Pharming Group N.V. conference call: Proposed acquisition of Abliva AB

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Sijmen de Vries, MD – Chief Executive Officer:
Thank you very much. Good morning, good afternoon, ladies and gentlemen. Welcome to this conference call where we will be discussing the proposed acquisition of Abliva AB, as announced yesterday.

And before I do that, I would like to go to the next slide with the forward-looking statements. Be aware, please, that we will be making forward-looking statements today, and forward-looking statements, of course, because we have, at this point in time, you know, ideas and expectations and plans, and those can be materially different from what can happen in the future, as we all know.

So let me go back one slide and introduce who's here with us. So, we have here Anurag Relan, our Chief Medical Officer; Stephen Toor, our Chief Commercial Officer, who will be speaking as well; and in the room here with me is my colleague Jeroen Wakkerman, our Chief Financial Officer, to help answer any questions that may arise. Let me start by going through to the first section of the whole thing, the strategic rationale. Please, let's forward to slide number five.

Thank you. So we're building this global rare disease company, and for those of you who have been listening to our webcasts, you're very familiar with this picture, at least most of it, where you can see clearly that we do that by means of Ruconest, which, of course, is our lead product, coming from our own research, where we actually are generating significant sales in the US market, which is a significant cash generator, and where we see continuing increase in prescribers and patients using it for treatment of acute attacks of hereditary angioedema.

And then, five years ago, we were able to in-license Joenja for the treatment of APDS, which has, in the meanwhile, been approved in the United States, United Kingdom and Israel, and is on the market in the United States, and where we expect to expand globally because we have regulatory submissions in, for instance, the European Union going on, Canada, Australia, and we're branching out to other territories with Joenja as well. Joenja is approved, of course, for patients 12 years and up, and we have pediatric trials and a Japan filing that we plan to do soon.

So that is our current business, obviously, and we've always said that we are hunting for new opportunities, and today is a very good moment because we've been able to identify a very

interesting product to broaden our pipeline. And we're talking today about KL1333 that is being developed by Abliva AB, a Swedish public company, for the treatment of mitochondrial DNA primary mitochondrial disease, and Anurag, our Chief Medical Officer, will go into detail more about the disease.

The product is in a pivotal study at the moment, following a successful futility analysis, and as you have seen from the press release, has, we believe, in the US, a blockbuster potential. So a very interesting compound, and not too far from the market, hence why you see it here on the right-hand side, at the top of our new products that we want to develop because it's going to hit the market before the subsequent indications that we are developing for leniolisib in the PIDs and an undisclosed third indication that we're planning to develop leniolisib for. So we're very excited about this possibility, this option to add to our pipeline and to get a product in our pipeline that has the potential to hit the market before the subsequent indications that we are developing for Joenja, and that is significantly more de-risked than Joenja second and third indications to date.

And then I would like to go to the next slide to go a little bit more into detail what the terms and the financial details are of the acquisition.

So obviously, as you have seen from the announcement, it is a public tender offer that we have under the Swedish Takeover Act, as it is a Swedish-listed company. We've offered SEK 0.45 in cash for each of the shares of Abliva AB, which total approximately to US$66 million at today's exchange rate. And we have defined a minimum share acquisition target of 90% of the shares plus one. And of course, the bid is still subject to customary regulatory approvals.

We will fund this with our available cash. As you well know, we have a significant cash balance. We reported more than US$170 million in the bank as per Q3. And of course, what is also very, very important is that the available cash and also the future cash flows that we have, are expected to be able to cover both the acquisition, of course, from existing cash, and the future cash flows will be able to cover the development costs for KL1333. KL1333 is not totally developed by Abliva. It is licensed from a South Korean company, Yungjin Pharm, and that company is entitled to milestones on royalty payments. But those are of a modest size, single-digit to low-double-digit royalties on net sales - tiered, obviously - plus some development milestones.

