Pharming Group N.V.
Jefferies Global Healthcare Conference 2025 – Fireside Chat

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PARTICIPANTS
Fabrice Chouraqui – Chief Executive Officer

Benjamin Jackson – Analyst (Jefferies):
I'm from the European biopharma team here at Jefferies. It's a pleasure today to be joined by Fabrice, CEO of Pharming for this fireside chat. Look, Fabrice, let's just dive right into it. I think if I hand over to you for a few introductory remarks, and then we'll kick off with a bit of Q&A.

Fabrice Chouraqui – Chief Executive Officer:
Absolutely. Thank you so much, Ben. Thank you for being with us today.

So I'm the new CEO of Pharming, joined about three months ago, spent the past five years at Flagship Pioneering in Boston. And prior to this, 10 years at Novartis and 10 years at BMS. I was the President of the U.S. at Novartis.

I mean, the fact that I had the opportunity to enjoy, I would say, the rigor and sophistication of big pharma and the nimbleness and value creation focus of biotech and specifically a biotech fund like Flagship, I mean, led me to Pharming. I see a lot of value to unlock. I mean, this is a company that's developed extremely well over the past decade or so. I'm very fortunate to inherit a very strong growth platform with steady revenue stream, very strong growth, I mean, you've seen the result that we've posted in Q1, plus 39% versus last year.

And also and potentially most importantly, a very, very strong late-stage pipeline. Not early stage asset, but really either a new indication on, or proven I would say, a drug like Joenja® already on the market. And also a very, very exciting opportunity with Abliva, which is in a registrational trial, having gone through already a positive interim study.

So very excited by the opportunity and obviously looking forward to interact with you, Ben, today.

Benjamin Jackson – Analyst (Jefferies):
Brilliant. Let's start on that then. Obviously, since joining as CEO earlier this year, what has been most striking to you about the company? And then if we do the flip side as well, what do you think is what you would like to deliver now that you are more familiar with the setup of the firm?

Fabrice Chouraqui – Chief Executive Officer:
Yeah. So as I said, I mean, I think, we are fairly, uniquely positioned in this environment, being a biotech with a steady and predictable revenue stream, strong growth in the short and long term, but also a very strong pipeline. So for me, it's a little bit the best of the two worlds in terms of the predictability of what you can call a small pharma, but really the value creation potential of a biotech. And I really see Pharming as a biotech.

And to perhaps to answer your questions, Ben, for me, it's very much about developing Pharming as a large, small company as opposed to a small, large company. And so, if there are any adjustments to make to make sure that as we transition to the next level, we remain really very close to our entrepreneurial roots, and develop as a large, small company and maintain this biotech mindset. That made Pharming a successful company so far.

Benjamin Jackson – Analyst (Jefferies):
Brilliant. And before we jump into the drugs, I want to touch on your recent first quarter results. You did raise the revenue guide. Can you tell us a little bit about what's contributing towards that momentum that you're saying?

Fabrice Chouraqui – Chief Executive Officer:
Yeah. I mean, it's very healthy growth from the two assets we have on the market, RUCONEST® and Joenja®. So, the underlying demand is very strong. I mean, RUCONEST® has been on the market for 10 years and has a very, very significant prospect in the future. That's also something that I was able to see in my first few months is that RUCONEST® is far from being at the end of its life cycle. It's a drug that may well be actually – and may remain a cornerstone of the HAE on-demand treatment for the next few decades.

And when you come to Joenja®, I mean, it's a drug which is at the very beginning of its life cycle. I'm sure we'll have the opportunity to talk about this. So, stronger underlying demand on both generate actually strong growth and, therefore, a strong quarter.

Benjamin Jackson – Analyst (Jefferies):
Makes sense. Let's start on RUCONEST® since we're there anyway. I guess you've been quite clear about your expectations for this year, especially when we're considering facing the potential approval of an oral version around middle of the year. So what underpins that confidence that RUCONEST® can continue growing at the high single digit percentage rate in the face of that competitor launch?

