing to work with the European regulators, and we expect an opinion from the CHMP later this year, with a potential approval, subject to a positive opinion, two months later. Then, if we think about the U.K., if the CHMP issues its positive opinion, we can file in the U.K. very soon thereafter, with the

potential approval also two months later. The Japanese clinical study is open for enrollment, and I expect the first patient to be enrolled and treated there in this quarter. And as we heard from Sijmen, we've also made progress with filings in Canada, Australia, and Israel.

We've launched our named patient program to make Joenja® available in certain markets and we announced earlier this year that the pediatric patients are able to enroll in the first pediatric study, which is in the age group of four to 11 years old. As a Sijmen mentioned, we're also making significant progress to looking at other indications for leniolisib. I expect to be able to talk about this in greater detail later this year, toward the end of the third quarter, beginning of fourth quarter, as we go through some of the regulatory discussions about our clinical development plans. Also, later this year, we're going to be starting our second pediatric study. This is in the youngest age group. These are, again, patients who are age one to six years old. And I expect to be able to start that study in the third quarter of this year.

And just a quick update on where we are with EMA and the progress that we've made so far. As you know, we submitted our responses to the CHMP Day 120 list of questions. And just a couple of weeks ago, we received further questions, what's called list of outstanding issues as part of the Day 180 procedure. We also understand now from CHMP, and we're quite pleased with this, that the CHMP will consult an Ad-hoc Expert Group and they've acknowledged that the condition is rare and there's an unmet need here, and they'd like to seek further guidance from an Ad-hoc Expert Group of clinicians to talk a little bit about APDS and the leniolisib trial results to put them in context.

This will be a closed meeting that will involve Pharming representatives, where we'll have some leniolisib investigators as well as APDS patients. As I said earlier, we're expecting the CHMP opinion in the fourth quarter of this year and, subject to a positive opinion, an approval two months later.

And over to Steve.

Stephen Toor – Chief Commercial Officer:
Thank you, Anurag. Good morning and good afternoon, everybody. I'm going to provide you with a short update on RUCONEST® progress in 2Q and in the first half, and also the Joenja® launch in 2Q of this year. Next slide, please.

Some of the disruptions we saw in the first quarter were HAE market-wide, and they also affected our competitors. And as indicated in the 1Q call, they were transitory. In the second quarter RUCONEST® performed well. The leading revenue indicators, including growth in unique prescribers, new patient enrollments and vials shipped to patients, were all strong. In fact, new enrollments of 70+ for both 1Q and 2Q, despite the 1Q market issues. And this really clearly reflects the underlying demand in the HAE market and for RUCONEST® specifically.

In the second quarter RUCONEST® revenues globally increased by 20% compared to the first quarter. Furthermore, we saw a 2% increase in revenues when comparing the second quarter of 2023 and 2022. So given the strong bounce back in the second quarter, which we did signal in the 1Q call, we're maintaining our outlook of low single digit revenue growth for the rest of 2023. Next slide, please.

This slide shows the number of unique prescribers continues to grow in the U.S. 687 represents 62% of the HAE prescribing community and this is still growing nine years post-launch, as Sijmen

mentioned earlier. And that's despite the significant changes to the market throughout those years. This clearly shows that despite the prophylactic launches and the genericization of Firazyr, there remains both the place and need in the treatment armamentarium for a C1 esterase inhibitor and the enduring need for RUCNONEST®. We expect to continue growing the unique prescriber base in the coming months and years. And, with it of course, the base of patients that benefits from RUCONEST®. Next slide, please.

Let's turn now to the Joenja® launch. As you would expect, Pharming has brought its A-game and all its rare disease commercialization experience to bear in the U.S., which, as with all companies, is a must-win market. We have 54 salespeople and leaders comprised of the existing RUCONEST® team where approximately 30% of APDS patients reside. And then the Joenja® institutional team, which joined us in August of last year. They focus on the Centers of Excellence to which the other 70% of APDS patients either already are being treated or will be referred to. These two teams, along with other colleagues that Sijmen had already outlined in an earlier slide, are tasked with both identifying patients and ensuring HCPs have of all the information and education they need to confidently prescribe Joenja®.