So, all in all, we believe we have a very interesting opportunity here at hand, which is significantly de-risked and which we hope will come to a conclusion by the time the acceptance period is expired, which is, as you can see on the right-hand side there, around February 7, 2025.

So it is really an important transaction. It is a confirmation of our strategy to develop a high-value pipeline with a number of upcoming product launches over the coming years, and this is an important one and the first one to come, of course, following the build-out of Joenja for APDS.

So, if you take a look now to the next slide, and we have already depicted the pipeline now, you see now that KL1333 is further advanced than leniolisib subsequent indications and therefore will become, once the acquisition is completed, an important value driver for the future growth of our company.

So, with that said, I would like to now hand over to our colleague Anurag Relan, our Chief Medical Officer, to talk more about the compound and the disease. Over to you, Anurag.

Anurag Relan, MD – Chief Medical Officer:
Thank you, Sijmen.

So let's talk a little bit now about why we're excited about this opportunity and specifically why we made this proposed acquisition today. And on the next slide, we can see some details about the disease as well as the opportunity here.

As a reminder, of course, mitochondria are the powerhouses of cells. It's something we all learned in grade school: that mitochondria are critical to producing ATP through a process called cellular respiration, and that ATP drives the energy and produces the energy of the cell. And that energy is needed across numerous cellular types, but in particular muscle cells, and we'll talk a little bit about why that's important for this program.

And we know that these patients who have primary mitochondrial diseases, they're rare diseases, and they affect the mitochondria's ability to generate energy. And although they're a heterogeneous group of diseases, they do have a common thread across them, and specifically that is that these patients suffer from severe fatigue and muscle weakness. And as a result of all of these different issues with their mitochondria's ability to generate energy, they also have reduced life expectancy, unfortunately. And the type of fatigue and muscle weakness that we're talking about really here is - these are debilitating symptoms. These patients cannot lead normal lives because of this significant problem in their mitochondria.

KL1333 is a novel first-in-disease therapy using a mechanism that addresses, really, this underlying disorder. We'll talk about what that mechanism is. And what we're targeting with this compound, is mitochondrial DNA mutations. And here we're talking about patients who are already diagnosed with this condition where you can see there's more than 30,000 patients who already have been diagnosed with mitochondrial DNA mutations in the US, Europe, and as well as in the UK. There is a pivotal study ongoing with KL1333, along with endpoints that have been agreed upon with the FDA. And, as Sijmen said, we anticipate readout of this in 2027 with potential approval by the end of 2028.

So, it's obviously a significant commercial opportunity with significant unmet medical need, and these patients suffer because of that. We have the experience and expertise to bring these types of therapies, as we've done with Ruconest and with Joenja, to patients. And then, as my colleague Steve will talk about later, we can leverage our existing commercial infrastructure to be able to do that.

So let's talk on the next slide a little bit more about these dysfunctional mitochondria that these patients have. And as I mentioned, mitochondria are critical for energy production. And what we're talking about here are a group of genetic disorders that patients have, that are driven by either mutations in their mitochondrial DNA or in their nuclear DNA. And specifically, in this program, we are focused on the mitochondrial DNA. Because of these dysfunctional mitochondria that these patients have, they result in an abnormal NAD+ to NADH ratio. And as a consequence

of that, they're not able to produce ATP in a normal way. And because they're not producing ATP, they have organ dysfunction and their overall health deteriorates.

And again, although there's a wide range of symptoms that these patients have, the most common symptoms that they have across this heterogeneous group of mitochondrial diseases is severe fatigue and muscle weakness. And this is well validated by patients across a number of studies and including across this recent report from the Mitochondrial Disease Foundation. Because these patients have decreased energy production, they're also not able to produce additional mitochondria through this process called mitochondrial biogenesis, which is also very important as part of normal processes.