Fabrice Chouraqui – Chief Executive Officer:
I think what makes RUCONEST® unique, is its value proposition to patients, which is linked to its unique mode of action and its efficacy profile. So it's a drug which over the years, has carved out a sub-segment of the HAE on-demand market and being used mostly by moderate to severe patients who have more severe, more frequent attacks. And these patients often have gone through other treatments. They failed other treatments and really like the high efficacy of a drug like RUCONEST®, allows them to get their life back.

So that's why you see a lot of stickiness when it comes to the prescription. You can see that – even actually with the prospect of new drugs being launched, quarter-after-quarter, there's still more doctors prescribing the drug, there's still more patients using the drug, given its highly differentiated profile in this segment.

Benjamin Jackson – Analyst (Jefferies):
And then what about beyond this year? Are all of those reasons relevant for 2026 that you believe that you can continue to grow strongly in similar fashion?

Fabrice Chouraqui – Chief Executive Officer:
Absolutely. Obviously, I mean, when you have new drugs being launched, you always see some short-term disruptions. And so, I don't have a crystal ball, I can't say how it is going to pan out. They're always, especially I mean, the U.S., a lot of free drugs floating around. Although I doubt that actually many patients who had actually a long journey to end up with a drug that can control their attacks will switch.

In the long run, I actually even think that this new treatment could actually be an opportunity for RUCONEST® to grow even further. Why is that? Because when you think about the on-demand market, you have a very established brand, and it's not a very dynamic market. Actually, very few doctors are engaging with their patient to know whether they are controlled. And so with new drug, you see actually a much more dynamic market, more switches, doctors actually spending more time with their patients, understanding whether they're controlled or not. And RUCONEST® is being seen as the high-efficacy treatment. So I'm really looking forward to this market becoming a bit more dynamic, and I see that as an opportunity for RUCONEST® in the long run.

Benjamin Jackson – Analyst (Jefferies):
I guess, are there any other growth levers to round it off that could be available for RUCONEST® in the near future?

Fabrice Chouraqui – Chief Executive Officer:
Already, I would say, if we can see actually more sub-optimally controlled patients being treated with RUCONEST®. I think that could be great for the drug.

Benjamin Jackson – Analyst (Jefferies):
Yeah, that makes sense. And look, let's move on to Joenja®, then. Do you want to just say a couple of words, just to set the scene for people who may be a little bit newer?

Fabrice Chouraqui – Chief Executive Officer:
Yeah, so Joenja® is a drug that treats APDS. It's a rare disease, an ultra-rare disease, affecting about one patient per million. I mean, what's very exciting with Joenja® is the long-term potential of the drug. So today, we have a label which is limited to the adult APDS population, so we saw a good uptake in the first year of the launch, and as usual for an ultra-rare disease drug, after an initial bolus of patient, then you are in the trenches. You see, you always have a small portion of the opportunity, those patients are well-identified that actually switch to your drug quickly, and then afterwards, you have to work to identify those patients and get them on drug. That's what we're doing. And we're very happy to see that actually the work is bearing fruit. In Q1, we saw a real acceleration of new patients on the drug.

And there are actually new opportunities coming, new growth catalysts. We will see the reclassification of VUS patients coming in the second part of the year. These are patients who got a genetic test, but for whom this test was inconclusive. And there'll be new data being published very soon that shows that actually a portion of these patients, about 20%, are actually APDS patients. So that's going to be another opportunity to expand the addressable population. It will not happen overnight. The same way we'll see a bolus of patients. But again, that's going to fuel the – it's going to be an additional growth lever that's going to fuel the growth for some years. And

then in early part of next year, we'll also expand our label. We expect to expand our label to the pediatric population, and that's going to be also the source of another lever.

So in the short term, a drug that will have actually a number of growth catalysts, and that should result in the acceleration of the uptake. In my opinion, that's traditional with what you see with an ultra-rare disease drug, as opposed to, I would say, a classic specialty or primary care drug. I mean, primary care drug, often you wait for access, so it's flat for a long time, and then you get the uptake or the specialty care, where actually it's more linear, I think, it's a drug which meets perfectly, actually, the uptake trajectory of what I believe is a strong value creation story for an ultra-rare disease drug.

