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PARTICIPANTS

Sijmen de Vries, MD – Chief Executive Officer

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Thank you very much. Welcome, ladies and gentlemen. I'm happy to take you through the Pharming story, but before I do that, I would like to point you to this slide where you see that I'll be making some forward-looking statements that are based upon our plans and perceptions of the markets, which may obviously be materially different in the future.

So, we are going to build this leading global rare disease company and we have a very nice position in that respect because we have RUCONEST®, which is a product that comes from our own research platform that is in the U.S. market and is made last year more than US$227 million in hereditary angioedema. It's approved for acute treatment of hereditary angioedema attacks, and it continues to grow after 10 years of being in the U.S. market.

And you saw the revenues in the first quarter being US$46 million, up 8% versus last year. Despite increasing competition, we continue to see an increasing number of patients and prescribers for RUCONEST®, because RUCONEST® really fulfills and continues to fulfill a big unmet medical need in that hereditary angioedema market.

In addition to that, we in-licensed from Novartis back in 2019, an ultra-rare disease compound, Joenja®, leniolisib for a newly discovered disease, a primary immune deficiency, APDS, activated phosphoinositide 3-delta kinase syndrome. And we launched that product last year in the U.S. market, and had for the first three quarters, US$18 million of revenues and for the fourth quarter in the market almost US$10 million of revenues, so US$28 million in the first year in the market. And like I said, it's an ultra-rare disease, about 1.5 patients per million population. So, it means you have to do a patient finding, and that is what we do. And I will elaborate a lot more on that later on.

We have a lot of regulatory reviews ongoing for that. We recently also had an approval in Israel for 12 and up. We have some pediatric trials and we also are branching out to Japan for that compound. In addition to that, we have found a secondary indication in that PIDs with immune dysregulation indication, three times as big as APDS. And we will be starting a Phase II study soon. I'll elaborate on that a little bit more as well.

On top of that, we have a very scalable commercialization infrastructure and therefore are looking to expand that because we have proven to be good at commercialization in those rare and ultra-rare diseases. That's why we're focusing on rare or ultra-rare opportunities in clinical stage development or beyond.

And we have given guidance this year of revenues between US$280 million and US$295 million, driven by Joenja®, of course, supported by RUCONEST®. And this is what our pipeline looks like. It's really all about RUCONEST® in the U.S. and Joenja® or leniolisib expanding and driving growth for the coming years.

And if you look at it in a slightly different way, you see here Joenja® for APDS in the market. A significant portion of the identified patients already on paid therapy tells you that's a serious disease and a very good drug. And there's a lot of ongoing work finding patients and especially clarifying the full breadth of the disease, because the disease, as a new disease, is not fully described.

So, when you do genetic testing, you get a lot of variants of uncertain significance which have to be clarified. And that's a lot of work going forward but will yield a lot of patients. Then going forward you will see a global expansion. And there's already a lot of patients on named patient programs and paid or unpaid access programs outside of the U.S.

Pediatric studies are ongoing to complete the label. And then, like I said before, there's a big inflection point ahead of us in a couple of years. That is a secondary indication that has a similar symptomatology to APDS and will drive the growth of Joenja® significantly forward.

So let's look at RUCONEST® briefly. Like I said, RUCONEST® has been approved for 10 years in the U.S. market and we have seen a lot of competitive entries. And most of the U.S. patients nowadays are on prophylactic therapy for hereditary angioedema. That means that the mode of action of almost all those prophylactic and new acute therapies coming through and are serving mainly in the middle of the slide, a kallikrein independent pathway. That means that these compounds are efficacious and patients have basically benefited from a lot better prophylaxis for these horrible attacks over the last few years.

However, there is still a significant number of patients, up to 50% on the best-in-case, and up to 80% in some of the other cases of prophylactic therapies that have breakthrough therapies from time to time, some very frequent, some not very frequent. However, this means that every U.S. patient will always have acute therapy at hand to be able to deal with those unexpected breakthrough attacks.

And that is where RUCONEST® plays a role, and continues to play a role, because RUCONEST® is the only C1 esterase inhibitor that is on the market, currently actively marketed, and that is basically the missing protein. So RUCONEST® covers all those three pathways. Hence why you see in the results of RUCONEST®, you see nothing about breakthrough attacks or something, but you see that you cannot get any closer to 100% efficacy than with RUCONEST® because it's the missing protein.