And on the next slide, we can see a little more about this in terms of how patients are presenting. Again, these patients often will present with weakness, with fatigue, and they'll often see their primary care doctor. And as with many rare diseases, they'll go on an arduous journey to get an eventual diagnosis. Oftentimes, they'll end up with a neurologist or a geneticist, and that will lead to a genetic test that can specifically diagnose this mitochondrial disease mutation that these patients have. This will often happen at an academic center where there are specialists in mitochondrial diseases. And these tests, again, there can be a number of tests that are done, routine lab tests, but specifically the genetic testing that is available to help diagnose mitochondrial DNA mutations.

Because these patients have these problems with their mitochondria, as I mentioned, this severely impacts their daily lives. There's loss of quality of life, there's loss of independence, and a whole variety of mental health issues that result from that. And unfortunately, because of the course of the disease, there's a significant impact on their life expectancy.

And lastly, as I said earlier, there are no approved treatment options. It's unfortunate because these patients are really limited to using vitamin supplements and trying to improve their muscle strength with physical therapy. And you can see from this quote on the bottom of the slide here, the types of muscle fatigue and weakness that these patients experience and how that impacts them just in their routine activities of daily living.

On the next slide, we can see how KL1333 works. And it really corrects the underlying pathophysiology in these patients. So, as I mentioned, these patients have dysfunctional mitochondria, and that results in an abnormal NAD+ to NADH ratio. And because of that abnormal ratio, they're not able to produce ATP in a normal way. KL1333 acts as a substrate for an enzyme called NQO1, and thereby being able to transfer electrons in this electron transport chain to increase NAD+ and eventually increase the production of ATP. And by doing this, it's able to restore the energy regulation and improve electron transport chain function. And as I mentioned earlier, it's also able to stimulate mitochondrial biogenesis, so the synthesis of additional mitochondria. And because of that, we expect to be able to see an improvement in symptom reduction and disease modification because of this ability to correct this underlying problem with NAD+ in these patients' mitochondria.

And on the next slide, we can see some of these attributes outlined.

So we've seen data showing that KL1333 directly increases that NAD+ to NADH ratio, and we've even seen this in cells of patients with mitochondrial diseases. This mechanism is upstream from other competing mechanisms that are being developed in primary mitochondrial diseases. The drug itself is an oral small molecule with twice-daily dosing.

The safety profile thus far has been favorable. There is good IP protection for this, and we also have with the compound regulatory designations, such as orphan drug, as well as fast-track with FDA. And as such, it becomes the potential first-in-disease compound for this disease, with a registrational clinical study ongoing.

And as I mentioned earlier, we've seen this improved energy regulation and electron transport chain function. We've seen these other things here, and I'll talk a little bit about the data that we have seen in terms of fatigue reduction as well as increased exercise capacity.

Now if we go to the next slide, we can see some of that data that's been collected in a Phase Ib program in patients. And what we've seen is that patients, even given a dose of 50 mg once a day, only after ten days had reduced fatigue. And you can see that on the right side in the top-left panel, where patients on placebo did not have this improvement in their fatigue scores, whereas patients treated with KL1333 did.

Likewise, we saw improvements in muscle function. So this was a test called a sit-to-stand test. So literally, it's testing patients' ability to count how many times they can move from a seated position to a standing position in 30 seconds. And you can see, again, patients who were treated with KL1333 had an improvement, again, within a short period of time. And importantly, we can see biochemical improvements too. So, in a dose-related manner, you can see on the bottom panel that these patients have an improved lactate-to-pyruvate ratio. And again, because these patients have impairments with their oxidative phosphorylation and an inability to use oxygen to generate ATP, they have higher levels of lactate. What we see when we treat these patients with KL1333, that those lactate levels come down, and you see that reflected in that lactate-to-pyruvate ratio improving, again reflecting the target engagement of the compound with the enzymes in the mitochondria. And there were no SAEs, or serious adverse events, reported in the study either.