Benjamin Jackson – Analyst (Jefferies):
That makes sense. And look, I think we've previously spoken about that there's about over 1,300 patients in the U.S. with one of these VUS test results, and perhaps you've just said 20%, which potentially could be reclassified depending on the publication. But could you outline what the next steps are in a tangible matter for this VUS in terms of the process required to begin converting the patients from the second half of the year?

Fabrice Chouraqui – Chief Executive Officer:
Absolutely. So as I said, again, these are patients who had a test, so they have been identified. Their doctors thought, actually, they may have had APDS, but the test came out inconclusive. So, now the new data that's going to be published in a top peer-reviewed journal anytime soon, says that actually 20% or so of those patients are actually APDS patients. So it's going to work. I think the genetic testing labs are going to incorporate those results into their model. They are going to look at their patient database, reclassify those patients that have to be reclassified, reach out to their doctors, and the doctors will have actually to reach out to their patients.

So that's why the process will go fast for some of the patients, it may take more time with others, but ultimately, it's a significant pool of patients that will be added onto the drug.

Benjamin Jackson – Analyst (Jefferies):
Makes a lot of sense. And look, I guess the last time we caught up in London, we were talking a little bit about the UK potential launch of Joenja®. So, could you talk what the initial feedback has been there from the physicians that you've been seeing in the region?

Fabrice Chouraqui – Chief Executive Officer:
Absolutely. I think I told you about some growth catalysts, inherent to the drug per se. There is another one as well in the near term, which is actually the geographic expansion. I mean, today, although we are a Dutch company, the bulk of our revenues are coming from the U.S. And so we are using Joenja® to expand geographically. We're doing that in a financially disciplined way, we've targeted actually eight countries in which we believe it makes actually sense to build operations. And the first one is the UK. I mean, we've launched a drug a month ago. I was there. I was very impressed with the engagement of the doctors. And so the first feedback I'm getting is very, very encouraging.

So again, we are expecting a substantial amount of patients to come from countries outside of the U.S., these key eight markets, but also outside those eight markets. We won't have presence, but

obviously, through access programs and potential local partnership, we expect to make the drug available in a financial, sensible way.

Benjamin Jackson – Analyst (Jefferies):
And two of the growth levers that we've got for Joenja®, we've got the first one, obviously, the U.S., second one, geographic, but arguably, there's a third one in there as well, which is potential expansion to very similar diseases. So, could you talk a little bit about what these diseases are and the relative prevalence of them, and also what data is supporting that transition into these alternative areas?

Fabrice Chouraqui – Chief Executive Officer:
Absolutely. I think this is actually the big opportunity. I've just described actually short-term growth catalysts that will fuel the uptick of the growth, but Ben, what you're describing, these potential two new indications could propel the brand to a whole new level. And here, we're talking about primary immunodeficiencies with immune dysregulation and CVID, common variable immunodeficiency, also with immune regulation.

So these are patients who have very similar phenotypes to APDS patients. Their phenotype, actually their clinical symptoms, is linked to a dysfunctional PIK3 delta pathway as well. And we have a handful of these patients in our Access Program, and we've heard, actually, very encouraging feedback from doctors. So, obviously, you cannot conclude on just a few patients, that's why we are carrying out these two Phase 2 proof-of-concept trials, but we have very high expectation for these two studies that we'll read out next year.

Benjamin Jackson – Analyst (Jefferies):
Very exciting. So, look, we've spoken a lot about volumes and areas for volume growth, but we also have to obviously touch on pricing as well. And I think in your first quarter release, you did mention that the gross-to-net adjustments for Joenja® were a bit of a drag on the growth. How is this net price of Joenja® expected to evolve throughout 2025 and beyond?

Fabrice Chouraqui – Chief Executive Officer:
There was a bit of impact, and it was linked, actually, to Medicare. Joenja® sets about 45% commercial, 45% Medicaid, and so 10% Medicare. Even though Medicare is a small portion, as you know, there has been, actually, some change in the Medicare program, and that has a small impact, actually, on the gross-to-net, an additional 2%. So, that was actually a one-off, very clearly with a clear rationale. I don't anticipate any additional changes during the year. This happened the first quarter, and that's it.