We also have, amongst others, clinical educators to drive family mapping and testing and Anurag outlined how important that is. And just to reiterate, APDS is an autosomal dominant condition so there's a 50/50 chance the siblings and the family members are also going to have APDS and so family testing is critical for both them and their welfare and the long-term growth of Joenja®. Which, as we know, is a progressive disease. Next slide, please.

Just briefly, before I pass over to Jeroen, a couple of things around the value proposition. You know, as Anurag already shared and we should just remind ourselves, this is the only indicated option for APDS patients. It is a precision medication and that's important: when a patient tests positive and a physician prescribes, both the physician and the payor know that they are prescribing a treatment that will actually work on modifying the root cause of disease.

Hence at Pharming we knew therefore that we were launching a product that physicians and their underserved patients would need access to and access to quickly. Let's briefly look at the impact on a patient. Next slide please.

This is John. He's a 20-something year old man who's been dealing with APDS since he was 11 years old. Like most patients he's been in and out of hospital, was having difficulty developing social skills, keeping friends and was basically willing to try anything to remove the stress and the burden of taking 11 pills in the morning and nine pills at night to try and manage his symptoms. Joenja® has removed that heavy pill burden and is treating the underlying cause of John's APDS. Since being prescribed Joenja®, John's life has already changed significantly for the better. His lymph nodes have decreased in size, and I think you can see from the thought bubbles his quote around that, and his perception that they have actually gone as opposed to just being reduced in size. And importantly, he started to think about his future and what he wants to do in life. And as you see here, that includes actually going to college, something he couldn't have envisaged previously. That's basically the promise that Joenja® can and will continue to deliver for our patients. And it's what will continue to fuel this launch phase and also our future growth. Next slide, please.

As we discussed in the last call, the preparation for this launch was very, very rigorous and thorough. And as expected, we are off to a very good start. Our first fully reimbursed commercial shipments of Joenja® occurred within two weeks of FDA approval, which is very quick and certainly unusual. And to date, we've enrolled 60 APDS patients and shipped to 43 of them on payor approved product, with the remaining 17 to the end of the first half in-process. With respect to market access, the teams are doing outstanding work and we continue to make good progress partnering with the national and regional payors, including state Medicaid programs, to prepare for clinical review and coverage policy development, many of which are now rolling out. And to date, no patient has been denied access to Joenja®.

As you can imagine, we're very pleased with the first quarter of post-launch progress and expect to build on our early success in the coming months to both get eligible patients on therapy and grow the patient funnel for future patients. And that will continue to bring growth and of course lay the foundations for Pharming's continued success into 2024. So now I'd like to hand over to Jeroen who will cover the financials.

Jeroen Wakkerman – Chief Financial Officer:
Thank you very much, Steve. Good morning. Good afternoon, everybody. Moving to the next slide on the financial highlights for the second quarter of 2023, our revenues grew to US$54.9 million, which is a growth of 9% versus last year. And the US$54.9 million consists of US$3.8 million of Joenja® sales in the first quarter that we report Joenja® sales. And US$51.1 million from RUCONEST® and that is a growth of 2% from last year's second quarter, but 20% from 1Q. So, a very strong recovery as Sijmen and Steve mentioned before as well from 1Q. Gross profits grew to US$49.2 million, which is plus 7%, which is roughly in line with the growth in revenues. Operating cost increased by US$23.4 million and that's on a number of items, mostly marketing and sales. But I'll give you some more detail later.

Important to understand for the second quarter is what we had in what we call other income. So just between gross profit and operating costs in the P&L, we had an income of US$21.1 million for the sale of the Priority Review Voucher to Novartis which was as per the agreed contract in 2019. And also, good to tell you that in the second quarter last year we also had some big other income that was connected to the BioConnection transaction, as you may remember, which brought us in US$12.8 million. To cut a long story short, the increase in other income from this year to last year was US$8 million. Then on the operating profit of US$5.3 million, that's a decline and that is roughly in line with the increase in the operating costs. And the net profit went from US$15.7 million to US$ 1.3 million, so a decline of US$14.4 million. And that is, by the way, an improvement from 1Q, where we had a net loss in the quarter.