So now let's turn to the pivotal study program that's under way, on the next slide. And this study was designed with input both from patients as well as regulators. And we have seen that with this program, both FDA and EMA have accepted that this single study could be used to support a registration. In fact, FDA has gone further to say that this alternative primary endpoint design, which I'll talk to you a little bit about, FDA has said that even achieving one of the two endpoints would be sufficient for filing and review. And there have been regular discussions, especially with FDA, to facilitate the alignment about the pivotal study program design.

And you see on the right side what that design looks like. It's a randomized double-blind placebo-controlled study. These are adult patients who have mitochondrial DNA mutations as well as documented fatigue and muscle weakness. And the two endpoints are a disease-specific mitochondrial fatigue assessment form called the PROMIS®, as well as the other endpoint that I mentioned earlier, which is an assessment of muscle weakness using this 30-second sit-to-stand test. So patients are randomized, three-to-two in favor of active drug. And there was a futility

analysis conducted after 40 patients completed 24 weeks on therapy, and then there eventually will be a primary efficacy analysis after all patients complete week 48, and then these patients will then have the ability to roll over into an open-label extension study.

And on the next slide, we can see the results of that futility analysis, which we think are actually quite important. And this was a positive futility analysis based on those 40 patients. Let me tell you a little bit about those 40 patients first. These are 40, again, adult patients with primary mitochondrial disease with mtDNA mutations. These were across several countries – you see them listed there - and across 18 sites in these countries. And again, this was a pre-planned interim analysis conducted at 24 weeks earlier this year.

And what this interim analysis showed was that both endpoints passed futility. So, what does that mean? What that means is that there were differences favoring the active arms, so favoring KL1333 for both endpoints, suggesting, when the independent data monitoring committee reviews this data, that they see that if these trends continue consistently, the study would be adequately powered to be able to detect the difference between active and placebo. And specifically, the data monitoring committee made the recommendations that the safety and tolerability profile was acceptable. They did not see any reason to change dosing or any other elements of the study design. And they confirmed that with a population of 180 total patients, we would have adequate power to be able to detect a difference between these two groups.

So this positive interim analysis, actually is a significant step forward in this program. And we think of this as a significant de-risking of the program as we evaluated it as part of our diligence. Of course, the program will continue to 180 patients and there still remain risks, but this was very important for us in terms of our evaluation of the program to be able to judge the efficacy of the product and judge that the program is well-suited to be able to evaluate the efficacy of the product.

And with that, I'm going to turn it over to Steve to talk to you a little bit more about the opportunity.

Stephen Toor – Chief Commercial Officer:

Thank you, Anurag. Good morning, everybody. If you could transition to my first slide, please.
So, as the title suggests, this is a first-in-disease rare disease therapy with blockbuster potential. And over the past decade, we've shown Pharming's expertise in rare disease commercialization, effectively working with patients, physicians, and advocacy groups to meet their often-unmet needs and build strong businesses, which is that some of the reasons I'm very pleased we've acquired KL1333 for PMD, actually a fairly large, rare disease market. It's a great strategic fit and expands our portfolio, adding another product for patients who today have no effective alternatives, as Anurag already stated. And it's important to note that KL1333 provides the opportunity to deliver on the significant unmet needs of a patient group with a relentlessly progressive disease, characterized by severe fatigue, muscle weakness, reduced quality of life, and unfortunately, as Anurag already said, often premature mortality.

With this product, we can deliver the option to become the standard of care for a significant population of patients who are mostly already diagnosed. And there's already significant advocacy for the disease, the patients and the medical community through organizations such as MitoAction, UNDF and the Mitochondrial Medicine Society. There are also centers of excellence across the US where the majority of these patients are already diagnosed and treated. So PMD is already quite a

mature market, waiting for an effective treatment option. And with KL1333, we can deliver that in a simple twice-daily oral presentation. If we move to the next slide.