Overall, I mean, what I can say, again, that, I mean, we have a label that will allow us to actually fuel the growth of the brand before those potential new indications, and with those two new indications we have a drug which, potentially, could well become, actually, the first billion-dollar drug for Pharming.

Benjamin Jackson – Analyst (Jefferies):
Good, good. And look, we'll move on and switch gears a little bit to Abliva, I think, that you recently acquired. So, the primary asset in there is KL1333, assuming that's how you guys refer to it, as well.

Fabrice Chouraqui – Chief Executive Officer:
I promise you we'll try to find a better name.

Benjamin Jackson – Analyst (Jefferies):
So, that's for the primary mitochondrial disease. So, what do you – when you were assessing that asset, what did you find particularly attractive about it and what led to that decision?

Fabrice Chouraqui – Chief Executive Officer:
primary mitochondrial disease, mtDNA, are clearly a group of disease which are extremely debilitating. That doesn't allow patients to live a normal life. Actually, there is a significant prevalence, I mean, just U.S. top five EU markets, we're talking about 30,000 patients. These are patients who are well-identified, treated in centers of excellence, including in the U.S., who often actually are part of an advocacy group.

And so, what really excited us is, obviously, first and foremost, the unmet need. I think that's why what we live and breathe at Pharming is being a rare disease company, is being able to provide very transformative treatment to these patients. So, that fits completely with our mission. That fits completely with our capabilities as well. I think we have developed amazing capabilities when it comes to clinical development, access, supply chain in this rare disease, which is not necessarily straightforward.

And the potential is there. I mean, this is a drug for which you have a very consistent set of data, preclinical data, animal, so in vitro, in vivo, Phase I. And then this is a company that we've been following for years, we've been following that program. And we could see that they had this registrational Phase II ongoing, and after they did this interim analysis and show that this interim analysis was positive on the two endpoints, two endpoints which were actually agreed with the regulators, so no biomarkers, real endpoints, we felt actually that we had a real opportunity to supplement our pipeline with a very high potential asset, and so that triggered actually the acquisition.

Benjamin Jackson – Analyst (Jefferies):
That makes sense, and I guess, as you say, you've had the interim analysis, we're still waiting for the full results from that trial. So, could you talk to us a little bit about how the design of the trial actually positions the asset versus competitors in that landscape? And then secondly, as a second part of that, what would a successful trial look like, I guess?

Fabrice Chouraqui – Chief Executive Officer:
So, it's a trial which will enroll about 180 patients. There has been an interim analysis being done on 40 patients, again, so it's really not negligible. There are no actually approved treatment for mitochondrial diseases at the present time. So the unmet need is very, very significant. As I said, the endpoints have been pre-agreed with the regulators, which is important, so there is no misunderstanding downstream. And so, we expect to complete the trial in 2027, and expect a launch probably early 2029.

Benjamin Jackson – Analyst (Jefferies):
That makes sense. And I guess, to what extent does Pharming's existing commercial set up support or provide synergies for what may be required if that trial is successful? And then how much additional investment could be required to set that up through to them?

Fabrice Chouraqui – Chief Executive Officer:
So we've said that we would completely self-fund this acquisition and the development, as well as the pre-launch of the drug. As I was alluding to at the very beginning, I think that's the great thing about Pharming is that, I mean, we can self-fund our development. We have a very robust cash flow coming from RUCONEST®, and so in my opinion that makes the company stand out in the current environment.

Given our capabilities, I mean, as I said earlier, there's a real fit, I think, when you think about those diseases often, I mean, people often ask me, oh, it's going to be a different salesforce, or it's going to be the same salesforce? When you think about your commercial resources, your commercial investments, nowadays, actually, the salesforce aspect is just actually a small portion. What makes or breaks, I would say, the success of a drug launch is very much your medical affairs capabilities and your access capabilities.

Two areas in which actually, we have demonstrated, we have a great track record, okay. RUCONEST®, Joenja®, so I would say those resources, we will not need more resources, and those people are very eager, actually, to start working on KL1333, again, one of the priorities to find a name. And so, it's clearly a product that will be value accretive very quickly for the company.