Then if we look at the first half results on the next slide, total revenue grew by 1% to US$97.4 million. The gross profit, US$87.6 million with a gross margin of 90% was a good gross profit income. Operating costs, US$118.5 million from US$82.2 million last year. So that's an increase of US$36.2 million. More detail about that later. The operating profit was US$8.4 million negative, so an operating loss, and the net loss has been US$10.9 million so far in the year.

If we then look at the revenue breakdown for the last few quarters on the next slide. You see indeed the enormous growth from 1Q, and the drop obviously was from the reimbursement issues that were apparent in all the HAE market. But we showed that strong recovery of 20% in the second

quarter to US$51.1 million. And you see the US$3.8 million sales from Joenja® for the first time in our history.

Moving to the next slide on the costs because they went up significantly. Just a bit of color on where we spent the money. In the second quarter we had US$10 million of a milestone payment to be paid to Novartis related to Joenja®. And we had over time a substantial increase in marketing and sales to support the launch of Joenja®. Steve just mentioned that the launch preparation was rigorous and thorough and this was also what you see in the money that we spend on Joenja®. You see that is a fairly stable development of G&A cost and an increase in R&D in the second quarter of this year of US$5.3 million, which is largely related to the work we're doing on the approvals in several international markets like Europe, Canada, Australia and Israel and a buildup of our medical departments both in the U.S. and in Europe.

So apart from the cost categories, another way of looking at the increase in cost in the first half of this year, that was plus US$36 million. We spent US$10.5 million in total on the milestones. We spent US$7 million of the US$36 million increase on leniolisib out of pocket expenses, and think about R&D cost, but also marketing, market access and amortization cost of the license. And we added US$16 million of payroll and general expenses. Most of it is in payroll because we increased the number of people since last year from 319 at the end of the first half last year to 383 now. An increase of 64 FTEs and that is across the board, most of them in marketing and sales. But across the board we're growing the company as an investment in future growth.

But a short overview on the next slide of the cash flow. Over the last quarter, the cash and cash equivalents increased by US$8 million to US$192.4 million and that was mostly related to the sale of the Priority Review Voucher, which is considered a cash flow from investing activities.

So that was the overview of the financial highlights. Then going to the outlook for this year on the next slide. Driven by the strong recovery, we continue to guide on low single digit growth for RUCONEST® revenues for the full year. Joenja® was approved at the end of March by the FDA and we've been commercializing the product since April this year. We expect a CHMP opinion from the EMA in the fourth quarter of this year and the marketing authorization, subject to that positive outcome, two months later for Europe. In the U.K., we will be filing leniolisib with the MHRA following the European Commission Decision Reliance Procedure. And again, that is subject to the positive outcome of the EMA review.

We continue to invest in operating costs to accelerate the growth of Joenja® and RUCONEST®, and further details on the plans to develop additional indications for leniolisib will be provided later this year. And last but not least, we continue to look at investments on acquisitions and in-licensing of mid- to late-stage opportunities in rare diseases to build the company further. With that I want to move to the next slide and get back to the operator to operate the Q&A. And please feel free to ask any questions. Thank you.

Operator:
Thank you. As a reminder, if you'd like to ask a question today, please press star followed by one on your telephone keypad now. When preparing to ask your question, please ensure your headset is fully plugged in and unmuted locally. Press star followed by one to ask a question today.

And our first question is from Christian Glennie from Stifel. Christian, your line is open. Please go ahead.

Christian Glennie – Stifel:
Good afternoon, guys. Thanks for taking the question. I guess to start with Joenja® and just initially to clarify the 60 patients on enrollment versus the 43 on paid therapy. Is it the case that some of the other 17 are on therapy already, but still working through in terms of reimbursement, or how should we think about the balance of those 17?

Sijmen de Vries, MD – Chief Executive Officer:
They are, Christian. When patients actually are enrolled, they get a free starter pack which is a one-month supply of Joenja®. So yes, once they are enrolled, they have access to the medication, so they have it. And then of course, the administrative procedures to get them reimbursed start and if that happens within the month, the second month will be a commercial pack. And if that procedure is not yet finished then there will be another month's supply in the form of a bridging pack provided. So, to answer your question, yes, they're all having access to therapy once they are all enrolled. Hope that is clear to you.