So, on this slide, you can see the opportunity clearly broken down. And as I said, with a prevalence which is over 230 per million, and with over 50% of those patients already diagnosed and eligible for the FALCON trial, and therefore for the post-approval label, this is a big opportunity.

As already mentioned previously, this is a severe and relentlessly progressive disease. And as the numbers show, it's on the larger side for a rare disease that represents an opportunity for us to deliver a therapeutic solution for a large patient population in desperate need that will also add value for our broader stakeholders and further strengthen our business here at Pharming.
We can transition to the next slide.

Here you can see the centers of excellence that have already been mentioned across the country. There's approximately 20 of them. And with KL1333, we can largely utilize the expertise and infrastructure we already have in place to access them. The remaining patients are predominantly in the larger academic institutions, and we're already present in those as well, often with Ruconest and certainly with Joenja.

As mentioned, KL1333 is a great fit for us, both slotting nicely into our existing portfolio and aligning to our existing expertise systems and processes. So, we're excited to make this acquisition and bring real value to all our stakeholders, but more especially PMD patients, their families, and their HCPs. And with that, I'd like to hand over to Sijmen to provide his concluding remarks.

Sijmen de Vries, MD – Chief Executive Officer:
Thank you, Stephen.
Yes, and if you would like to forward the slide to number 22. So, as you heard from my colleagues, we're very excited about, having made a bid for Abliva to acquire KL1333, because I hope you understood that it fits perfectly in our strategy. We always said we were looking for products with clinical proof of concept. And in fact, what you see here is a product that had a clinical proof of concept in Phase Ib, but is already in a de-risked pivotal trial, because of the futility analysis. So, we're very excited about the prospect of being able to continue to work on the development of KL1333 and bring it to the market.

We're also very excited about the fact that the main shareholders in Abliva and the board and management of Abliva are unanimously backing this transaction. And as you saw from the press release, almost 50% of the shares of Abliva have already been pledged to actually accept the offer going forward. So, we look forward to bringing this further forward. We look forward to updating you on the next occasion when the bid process starts.

So, with that said, I would like to now open the floor for questions.

Operator: Thank you. Our first question comes from the line of Jeff Jones from Oppenheimer. Please go ahead. Your line is open.

Jeff Jones (Oppenheimer): Thank you very much for taking the question, guys, and congrats on the deal. It sounds very exciting.

Sijmen de Vries: Thank you.

Jeff Jones: Can you comment on how much you anticipate it will cost to complete the FALCON trial and any cash that's coming in on the balance sheet for Abliva? And then, on the technical side, can you comment on how these patients are managed today?

Sijmen de Vries: Yeah, so the cash, so we estimate, I think we have said, between 100 and 125 for the development costs going forward from our own future cash flows. And do you have any details on the cash balance on Abliva, Jeroen? It's pretty small, right?

Jeroen Wakkerman: Very small. It's about SEK 45 million.

Sijmen de Vries: Yeah, so about €4 million, right?

Jeroen Wakkerman: It's almost nothing.

Sijmen de Vries: Almost nothing, yeah. And then the technical question, Anurag - the current state of affairs with regards to treatments?

Anurag Relan: Yeah, Jeff, so essentially these patients have a number of other symptoms. So, in terms of the fatigue, there's not much for them. Often times, they will get these vitamin supplements, but the rest of the symptoms are managed. So, if they have seizures, those are managed. If they have issues with cardiomyopathy, that is managed in the traditional way. But in terms of trying to address the muscle weakness or the fatigue, it's actually quite limited in terms of what's available to them today.

Jeff Jones: Great. Thank you very much, guys. I'll jump back into queue.

Operator: Thank you. We'll now move on to our next question. Our next question comes from the line of Joe Pantginis from H.C. Wainwright. Please go ahead. Your line is open.

Joe Pantginis (H.C. Wainwright): Everybody, good morning. Thanks for taking the question. And congrats on the transaction, as you've delivered on one of your goals here. And happy holidays to everybody.