Benjamin Jackson – Analyst (Jefferies):
That's good. And I think your management is building a bit of a track record about their BD and M&A in the smart way and opportunistic way that they've gone about that. So, how do you view BD going forward? Is it something that remains a priority or something that's more opportunistic? So that's the first part. But I also would be interested to see what you're hearing or seeing about the actual BD and M&A environment as it stands, given the ongoing U.S. politics, which I promise you we'll touch on in a moment.

Fabrice Chouraqui – Chief Executive Officer:
Listen, BD is very much at the core of our development. The company made a conscious decision a few years ago to dismantle its research operation, feeling that we didn't have the critical mass. I mean, coming from being a scientist by training, having done actually five years of very intense research and taking off a great company in this field, I completely concur with the decision of the previous management.

So now, as BD is at the center of our strategy, I mean, it's not that we're going to be working hastily, and we need actually to do BD deals every year or we set targets. I think today, as I've explained, we have actually a lot to do. We have very clear and significant growth catalysts in the near term, we have a pipeline with a very significant value creation and some value inflection points in the near- and mid-term. So there is no urgency to act. Yet, it is at the center. So we continue to identify opportunities. We want to be disciplined, it has to be value accretive.

The company, as you've said, Ben, has redeveloped a very significant know-how in this field. And I know well, having done a lot of BD in my life, that these are our real capabilities that you develop. I mean, it's easy to do deals, it's not easy to do good deals. And I think companies are always very happy to be on the front page of the newspaper for their deals, but then afterwards, you need to see whether those deals have been value accretive, and very few are.

So I think we have, at Pharming, developed a track record to do value accretive deals. And I intend to pursue this mindset. And so we'll continue to do this. As I said, not a priority if we believe that we have to act on some opportunity that we follow, we will do so, but that's not the priority at the present time.

Benjamin Jackson – Analyst (Jefferies):
That makes sense. And look, the business is largely focused at the moment in the U.S. on the sales basis. I think your manufacturing is based in Europe. So that begs the question, how would you fare, or would you look to approach potential pharma tariffs? I know we don't have a crystal ball to see whether it's going to happen, but what are your thoughts around that?

Fabrice Chouraqui – Chief Executive Officer:
Sure. I mean, my thought is that obviously you have to be prepared. I don't have a crystal ball indeed. So, I don't know whether there'll be tariffs on drugs, whether those tariffs will impact rare disease drugs, when this may happen. Yet, I want to make sure that we are at the forefront of this and can mitigate the impact. When you look at our portfolio, I mean, when it comes to KL1333, and Joenja® we're talking about small molecules, so here it's fairly easy, actually, to make adjustments in your supply chain, in your manufacturing process. These are low-volume, small molecules and so this is fairly easy.

For RUCONEST®, I mean, the fact that RUCONEST® has, in my opinion, many decades ahead, is the very, very specific manufacturing process, proprietary, very complex, for those of you who don't know, I mean, this is a drug which is a recombinant protein made out of the milk of transgenic rabbits. So that, as you can imagine, cannot be transferred overnight. Now, it doesn't mean that there aren't aspects of your supply chain, of your manufacturing process, that you cannot transfer, that means that your entire supply chain cannot be transferred. There's still actually a lot of things that you can do to mitigate the impact of tariffs, and we are actually actively looking at it. And if tariffs were to kick in at some point, we'll be ready to mitigate their impact. But I want to manage expectations here. I don't want to trade the long-term for the short term.

I think we have created an amazing transgenic platform in Netherlands that actually will be the source of very significant cash flow in the long run that will remain. Now again there may be, there will probably some adjustment to the supply chain that can allow us to mitigate tariff on RUCONEST®.

Benjamin Jackson – Analyst (Jefferies):
All right, makes sense. Thank you. So that takes us to the top of the time. Fabrice thank you so much for joining me. It's been a pleasure as always. Thank you all for listening in. If you have any questions, please reach out to the team. Thank you.

Fabrice Chouraqui – Chief Executive Officer:
Thank you, Ben.

[END OF TRANSCRIPT]