Christian Glennie – Stifel:
Yeah, that's good. Thanks. And then in terms of the identified patient numbers, you've added 140 in terms of the 640 versus 500 previously reported. I noticed that the U.S. has stayed the same, so where have those extra 140 patients come from? Is that mostly these new international markets, Canada, Australia, Japan or where are they from?

Sijmen de Vries, MD – Chief Executive Officer:
Could you comment on that Anurag? Is that possible?

Anurag Relan, MD – Chief Medical Officer:
Sure. Hi, Christian. We're continuing to find patients in the U.S., but we're also finding patients in other markets. So that does include Japan, Australia and other markets where we intend to commercialize first.

Christian Glennie – Stifel:
Okay. Thank you. And then you've got 200 patients in the U.S. identified – for your 1.5 per million, in the U.S., there's 500 or so out there so that's already a 40% diagnosis rate. Where do you think that diagnosis rate could go over the next few years?

Sijmen de Vries, MD – Chief Executive Officer:
Would you like to answer that as well Anurag?

Anurag Relan, MD – Chief Medical Officer:
Sure. We definitely see this trending up. I think, when we think about markets in Europe and some other countries across the world where there are more centralized healthcare systems, we can see that the prevalence there already exceeds one per million. In the U.S., we have a more distributed and less centralized system, so the diagnosis of these patients and the care for these patients is a little bit more fragmented. And so, we expect that over time the diagnosis rate will go up as there's increasing awareness of the disease, but that there's also an available therapy for these patients. I

think that we expect that to increase, at the minimum by what we see in some other countries, but likely increase beyond that. And that's why we think we're being reasonable about an estimate of 1.5 per million. As I mentioned earlier, there's a significant population of patients that we found beyond the 200 in the U.S., for example, that have these Variants of Uncertain Significance, or what are termed VUS. And that's another pool of patients who have symptoms of a primary immune deficiency and have had a genetic test – oftentimes that's well before we've been involved in the picture. But they still don't have a clear diagnosis and I think this will likely lead to an increase in the number of diagnosed patients over time.

Christian Glennie – Stifel:
Sorry, as a quick follow up to that then: is it your understanding that those patients would also be appropriate for Joenja® even if they haven't got formally diagnosed with APDS currently?

Anurag Relan, MD – Chief Medical Officer:
Yes. That's correct. They would be appropriate for Joenja® because they would have APDS if that's confirmed. Right now, oftentimes they have symptoms and clinical manifestations of APDS and they have variants or mutations in one of the two genes that leads to APDS. The question is, are those mutations, are those variants, disease-causing? And that's part of the effort that we're helping clinicians gather information on, get access to further testing and help them resolve that question for those patients, because they're also, like I said earlier, sitting in limbo and don't have a clear diagnosis yet. If they get a clear diagnosis of APDS, then those patients would be potentially eligible for treatment with Joenja®.

Christian Glennie – Stifel:
One final one if I can before I get back in the queue. Is it possible to give any rough sort of guidance for Joenja® for the year, whether that's in terms of patient numbers or revenues? Obviously, you've got 60 patients in the program already, US$3.8 million revenue in the second quarter. The 60 patients is already a 40% penetration of the 150 patients in the US that are over 12. Any sense for penetration rates or patient numbers by the end of the year would be great.

Sijmen de Vries, MD – Chief Executive Officer:
Yes, I understand that, Christian. But you know, its early days, right? And yes, we're off to a good start and we would like to see a bit more development of the numbers. Obviously, the numbers are ticking up all the time. And we'll keep you updated on a quarterly basis for now until such time that we get a little bit more of feel for the market and the development going forward. And then we will start giving some different guidance. So for now, no, not yet. We have to be a little bit more patient for that. But we will eventually, of course, do that. But we'll keep you on a quarterly basis updated on the number of patients that are on therapy and are enrolled. Okay?