Sijmen de Vries: Thank you.

Joe Pantginis: You bet. So, first, I guess from the clinical side going forward, would you be looking to working towards any improvements in the diagnosis and patient identification?

Sijmen de Vries: Would you comment on that, Anurag?

Anurag Relan: I'm sorry. I didn't hear the question.

Joe Pantginis: Sure. Would you be working towards any improvements in genetic testing, diagnoses and patient identification going forward?

Anurag Relan: Sijmen, could you repeat the question? I only caught –

Sijmen de Vries: Joe asked if we're working on improving the diagnosis for the disease now. But I think, in this case, it's not the biggest issue, right, to diagnose, because all – a number of these – all those numbers of patients that we're quoting have been diagnosed and are confirmed for diagnosis. Right, Anurag?

Anurag Relan: Yeah, absolutely. So I think there's two things here, Joe. The first is there is a large, diagnosed pool already out there. And this is something that we've confirmed through a number of different ways, including actual claims data in the United States. We were actually able to confirm the number of patients that are out there with a diagnosis of mitochondrial DNA - primary mitochondrial disease. That's number one.

Number two, to your question, there are also probably a significant number of undiagnosed patients. And again, as we continue our efforts in terms of the drug development, as well as disease education, we also think that these numbers will grow as a result of that.

Joe Pantginis: No, that's helpful. Thank you. And then one question about the FALCON study, especially when you consider endpoints like sit-to-stand test. How do you consider the risk of, say, the training effect of the placebo arm? Because if you continue to repeat something over time, you tend to get better anyway. Or does the underlying impact from mitochondria or lack of mitochondria energy supplant that?

Anurag Relan: It's really the latter, Joe. So these patients have a progressive disease that, again, their muscle strength worsens over time. And in the absence of anything such as KL1333, they don't typically have spontaneous improvement. Now, there can be some improvement. Of course, that's the whole purpose of the placebo-controlled design, is to have those patients distributed across the two groups. But we think that with this type of design, as well as the assessment of the second endpoint, fatigue, that the study is well positioned. And now we know also with the interim analysis, we know that it's adequately powered to be able to detect the types of differences that were originally anticipated when the study was designed.

Joe Pantginis: Very helpful. And if you don't mind, I'm going to really ask a forward-looking statement here, because, you know, investors are fickle, and they're always about what's next. So, Sijmen, do you anticipate, or are you still in shopping mode right now?

Sijmen de Vries: Yes, Joe, this is not the last deal, definitely not. We have a clearly outspoken strategy to build that global rare disease company. And today, of course, is an important step forward, as I was already alluding to earlier, but it's definitely not the last one. It's the beginning of, I believe, more deals to come in the future, because that's definitely what we're after. We're building this global rare disease company.

Joe Pantginis: Great. Thanks, guys.

Sijmen de Vries: Thank you, Joe.

Operator: Thank you. We'll now move on to our next question. Our next question comes from the line of Alistair Campbell from RBC. Please go ahead. Your line is open.

Alistair Campbell (RBC Capital Markets): Thanks very much, and thanks for providing me time today. It looks like a pretty interesting molecule. Just a couple of questions on the FALCON trial.

Just when I was looking on clinicaltrials.gov, it was sort of pointing towards what looked like primary completion at the end of 2025, but obviously you're talking about 2027. So, I just wondered if there's a discrepancy there, or is there something which has sort of pushed the endpoint out a bit from the original plan?

And then secondly, can you just confirm the dosing you're using? It's BID, but sort of what dose level that is, and what sort of headroom you think you've got on top of that before we start to see some of the GI tolerability issues that came up in Phase I? Thank you.

Sijmen de Vries: Anurag?

