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ONCO.ST - Oncopeptides AB to Discuss Positive Topline Results From the Phase 3 OCEAN Call

EVENT DATE/TIME: MAY 25, 2021 / 9:00AM GMT

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PRESENTATION

Operator

Hello, and welcome to the Oncopeptides (technical difficulty) conference 2021. (Operator Instructions)

Today, I'm pleased to present CEO, Marty Duvall. Please go ahead with your meeting.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thank you very much and good morning to everyone, and thank you for joining us on this great occasion to provide the top line results for our Phase III OCEAN trial. We're very excited to communicate our thoughts and the top line results and implications for this fantastic outcome of a head-to-head comparison.

So on Slide 2, I will be joined today by Jakob Lindberg, our Chief Scientific Officer; by Klaas Bakker, our Chief Medical Officer; and by Anders Martin-Löf, our Chief Financial Officer. And as far as the next slide and on disclaimers, of course, we will be making speculative and forward-looking statements regarding our drug and our company. So we ask you to please look at our filings for the full risks and balances. So Slide 4. We're just so pleased to report these very positive results.

The OCEAN trial is a positive study. And certainly, with the outcomes that we're seeing, as a CEO, I'll sign up for this outcome every single day. I think the key for all of us, as investors and stakeholders in the company, though, is how will it be perceived by clinicians and regulators. And on that point, we feel very positive. We believe that this is a major milestone for relapsed and refractory multiple myeloma patients, and we look forward to the future communication of the final results and working with regulators to improve the label and expand our -- the availability of PEPAXTO worldwide.

So in terms of our key takeaways on Slide 4, the OCEAN trial, no one dare contend with the fact that this was a very boldly designed study with a successful outcome. And this is the first head-to-head positive trial in multiple myeloma in 6 years. And we didn't just choose any agent in terms of the head-to-head comparison. We chose the drug that is the most widely used drug in relapsed/refractory multiple myeloma, that being pomalidomide. And on our top line results with the primary endpoint of progression-free survival, on the point estimate of median PFS, PEPAXTO was 40% higher than pomalidomide.

And as a reminder, pomalidomide has sales worldwide of $3 billion and is growing at 20% on an annual basis. When we set out with the HORIZON trial and passed the FDA hurdle and got accelerated approval for our drug in fifth line plus population, we suggested that we were starting a journey. And we continue with the results of the OCEAN trial to be on a path to make this agent a foundational treatment in relapsed/refractory multiple myeloma.

As you'll hear through this presentation, we are working as hard as we can and as quickly as we can to present this full data set at conferences throughout the remainder of 2021, with the first showing as soon as possible in terms of being able to submit this and getting at the stage that it deserves. And we are already well underway as it relates to regulatory bodies and in particular the FDA in discussing our plans for supplemental New Drug Application for PEPAXTO for earlier lines of therapy and to get this outstanding comparative data into our label.

So in closing this opening here, thanks to patients that participated in this trial, thanks to the investigators for this outstanding result, thanks to you, the investment community, for making it possible and also, of course, the Oncopeptides team that executed this trial flawlessly through a global pandemic with certainly an outstanding result. So on the next slide, I'm going to turn it over first to -- in looking at the agenda to Jakob Lindberg. Then Klaas Bakker will take us through the top line data. I'll come back with the commercial opportunity, and then we'll move to Q&A.

So with that, I'll turn it over to Jakob Lindberg. Thank you, Jakob.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

Thank you, Martin. Jakob Lindberg here. So please slip -- go to Slide #6. Before we look and talk about the results in the next section, it's worth doing a few reflections around what type of trial this is. The vast majority of trials in oncology are so-called add-on trials, and we'll come to that. And as Marty just mentioned, the OCEAN trial was head-to-head. It's a head-to-head comparison, which is actually in myeloma quite rare. And on top of that, when you do a head-to-head, it is rare to also do it with a full-blown relative and relevant comparator, in this case, with pomalidomide, that is as close to standard of care in relapsed/refractory multiple myeloma as you can come.

However, there is a challenge in doing these bold trials. And that is because the dominance of the add-on trials is such that most developers and clinicians instinctively interpret results and to some extent also the market, regardless of the design through the lens of add-on trial outcomes. And as an example, when you have a hazard ratio 1 in an add-on trial, it means your drug is doing 0. A hazard ratio of 1 in a head-to-head trial means that you are equipotent with the drug you're comparing with. The statistics are the same, but the interpretation is vastly different of the same number. And this poses a true challenge when communicating head-to-head clinical trial results despite being the preferred design from a clinical relevance point of view.