Christian Glennie – Stifel:
Yep. Appreciate it. Thank you.

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

Operator:
The next question comes from Joe Pantginis from H.C. Wainwright. Joe, your line is open. Please

go ahead.

Joe Pantginis – H.C. Wainwright:
Hey, guys. Thanks for all the details, I appreciate it. So, a couple questions on the initial dynamics of the Joenja® launch. So, I guess, and of course, prefacing it by this is all early still, what is the general time it takes or approximate time it takes to get drugs to patients once they're identified? And then second, when you look at these numbers, you know, the 60 and the 43, how many patients, have gotten the drug or are on the drug? Of the revenue number that you posted today, which, like you said, beat overall expectations, how much of that, if any, is based on just getting drugs into the inventory channel? Thanks.

Sijmen de Vries, MD – Chief Executive Officer:
I mean, that's an excellent question. Probably for Steve to go into a little bit more detail. Steve, could you this pick up?

Stephen Toor – Chief Commercial Officer:
Sure. Thank you, Sijmen. Morning, Joe. So, the first thing is what I alluded to, once the patient's enrolled, we get the starter pack out. And they're on therapy pretty quickly. And then in terms of the time it takes to get approval for patients, it's typically so far between four and six weeks, which is pretty quick. We've seen one or two is a little quicker or one or two is a little more. But think of it in a 4-to-6-week period. So, we're not having to bridge too many patients beyond the starter pack. And then in terms of stocking, it's almost zero. I mean, we put some in right at the very start. We were able to process patients very quickly after launch, which meant we burned through that very quickly. And the way in which our partner works, PANTHERx, means we're basically practicing just-in-time delivery. So, for the most part that revenue is generated by patient demand.

Joe Pantginis – H.C. Wainwright:
That's great to hear. Thanks. And then I feel I have to ask my next obligatory question that I usually go to: when you look at OTL-105, obviously, you're holding it close to your chest. But I'm curious with the ongoing preclinical models that you're doing, will we be able to see or when will we be able to see any of the data coming out of these models?

Sijmen de Vries, MD – Chief Executive Officer:
Would you like to comment on that, Anurag?

Anurag Relan, MD – Chief Medical Officer:
Sure. Good morning, Joe. So, we're making progress together with Orchard in those models. I don't have a firm timeline that I can say that this is going to progress, you know, in the next couple of weeks or not. But I can say that I expect probably toward later this year, we should be able to provide some further updates on where we are with that program, the data that those preclinical models are generating and how we plan to move the program forward.

Joe Pantginis – H.C. Wainwright:
Great. Appreciate the details and nice to see the strong launch on Joenja®.

Sijmen de Vries, MD – Chief Executive Officer:
Thanks, Joe.

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

Sushila Hernandez – Van Lanschot Kempen:
Yes. Thank you for taking my question. Also on Joenja®, you mentioned it takes 4 to 6 weeks to get approval, but could you also elaborate a bit more on time from identification to enrollment. Could you share a bit more on that? Thank you.

Sijmen de Vries, MD – Chief Executive Officer:
Steve, would you like to comment on that?

Stephen Toor – Chief Commercial Officer:
Yeah, thanks Sijmen. So, it's obviously variable, but relatively quick. Once a physician and the patient have that discussion, it's literally governed by the time it takes to complete the form, send it into the hub and then generate the starter pack. So, I mean, it's one day to a week, but really dependent on the speed at which that process happens. But it can be very, very quick, easily a day or two.

Sushila Hernandez – Van Lanschot Kempen:
Okay, that's clear, thank you. And just on RUCONEST®, you mentioned that there are over 70 quarterly new patients. Where are these patients coming from? Are these treatment-naïve patients, or did they switch?

Stephen Toor – Chief Commercial Officer:
For the most part, these will be switched patients. At this point in the evolution of the market, there are very few treatment-naïve patients. So, these are patients who are either not satisfied on their current acute therapy or require, as per the guidelines, a second acute therapy to make sure that they have what they need to deal with any breakthrough attacks.

Sushila Hernandez – Van Lanschot Kempen:
That’s clear, thank you. And then the final question: could you share progress on your BD activities? How is that progressing?