Anurag Relan: Yeah. So, Alistair, I think in terms of the clinicaltrials.gov listing, I don't think it's fully updated. This information that we're providing today reflects our understanding of where the study is right now, what time it would take to recruit, and what time it will take to analyze all of that data. So, it reflects all of that, and we also anticipate opening additional centers, including in the US, so it definitely takes all of that into account. It's possible that the listing only shows the final date for when the last patient is enrolled, so maybe that is part of the discrepancy, but I can't really speak more to that. In terms of your other question, that was relating to, sorry, what?

Alistair Campbell: Yeah, it's just dosing you're using in FALCON, in terms of the headroom there, in terms of some of the GI tolerability issues that were seen at the high-level dosing in the Phase I.

Anurag Relan: Sure. So there is a dose titration schedule in the FALCON study where patients start at a certain dose level and then increase, and what we've seen so far is that all patients are able to increase. What we've also seen is that some patients do need to drop down, but they are able to increase again. But in general, we've seen good tolerability of this dose level, and again, the effects that we've seen in the Phase Ib study were at the 50 mg once-daily dose, so we think that with the 50 mg twice-daily dose that is being seen now, I think we have a good opportunity to see a positive effect.

Alistair Campbell: Great. Super helpful. Thanks so much.

Operator: Thank you. We'll now move on to our next question. Our next question comes from the line of Ben Jackson from Jefferies. Please go ahead. Your line is open.

Benjamin Jackson (Jefferies): Brilliant. Thank you for the question. So just three short ones, if I may. The first of which, have you done any due diligence with payers on the endpoints that are being used in this trial, and is there perhaps any benefit for both of those reading out positively, rather than one from a payer perspective?

The second is more on is there any scope, perhaps in the open-label extension, to assess more on an outcomes basis? We obviously are referencing here, life expectancy being shorter, and perhaps that could provide an even more compelling argument to the payers themselves.

And then the third one that I'll just briefly ask on, could you just touch on the margins for the drug comparative to the other two that you have in the commercial portfolio, specifically referencing to what extent you will have to consider increasing your salesforce, and to what extent they overlap with the current salesforce that you have in place? Thank you.

Sijmen de Vries: So, shall we start with the open-label outcomes? Anurag, would you like to comment on that?

Anurag Relan: Sure. So certainly, there will be an opportunity, especially in the open-label study, to take a look at additional outcomes. There's a number of secondary endpoints already being evaluated, but we can certainly look at additional outcomes to be able to track these patients, especially some patients who have already entered the study, who by 2027 when the study reads out, would have been on the drug for several years.

So, there will be a mechanism to collect that data. We look forward to collecting that data and hopefully being able to see the types of things that you describe, because I think that will certainly strengthen the support for reimbursement. Now, that said, and Steve can speak to this more, we have already done significant US payer research. Steve, I don't know if you want to comment on that.

Stephen Toor: Sure. Thank you, Anurag. Yeah, I mean, there has been some payer research done, obviously, as part of our due diligence, and Abliva also did. As you can imagine, we're still quite some distance out, though. So, over the next two, three years, we'll be doing quite a bit more to understand the differences, obviously, between the US, which isn't cost-effective-driven, and outside the US, where it is.

You also asked about the salesforce and overlap. I think there is a reasonable amount, certainly with the institutional team we have, where slightly under half of patients will be situated. And obviously, there's those 20 centers of excellence. So, it's not a perfect overlap. We'll certainly need to add a few salespeople, but it's not going to be a significant burden to us as an organization.

Sijmen de Vries: Thanks, Steve. And with regards to the margin question, you've seen the single-digit royalties, which are considerably less, of course, than Joenja, and going up to low double-digit, which is also considerably lower than Joenja. So, in other words, given that it's a small molecule and the cost of goods will be pretty low, we don't know the price, of course, yet at this point in time, but we are definitely seeing margins that are probably better, in the end, on gross margins, better than Joenja, and probably maybe initially on par with Ruconest, but that's just a thought that

I have here. So, in other words, it's a very good addition also from that perspective to the portfolio. I hope that answers your question as far as we can do today, Ben.