That also means that the patients that we mainly serve are on the severe end of the market i.e., they have failed on all therapies and have to rely on a product like RUCONEST®. And RUCONEST® is not a convenient product like most of the new therapies that are oral or that are subcutaneous every two or four weeks. RUCONEST® is a slow IV injection that patients inject themselves. We train them, we help them, we support them, and they're very confident with that. And that is also

because they have to rely on RUCONEST®. And RUCONEST®, therefore, serves patients that mainly have failed on all other therapies.

And that is actually a notable difference from the new trials that you see, for instance, there is some orals coming to the market for acute therapy as well, where you see they have excluded in their clinical trials patients that are, for instance, not responding to the most commonly used acute therapy, icatibant that is mainly serving that middle pathway.

So, RUCONEST® is really serving a very specific segment of the market, apart from the fact that it also is being used for breakthrough attacks on the prophylaxis. So therefore, we see for RUCONEST® a long-lasting presence in this market, because there's nothing else really that actually stops the attacks so reliably as RUCONEST®. So RUCONEST® continues to be the sort of cornerstone of our company from which we can invest the excess cash flows, as it is highly profitable because it's a 90% gross margin, into new products such as Joenja®.

So let's look at Joenja®. How is this going to drive this? So, let's first look at the disease here. It is a recently described disease, hyperactive pathway, PI3 kinase delta pathway. And it basically upsets the immune system such that the immune system does not produce functioning T and B cells. And that relates to a whole cascade of symptoms that you see on the right-hand side of this slide.

Basically it says, you cannot defend yourself against infections, so severe and recurrent persistent infections. The immune system is also in overdrive because it creates those cells that don't function and there's no signaling back and the hyperactive pathway doesn't stop it. So, you get the lymphoproliferation in all sorts of organs, lymphadenopathy, splenomegaly, hepatomegaly, and of course those tend to be going malignant at some point in time.

So in other words, these patients are on a downward spiral towards lymphomas, which is associated with a very high mortality. And there's nothing that stops it, basically speaking, because that pathway continues to be hyperactive. You can try to manage the symptoms, of course, with lots of IVIG infusions with sirolimus, for instance, but it doesn't really stop the cascade going down, and that's where actually leniolisib plays an important role. Also, these patients can have weird enteropathies, autoimmunity and bronchiectasis for a long time. So it's a miserable disease that actually hits young adults and children and is on a downward spiral with a high mortality.

Now, leniolisib or Joenja® is unique because it's the first PI3 kinase delta inhibitor that was approved on the basis of double-blind placebo-controlled trial and met all the endpoints, and also has a very benign safety profile. And that's not where necessarily the PI3s are known for, but this is the exception here. And what Joenja® does is it modulates back that hyperactive pathway towards normal levels, such that the immune system doesn't stay in overdrive and starts producing normal immune functioning cells. And that means that over time, the immune system restores and you see all the symptomatology turning back to normal. And we have very long exposure data already.

We have patients up to eight years already on drug currently, and we continue to see the symptoms improving and the tolerability stays the same. So Joenja® is a real disease modifying drug, and that for a disease that was only discovered 10 years ago, and here it's very simple, it works, it doesn't

stop and block the PI3 kinase delta pathway. It brings it back to normal activities such that the immune system can start functioning again.

Now, of course, a new disease, ultra-rare, that is not an easy job because there's no competition, but you have to find the patients, and that's what we do. We do a lot of medical education for those different types of physicians where these patients can be hiding, mainly immunologists, of course, but also in hematology, pulmonology and gastroenterology. We have an extensive genetic testing program going on where we sponsored and provide in the U.S. free genetic tests, and that yields patients. That doesn't yield a massive number of patients, but it does yield patients that are coming into our patients group.

Because the U.S. is a very diversified landscape with regards to these patients. Outside of the U.S., it's a lot more centralized and these patients are more easily found. There's also no systematic approach to family testing in the U.S., to our surprise. So, we have started to actively approach the individual patients that we have identified already and offer them genetic testing as well for their families, because it's a dominant autosomal mutation. Therefore, there will be family members from those patients that already have been identified, and we are starting to find them.