And of course, we all know that placebo-controlled trials are rarely, if ever, executed in oncology due to the severity of the disease, and, hence, why that is something that is rarely contemplated. If we go to the next slide, Slide #7. Another way to look at add-on trials in head-to-head is to look at what is it you actually measure. If you look to the left on this slide, you see that in a head-to-head trial, you measure the delta between the 2 drugs. And of course, once again, if the comparator is highly relevant, such as in the case with pomalidomide, even if you have an amazingly good drug, the delta will be numerically smaller in absolutes than the additive effect you have in an add-on trial where you add a drug.

In the slide to the -- in the box at the right, you see that drug B is relatively less efficacious than drug B in the head-to-head design, but the additive effect will look numerically larger despite being the inferior drug. And I think this is a very, very important point when interpreting results. Very few drugs in add-on designs would ever reach, if anything else, an inferiority in a head-to-head design, very important point to realize.

If we go to the next slide, Slide #8. Why did we go for a head-to-head design? Well, successful drugs in multiple myeloma all have labels that allow for single-agent plus/minus steroid use. And at this time, when we initiated this trial, we were not aware that we would get such great results in HORIZON, that that would support an accelerated approval. And while some argue that this is a triplet market, almost half of all the patients are still only receiving single agents plus steroids, and there's a significant off-label combination use. Meaning that labels for single-agent plus/minus steroid is still the staple in the foundation of the multiple myeloma market.

And the reason for single-agent plus steroid use is such as the 2 drugs that are compared in OCEAN is because of tolerability, resistance development, comorbidities and refractory status. The number of treatment options for these later line patients shrink to minimum very, very rapidly, and there is a huge need for new treatment options. So we actually had a choice back in 2016 to either go head-to-head with lenalidomide, with bortezomib or pomalidomide. And the choice was simple because pomalidomide was the de facto standard of care already then in the later lines, which is where you start when you try to build the clinical database for a new drug.

If we go to Slide #9, you can see the various outcomes that the head-to-head comparison can give. Either you're superior, which means that your point estimate with the standard error bars are all below 1; or noninferiority -- it means that you're somewhere around the middle mark with some standard errors predefined that you have predefined together with regulatory authorities. And of course, if you're far to the right, you would finally be inferior.

What does these 2 results mean? And I think this is very important when we go to Slide #10. So any drug you test has a true treatment effect. We don't know it, but there is one. That is the true and absolute treatment effect of any given medicine in an indication. And when you do a trial and you compare it to something, the true treatment effect can either be worse, same or better than your comparator. The problem is that you don't know the true treatment effect. You approach your clinical trial database with statistics, and you try to either verify or exclude, under certain conditions, certain outcomes.

What noninferiority means is you have excluded, under certain conditions, that your drug isn't worse. So it's either same or better than your comparator, but you can't say whether it's same or better. The only thing you can say from a statistical point of view is that you have excluded that it's worse. And of course, if you go to the next slide, Slide 11, the only difference between noninferiority and superiority is that now, with the superiority outcome, you have also excluded that you're the same, of course, under statistical boundaries that you have predetermined together with the regulatory authorities. But ultimately, they are more similar than one might think in terms of real clinical outcome.

So with that being said, before we go into the results section, we go to Slide #12 and just look at the market shares in relapsed/refractory multiple myeloma in the U.S. in 2020. Pomalidomide is still the largest drug. And please note that the total market share here can be larger than 100% because there's a lot of combination use as well. And as you can see, given that we have gone head-to-head with this drug, it also means that we have gotten data with melflufen, where we can compare it with the key reference in RRMM for both safety and efficacy. The database is a treasure trove to understand melflufen.

With that, I would like to bring the word to our Chief Medical Officer, Klaas Bakker, to go through the results. Klaas?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Thank you, Jakob, and good morning, everyone. Before I start, I just want to share on a personal note, that today, you hear a very, very satisfied CMO who is really pleased with the results. And I'm excited to be able to share these results -- these top line results with you today.

Let's start now at Slide #14. So this is the design of the OCEAN study. And Jakob touched upon it already a couple of times. But this is a trial where we have included multiple myeloma patients who have failed 2 to 4 prior lines of therapy, including being refractory to lenalidomide within 18 months or have progressed on lenalidomide within 60 days of randomization. We enrolled 495 patients in a randomized way 1:1 with the primary endpoint being PFS and key secondary endpoints being overall response rate and overall survival. This was a global study with over 100 participating sites in 21 countries, so a true global study with a huge impact.