Benjamin Jackson: Yeah, that's brilliant. Thank you so much.

Sijmen de Vries: All right.

Operator: Thank you. We'll now move on to our next question. Our next question comes from the line of Sushila Hernandez from Van Lanschot Kempen. Please go ahead. Your line is open.

Sushila Hernandez (Van Lanschot Kempen): Yes, thank you for taking my question. Could you remind us about the IP estate for KL1333? And also, does your strategy for Joenja remain unchanged, so building out the franchise and PIDs? Thank you.

Sijmen de Vries: Yes, Sushila, thanks for the question. Yes, the strategy remains the same for leniolisib/Joenja, to build it out for APDS and for the subsequent indications, of course. And the patent estate for KL1333 is, I believe, 2041. Anurag, could you correct me on that?

Anurag Relan: I believe it's 2038 - including extensions, but we can come back to that, yeah.

Sijmen de Vries: Yeah, yeah. At least in that range, Sushila.

Sushila Hernandez: Very good. Thank you.

Operator: Thank you. We'll now move on to our next question. Our next question comes from the line of Simon Scholes from First Berlin. Please go ahead. Your line is open.

Simon Scholes (First Berlin): Yes, good afternoon. Thanks for taking my questions. I've just been looking at an Abliva presentation from July of this year, in which they're talking about their patient assumptions, and I noticed that the assumption for treatment with KL1333 is that the number of treated patients should be roughly half the number of diagnosed patients. I'm just wondering why there's such a difference. And I believe it also shows a slide showing competitors. You've got Stealth, you've got Tisento, and you've got Khondrion, and two of those competitors are concentrating on MELAS, which, I think, is a subset of the market you're addressing with KL1333. And I was just wondering if you expect KL1333 to be equally efficacious in MELAS as these competing products.

Sijmen de Vries: Right. Anurag, could you elaborate a little bit on this?

Anurag Relan: Yeah. So the MELAS patients are also included in the pivotal study with KL1333.

Simon Scholes: Okay.

Sijmen de Vries: And with regards to those mode of actions of those compounds?

Anurag Relan: Yes. I think one of the things that we've seen is that KL1333 directly increases that NAD+/NADH balance and reducing lactate. And what I talked about also in terms of increasing the synthesis or what's called mitochondrial biogenesis, versus some of these other drugs that are in development, let's say, are more indirect or downstream from that in terms of how they may be working.

Simon Scholes: Okay. And how about this difference between the diagnosed patients and treated patients? I mean, it seems that Abliva were not expecting about half of diagnosed patients to be treated with, or at all treated.

Anurag Relan: So what we've determined is that there are maybe around 30,000 patients in the United States, for example, that are clinically affected by mtDNA mutations. And then on top of that, we think that about half of those would meet the inclusion criteria requirements for the FALCON study. So that's how we ended up with 16,000 in the US, for example.

Simon Scholes: Very good. Thanks.

Sijmen de Vries: In other words, it's pretty consistent, right, with what Abliva stated. Thanks, Simon, for that question.

Operator: Thank you. There are no further questions at this time, so I'll hand the conference back to Sijmen for closing remarks.

Sijmen de Vries: Thank you very much, ladies and gentlemen. I hope you have been able to share our enthusiasm and our dedication to actually bring this compound further forward. Of course, following the successful completion of the bid, which, of course, will take some time, but we're very focused, and we're very motivated to bring this compound, which as I stated before, is a significantly de-risked product with blockbuster portfolio and could be in the market before the other internal programs that we currently have. So therefore, we think it's a very, very important accelerator of the growth of the company and a significant step forward to building our leading global rare disease company.

Thank you very much for your attendance. And we'll be back, of course, with more news on progress of the acquisition of Abliva. Thank you very much. Goodbye.

[END OF TRANSCRIPT]