Sijmen de Vries, MD – Chief Executive Officer:
That's a good one, Sushila. We have a very active team. I think if I provide some numbers, they turned over 150 opportunities over the last 12 months. The vast majority, of course, were quickly disposed of. But then we have an internal committee, including Anurag and Stephen, who take a next look. And then out of that, a much smaller number, comes to the fore and we do some more analysis on that. We've done a few due diligences over this last year as well. So, think I can say the efficiency of the BD process has significantly improved over the last year. And, you know, we came very close a couple of times. But we're very careful, of course. And as you know, in business development, it's all or nothing, right? There's no deal or there is a deal. So, we remain optimistic about being able to acquire or in-license, preferably in-license, that's the preferred mode of action, another asset that is in mid- to late-stage development, so that we can actually plan for another asset to be launched in the not-too-distant future. And that's all I can say at the moment, I'm afraid.

Sushila Hernandez – Van Lanschot Kempen:
Okay. Thank you. Looking forward to updates on that front as well.

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

Operator:
As a reminder that's star followed by one on telephone keypad to ask a question. And the next question comes from Natalia Webster from RBC. Natalia, your line is open. Please go ahead.

Natalia Webster – RBC:
Hi there. Thank you for taking my questions. I just have two, one on RUCONEST® and one on leniolisib. So, on RUCONEST® we saw an improvement in 2Q and you've reiterated your guidance for the full year. But I was wondering if there's any risk of the reimbursement issues that we saw in 1Q recurring at all. And then also you mentioned strong market demand and continued growth in unique prescribers, but I was wondering if you're able to provide some color specifically around what you're expecting for 2H and in the near term into 2024.

Sijmen de Vries, MD – Chief Executive Officer:
Yeah, probably a good idea to let Stephen answer that question first. Okay?

Stephen Toor – Chief Commercial Officer:
So firstly, the event that happened in 1Q, we're not expecting a repeat of that. It was a very specific market-wide event that was unrelated to Pharming. It affected everybody and it was rectified towards the end of 1Q. My suspicion is that's very much a onetime event. I don't expect that to occur again in the future.

And then in terms of growth in prescribers, it's actually been very consistent for the last seven or eight years, certainly since I've been with Pharming and we retook control of RUCONEST®. There's still, as I mentioned, another 38% of HAE-specific prescribers in the U.S. who've not yet tried RUCONEST® and we continue to call on them as well as our core customers. So, at this point I see no reason, given the last seven or eight years, not to expect that same steady growth as we move forwards.

Natalia Webster – RBC:
Right. Thank you. And then just secondly, on leniolisib regarding the timing for this CHMP decision. You say that you expect it in 4Q of this year, but I think you previously said 2H 2023 so I'm just wondering if there's been any delay there or if you're just being a bit more specific?

Sijmen de Vries, MD – Chief Executive Officer:
You want to be answering that, Anurag?

Anurag Relan, MD – Chief Medical Officer:
Yeah, I think we're just continuing to work collaboratively with the CHMP and EMA and answering their questions. And I think we're just giving some more detail, as we have more detail around the timeline there, that's why we're saying the fourth quarter now.

Natalia Webster – RBC:
Okay, thank you.

Operator:
Next question comes from Hartaj Singh from Oppenheimer. Hartaj, your line is open. Please go ahead.

Fanyi Zhong – Oppenheimer:
Hi, this is Fanyi Zhong on for Hartaj. Congratulations on the good quarter for RUCONEST® and the nice start for Joenja®. I have a question on the APDS patients: how can we think about the growth rate in APDS numbers quarter by quarter? Is seasonality a factor to consider? Thanks.

Sijmen de Vries, MD – Chief Executive Officer:
So you asked, is there any seasonality in ADPS? Is that right?

Fanyi Zhong – Oppenheimer:
Yeah. I mean how can we think of the growth in ADPS patient numbers quarter by quarter? And would seasonality be a factor to consider down the road?

Sijmen de Vries, MD – Chief Executive Officer:
Right. Stephen, would you like to comment on that?