If we now go to Slide #15, and we look at the detailed time line of OCEAN. To the left, we have the earlier years. To the right, we are in the future. So it is already almost 4 years ago that the first patient was treated in OCEAN in June 14, 2017. Then in May 22, 2020, we were complete in terms of enrollment of the 450 initially planned patients. However, to our positive surprise, we saw that patients stayed on treatment longer than anticipated, basically saying that they were benefiting from the drug more than we had anticipated on average.

This made us decide to over-recruit a little bit to 495 patients, which made the study fully recruited at the 4th of September 2020. Then in March 2021, we reached the number of events for our primary endpoint. And here we are today at May 25 to disclose our top line results. And of course,

and we'll talk more about that in a minute, we will also talk about our planning with regards to a publication and the presentation of the data at a future scientific conference. And we'll also talk about our regulatory interactions.

Let's go to the results on Slide #16. This is the result for the primary endpoint being progression-free survival. And before I start, I just want to go back to what Jakob said where you have an equal drug when you are at a hazard ratio of 1 in a head-to-head study. If you look at the hazard ratio that we present here today, both by the Independent Review Committee as well as by the investigator test results, we have a hazard ratio of approximately 0.8, which means that across the intent-to-treat population, the full population, melflufen performed better than pomalidomide in the OCEAN study. The p-values differ but this means that the result is so close between Independent Review Committee and the investigator assessed result that it is really important to note that, ultimately, the regulatory authority will do its own assessment and look at the individual patient data.

And when you actually realize that we are so close to being superior on IRC, the totality of the data becomes important. And when I speak about the totality of the data, I cannot share everything today. But we, as a company, believe, looking at the results that we have seen so far, that our total package is very strong. It's not only the hazard ratio of the primary endpoint, but also other endpoints where we are very confident that we have a good package to submit to regulatory authorities. And please note that when we talk about the improvement of median PFS, we talk about a 40% addition in a head-to-head trial.

Almost everyone would be really pleased to see this in an add-on trial. It would be great. And here, we see it in a head-to-head trial where we almost are above 40% of relative median PFS improvement. That's huge. Either being noninferiority or superiority, it's very close, but it is a huge advantage for melflufen over pomalidomide. We also choose to share the overall response rate today. And why is that important? It is important because that tells you if the patient responds to the treatment and what can you tell as a physician to the patient who comes for his or her first response assessment.

And this is where we -- with a doublet -- are solidly above the 30% mark, which is a really good result. And this actually means if you -- especially when you compare with pomalidomide, that in terms of the doublet, the response rate is substantially higher for melflufen when compared to pomalidomide. Together with other efficacy results, which we hope to share at a later scientific meeting, we feel that the outcome of this trial is very, very positive.

Go to the next slide, please. The safety summary. Most physicians, and we know this from market research, would have anticipated that melflufen would do worse in terms of safety. Pomalidomide is perceived as a very well-tolerated drug. It's used very often, as you could see from the market share numbers. But if we look at the safety, and we compare it between the 2 drugs, that we had [won] no new signals for melflufen. So the safety profile with the number -- with a relatively high number of hematological adverse events, we call them lab value abnormalities or paper tox, was in line with what we know from previous studies. No new safety signals were identified, and the hematological adverse events could be managed clinically very well.

What we also could see is that despite the fact that we had more neutropenia as part of the hematological toxicity, pomalidomide actually had more infections than melflufen, and this was a surprise to us. More hematological toxicity on paper, but, ultimately, less grade 3 or 4 infections than pomalidomide. In terms of nonhematological toxicities other than infections, we had similar results when compared to pomalidomide, which is very encouraging.

And then the last point, which I'd like to focus on, is the discontinuation rate. Because this tells you something about the tolerability of the drug. Pomalidomide is an oral drug, and it is perceived to be very well tolerated. Melflufen is an intravenous drug. Despite that, we have similar discontinuation rate for adverse events for both drugs being very, very reasonable. So we were very pleased to see this, but we were on par from a safety perspective with pomalidomide.

If we go to the next slide, Slide #18. This is about next steps. And what does this actually -- what does this result mean for engaging FDA? Well, of course, we are currently in the process of engaging the FDA on the OCEAN data. We will present data at a key scientific conference as soon as possible, and the publication work has already started; because we believe we have such a good result, we want to share this with the outside world as soon as possible. And that speaks to the confidence we have in our data that we want to progress this as soon as possible.