And then last but not least, a very important aspect here, the VUS resolution. The disease is just not yet fully described. In other words, we have, of the 220 patients that we have already identified, we have in excess of that 1,100 patients in the U.S. that have the symptoms of the disease, the signs and the symptoms, they have a positive mutation in the relevant gene, but it has not yet been identified with APDS. And that is what we're working on.

We're working on that at this point in time in individual batches to clarify those. Then we will see a significant percentage of those patients turning into APDS patients in the future. And we'll have a full description of the gene by the end of the year by means of the MAVE study, that you see on the bottom of this slide here. And that will clarify fully which mutation is basically associated with that hyperactive pathway and therefore will become eligible for Joenja®.

In other words, we will clarify that this year and that means that next year the U.S. will be seeing a big bulk of patients coming in that are confirmed to have APDS. And this is not only for the U.S. because once this is confirmed the public databases will be updated. And all over the world, of course, everybody who will have such a sub-mutation, that's currently not yet confirmed, will be confirmed and will be eligible for Joenja® treatments.

So, we see a growth story that is panning out currently on the left-hand side. You see there with the 220 patients that we have found so far in the U.S. of which more than 50 are below 12 years old, so not yet eligible. More than 50 are diagnosed and we still are actually chasing them and getting them on therapy. We had reported 83 patients on drug after the first quarter of this year. And like I said, the VUS will be a big boost to actually get a full description of the disease next year and will drive a big bolus of patients into becoming eligible for Joenja®. Outside of the U.S. of course, there's global expansion going on. There are a lot of regulatory actions.

The pediatric studies will start reporting next year. So that will be a growth story for 2026. In addition, you see there's a considerable number of patients that we have found in excess of the

U.S. patients i.e. more than 600 already outside of the U.S. of which more than almost 140 are already on either paid access therapies or unpaid access therapies. And you see further down the line, that's the growth story for, let's say 2027 and beyond, a huge inflection point potentially for the PIDs with immune dysregulation as a secondary indication.

You see the pricing point here, it's an ultra-rare disease. We price it to more or less middle down the fairway in the U.S. and expect this pricing point to be the same, of course, for that secondary indication, because obviously that is also still an ultra-rare disease.

Now let's look at the geographic expansion here. We have a lot of regulatory interactions ongoing. We recently had an outcome from the CHMP review that basically confirmed the clinical benefit of Joenja®. However, they wanted us to go back with our starting materials to have more GMP steps involved. And whilst we clarify that, they gave us an 18-months clock stop so we can submit the replies for than and expect a positive opinion from the CHMP in the first quarter of 2026 on the basis of the confirmed benefits, and the agreed changes to the manufacturing process.

We received marketing authorization for Israel recently. We have a Japan study completed and we expect to bring the file to Japan either at the end of this year or beginning of next year. So, Japan will start coming online from 2026 as a growth opportunity. We have a UK submission where we submitted the FDA file with the UK authority, so we expect to have regulatory actions this year. And Canada and Australia, we also have regulatory actions going on for this year and respectively next year.

Pediatric I was already explaining to you, the major pediatric study is the 4- to 11-year-olds, which has completed enrollment now. So again, it will continue to bring patients in 2026 and beyond and there is a smaller pediatric study going on as well, which we'll report slightly later.

I was already alluding to the number of patients that are continuing to come on the expanded access and named patient programs. And of course, last but not least, we have the secondary indication. And the secondary indication is just an enlargement of the APDS indication. It has the same symptomatology. It has, though, a number of genetic mutations that are already associated with the hyperactive PI3 kinase delta pathway. So, we will not have to go on a patient search as intensive as APDS, because those patients have already been identified.

And this is clearly you see here more or less the same picture. It's again the hyperactive pathway that actually stopping the immune system from producing these functioning immune cells. And you see here that we're talking about well described mutations such as ALPS-FAS, CTLA4 and PTEN and that actually bring patients down the same cascade. And again, we expect Joenja® to have the same clinical benefits as they have in APDS because we are, again, basically modulating back the overactive immune system here towards normal, such that these patients can also reverse all those symptoms and can have a balanced immune system.

We do that in collaboration with the NIH. The NIH which were also one of the discoverers of the APDS disease. Dr. Uzel here was the one that actually first described APDS, and Dr. Koneti Rao was the principal investigator in the Phase III study. They've teamed up again, and they have a big bulk of those patients, and they're going to do this dose range-finding study in twelve patients and we

anticipate this to start in the very near future and of course will report on that over the coming time as and when we see results, and then go back to the agency to discuss the Phase III pivotal trial, which will have to come after that.