Stephen Toor – Chief Commercial Officer:
Thank you, Sijmen. I mean, obviously, it's an ultra-rare disease. We had a bonus of patients to start with, but we're still in the very much in the launch phase. So, we expect a steady growth as we move forward and really no seasonality. But obviously, in the outer years, you start to see the rate of growth slows you would do with any launch. But nothing specific in terms of seasonality where we would expect significant troughs or spikes. Does that answer the question?

Operator:
We have a follow up from Christian Glennie from Stifel. Christian, your line is open. Please go ahead.

Christian Glennie – Stifel:
Hi. Thanks. I thought it worth following up on the filed applications in Canada, Australia, Israel, and the potential approvals next year. What's the commercial strategy there, is it better to find partners? Is it worth doing yourself in some of these markets? What should we expect there? And obviously in Japan, you need to run a trial which is a bit further down the track. What's the strategy there?

Sijmen de Vries, MD – Chief Executive Officer:
Yeah. First of all, Christian, before I hand over to Stephen, there's no trial requirements in Australia and in Canada, we have submitted the files. We have received a priority review as well from those authorities. So, it's a matter of waiting for, first of all, the validation of the regulatory files, and then of course we'll go through that and then will await the opinion of those regulatory authorities somewhere, as alluded to, probably the first half of next year. Then I'll hand over to Stephen to explain how we are approaching the commercialization in those markets.

Stephen Toor – Chief Commercial Officer:
Thank you and hi, Christian. So certainly, I think to answer what's at the heart of the question, we have no plans to out-license in any of those markets and giveaway, frankly, the value of the product to other companies. The structure, though, and the go-to-markets with Joenja® would be different from, say, the US or Europe, where we have a pretty large footprint. So, it would be a light, cost-efficient footprint. And in somewhere, for example, like Australia, you would service that market directly with probably a hybrid model or a combination of directly employed Pharming employees and then local distributors and partners who can help us service certain elements of the Australian infrastructure. But to keep things really efficient from that market, you would also serve Hong Kong, South Korea, which is the third biggest market in Asia PAC and other smaller markets. So, the plan is very much for us to keep control of the product, keep control of bringing patients on therapy and obviously reaping the benefits of that for ourselves and our stakeholders. Does that answer the question, Christian?

Christian Glennie – Stifel:
Yep, that's very clear. Thank you.

Stephen Toor – Chief Commercial Officer:
Thank you.

Operator:
As a final reminder that's star followed by one on your telephone keypad.

As we have no further questions at hand, I will hand back to the management team for any concluding remarks.

Sijmen de Vries, MD – Chief Executive Officer:
Thank you very much. Thank you, ladies and gentlemen, for attending our conference call. And I would like to remind you of some of the elements of the outlook for the remainder of the year. You've heard all the positive developments on the leading indicators to the forward-looking indicators underpinning the sales of RUCONEST®, that we remain confident to continue to guide on the low single digit growth for RUCONEST® revenues for the for this year. You've heard about the steady flow of new patients coming in for Joenja® and of course the immediately available patients that could be brought to Joenja® therapy over the coming quarters. You've heard about the regulatory interactions that we have with the Europeans, the plans to follow up in the United Kingdom and the submissions to broaden our footprint with the submissions in Canada and Australia. And of course, that we continue to invest in the launch preparations in those markets, and the clinical trial plans that we have for the second indication of leniolisib, which we will update you on as and when we have received the feedback from the regulator on our plans that have been submitted with regards to both what the new indication is and that indication, by the way, as you already have learned from us, has a bigger patient potential than APDS. And we will reveal that later on during the year.

And last but not least, we hope to be able to come back to you during the remainder of the year, with in-licensing or acquisition news, because we are very keen and have the capabilities to further leverage our commercialization infrastructure that we have and that we are expanding towards the

coming years, including markets like Australia, Canada, as you just heard from Stephen, but also Japan, and to bring more products to those markets to further significantly accelerate the growth of our company going forward. So, thank you again for being at our conference and we look forward to updating you on the next quarter results somewhere in the last week of October. Thank you very much. Goodbye.

[end of transcript]