Based on the data, we plan to file for a supplementary NDA in the fourth quarter of this year. And in light of the OCEAN trial results, which we believe are fairly strong, we plan to ask for 2 things: one, a label change; two, also a full approval for PEPAXTO as a requirement for the accelerated approval. And we think we are in a good position to fulfill both based on the totality of the data again. And in the meantime, before I hand over to Marty, we, of course, continue and focus on our commercialization efforts with PEPAXTO in the United States until we have updated our label and hopefully reached a full approval.

Now I'd like to hand over to our CEO, Marty Duvall, who will take you through some further commercialization thoughts. Marty, over to you.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thank you very much, Klaas. So I'll cover commercial opportunity and future plans. So if we flip over to Slide 20. So the first critical thing when we work towards bringing a drug to market is to establish that we have single-agent efficacy. That's absolutely critical. And we did that with the HORIZON trial. We did it in an area of high unmet need, resulting in an accelerated approval in the U.S. Now with our clinical development program, OCEAN and LIGHTHOUSE, significantly expand the opportunity.

So in thinking about the OCEAN trial, we are proving that single agent efficacy, combined with dex, through head-to-head against one of the most widely used drugs in the space. And what are the results of that? We see more patients responding, as Klaas outlined, and having a longer progression-free survival than the most widely used drug. So as it relates to OCEAN, it's the head-to-head with pomalidomide, $3.1 billion, 20% growth. We know that approximately $600 million of utilization in the U.S. is attributed to the PomDex combination alone and that the OCEAN trial and the results here provide the opportunity for us to move up in line of therapy to third-line-and greater setting. So we certainly look forward to those interactions with regulatory bodies and sharing this data with physicians.

The next step here that further links our trials and clinical development program with the fastest and largest growing drugs in the space. We have the LIGHTHOUSE trial, a very important combination with DARZALEX. Again, another $600 million in PomDex sales in the U.S. are tied to the triplet regimen, which adds dara. So again, a fastest-growing drug -- one of the fastest-growing drugs in the space. And a significant opportunity for us is we know, besides showing that single agent efficacy, that combination use is very important in the space as we look to set ourselves as a foundational treatment.

Moving to the next slide, I'd like to tie in also, on 21, our PEPAXTO commercialization strategy and why the OCEAN trial is so important to us becoming that foundational treatment. As we think about the utilization of products today, there's a domination of the IMiDs, the PIs, anti-CD38s and a recycling of old classes of drugs and a small use but now growing use of drugs with new mechanisms of action. When we launched into this category and became -- or have become 1 of the agents here with a new mechanism of action, we are competing head-to-head with belantamab, selinexor, ide-cel and others as a key agent here with the new mechanism of action.

But the second part of the strategy is to move away from this recycling of failed drug classes. That's exactly what the OCEAN trial shows, right? We have a lenalidomide refractory population where the randomization was to recycling the next IMiD, pomalidomide, or use melflufen, dex. And in this case, we showed a result that was significant versus that drug. So very exciting results that tie in and will help fuel our strategy and fuel the commercial uptake of PEPAXTO in the United States and we believe around the world once we receive regulatory approval.

Looking at Slide 22, just to show some of the momentum from a pomalidomide perspective over the years. We see the growth on the left-hand side of the slide of worldwide sales for pomalidomide reaching $3.1 billion, as noted earlier, $2.1 billion in the U.S. alone with that 20% growth. And on the right-hand side of the slide, we see that currently, PomDex and PomDex combos comprised 33% of the U.S. share of patient market share according to Intrinsiq data. So a very significant commercial opportunity that is at our fingertips with our current clinical development program.

Turning to Slide 23. So we see now the worldwide sales for DARZALEX on the far right-hand side and it growing at 40%, so $4.2 billion drug. So we start with OCEAN. This really sets a new bar with PEPAXTO, positive head-to-head data, strong efficacy profile for our drug as a doublet in third and fourth-line. LIGHTHOUSE will provide that first opportunity to expand our label with PEPAXTO being part of a triplet regimen in relapsed and

refractory multiple myeloma. So really look forward to now our attention turned to accelerating approval to the LIGHTHOUSE trial so that these results can also be made available.

So flipping to the last slide and in summary. In June of 2017, we set out on a very bold mission to show that we could be one of the best drugs in relapsed/refractory multiple myeloma, and we have a successful outcome with the OCEAN trial. Superior according to the investigators, noninferior but right on the edge according to the Independent Review Committee. The 40% higher median PFS is very important -- will be important to clinicians who make decisions on which drug to choose to treat their patients. We believe that we're on this path to be a foundational treatment in relapsed or refractory multiple myeloma.