And this is basically speaking to illustrate the point here that, yes, this is still an ultra-rare disease but this is not a very small numbers of patients. You can see here well in excess of 1,100 patients that already have been confirmed in various publications with these mutations. And therefore we think this is going to be a significantly bigger opportunity for the company to expand going forward.

So finally, let's briefly look at the financials of the company, because I was already alluding to the fact that we are very grateful, of course, and very proud that we have our asset RUCONEST®, which continues to grow after ten years in the market. You see that illustrated here. In December 2016 we bought back the North American rights from Valeant that started driving revenues, and you see that we have not looked back since then.

And of course, we have now embarked, since 2023, on a long trajectory of growth driven by Joenja® and the various expansions of the indication, as I was alluding to. Of course, this comes with investments as well whereas we turned very profitable very quickly after we started marketing RUCONEST® in the North American market. Over the last few years, we have started to significantly invest in the preparations for the launch of Joenja®, and we're in full launch mode, as you can see here with regards to the OpEx development versus the sales.

Now, I would like to note here that for Q1 we always warn for fluctuations in sales of RUCONEST®. Q1 is always the quarter where the annual prescription renewals are actually taking place in the U.S. So there's always a wobble there but you see that compared to last year, we significantly grew RUCONEST® in that significant quarter.

It created a loss, of course, in the beginning, but we can handle it. As you can see here, our cash and marketable securities give us a very nice basis to actually continue to invest with the expansion of Joenja®. And here you see that Joenja® is, ahead of approvals outside of the U.S., already basically starting to create revenues from the paid early access programs that are existing in a number of, also in European, countries that are continuing to go ahead.

And in European countries we have identified already in many cases more than one per million patients. So that is actually much more organized and centralized. And we expect those early access programs and the named patient programs that are outside of Europe and in Southeast Asia that already going and the revenues to continue to increase as well. And there you can see that Joenja® will start to become a significant driver of the growth of the company.

And that is what we are guiding for. As I was telling in the beginning, we guide for significant continued growth this year and of course ahead of the big bulk of new patients and accelerated growth that we see in the U.S. for next year, because we will continue to, of course, invest in that clarification or that full description of the disease APDS, by means of the VUS validation efforts.

We continue to work on growing the business ex-U.S. ahead of the regulatory approvals and after the regulatory approvals. We work on the continued clinical trials and most notably also working

towards submitting the file and getting hopefully a regulatory approval in Japan. That will be a very significant step for our company going forward to go into the Japanese market, and of course we expect those regulatory actions in the other areas.

And we look forward to starting the Phase II trial in the new indications. And last but not least, we have a very scalable commercialization operation. We've proven that we can actually commercialize against fierce competition in the hereditary angioedema market. We also are proving that we can actually develop a new market, such as APDS. So we are in the market, and we are beginning to get a lot of traction to look for acquisitions, or in-licensing of additional rare disease opportunities.

And we are looking at this point in time, because we have this commercialization machinery for clinical proof of concept, or beyond that assets that we can actually bolt on to our existing commercialization infrastructure, to accelerate the further growth of the company, other than with Joenja®, and secondary indications, of course, and basically with a solid foundation, and the continued growing foundation of RUCONEST®.

So, this is the story of Pharming at this point in time. And I think we still have two minutes, if there's anybody who would like to ask some questions. Thank you very much. Any questions that I can answer?

[Question inaudible]

Sijmen de Vries, MD – Chief Executive Officer: We don't expect RUCONEST® to go generic. RUCONEST® will lose exclusivity in 2026. RUCONEST® is produced from the milk of our transgenic rabbits. And C1 inhibitor is a notoriously difficult protein to express. So, we have no inclinations whatsoever that anybody is even trying to, because we tried many ways as well, trying to get anything with a C1 inhibitor done. So basically, the exclusivity is a non-event, and there's no indication whatsoever that there's a biosimilar coming into the market. Apart from the fact, of course, that RUCONEST® is not such a big product, that it is worth your while to do that. So that's basically the answer. It's an irrelevant moment.

Does it answer your question?

Sijmen de Vries, MD – Chief Executive Officer:
Good. Thank you. Okay, thank you very much for your attention, and goodbye.

[END OF TRANSCRIPT]