We really look forward to the deeper dive in being able to share all the results, the totality of the evidence for PEPAXTO in the OCEAN trial, at a conference as soon as possible and look forward to also interacting with regulatory authorities, in particular, the FDA with a supplementary NDA that we have planned for Q4 of 2021. So before turning it over to the Q&A, I'd like to once again thank the patients, the investigators, the investors and the team that made this trial and the execution possible.

So with that, I'll turn it back over to the moderator for the beginning of the Q&A session.

QUESTIONS AND ANSWERS

Operator

(Operator Instructions) Our first question comes from the line of Erik Hultgård from Carnegie.

Erik Hultgård - *Carnegie Investment Bank AB, Research Division - Research Analyst*

Yes. And first, I want to extend my congratulations to a very solid OCEAN Phase III data to the whole team. I have a few questions. First, given that the hazard ratio was very close to fall into the superiority bracket. As progression-free survival data continues to mature until submission in the fourth quarter, do you see a chance that we would actually turn out to get in at the hazard ratio below 0.1 -- 0.8, sorry? That's my first question.

Then secondly, could you comment on whether you had another look at the overall survival data and whether that is trending in favor of PEPAXTO? And finally, also, if you could comment on the shape of the progression-free survival curve, because you were seeing early separation of curves, and whether the difference is also in favor of PEPAXTO [in the tail] of the curve.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thank you, Erik, and we really appreciate your congratulations on the trial. So I'll turn it over to Klaas to address the point on the hazard ratio being close, early look at overall survival and the shape of the curves.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Good. Thank you, and more than happy to answer the questions. So first, about an updated PFS figure in the future. We believe -- but this is, of course, not something we know, but we believe based on what we see that there is a good chance that the PFS, as it matures, will go to a hazard ratio below 0.8. So we are confident that in a potential later data cut this will become visible. Whether this will be used in regulatory interactions is, of course, a review issue, but we feel confident that it may turn that way.

Second question, overall survival. The overall survival is not mature yet. So we cannot draw any conclusion also because the trial was not powered for overall survival. And as you know, there are a lot of subsequent treatments available. So there is always a watering down effect in overall survival.

At this stage, I cannot say whether it's going in one or the other direction. But we strongly feel that the overall survival supports the data that we see in the progression-free survival.

Then to the shape of the PFS curve, and I'm happy that you asked that question because if you think about the PFS, I can disclose that the curve does not look strange. The curve looks like a very light PFS curve where you see a split that continues, no crossing of the lines and nice white space in between the curves. I think that's all I can share at this stage. But needless to say that I'm very, very confident at our data looking at the shape of the PFS growth. I hope that answers your question.

Erik Hultgård - *Carnegie Investment Bank AB, Research Division - Research Analyst*

Yes. It did. Again, congratulations to the data.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thanks, Erik.

Operator

And the next question comes from the line of Peter Welford from Jefferies.

Peter James Welford - *Jefferies LLC, Research Division - Senior Equity Analyst & European Pharmaceuticals Analyst*

So just continuing in a similar vein. Firstly, just with regards to the patients in the study. I wonder if you could just comment how many patients are still on treatment at this point in time in the study? Secondly then, just in regards to the enrollment. Clearly, the study is roughly 10% overenrolled. I guess could you make any comment at all, given the study actually is designed for a hazard ratio of 0.80 but clearly the study was overenrolled, so I guess, clearly, the number of events is still at originally anticipated.

But can you comment at all how many events were in this analysis that you're presenting? Because I guess, I would have thought, given the over-enrollment, the p-value could potentially have been smaller. But if you can comment on the number of events included in the analysis, that would be helpful. And then third, just on subgroups. I wonder if -- I appreciate it's early days, but any comment at the moment at all on any interesting subgroups at all or any signs that you've seen within the subgroup analysis?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thank you, Peter. And again, I'll turn it over to Klaas for comments on your questions.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. And I'd like to start with a question about enrollment. We did over-recruit, but we were not changing the number of events that were needed for the study. So this data cut that we saw was aimed at the 339 events. And I can tell you that the number of events was somewhat above the 339 events, but still very close, so exactly as we would have planned for. So this over-recruitment made us actually reach the number of events somewhat earlier, but the whole analysis is still the same, and there should be no difference in the p-value as such.

Number of patients still on treatment, we have about 70 patients still on treatment distributed across both arms. On the subgroups, that's a very interesting question. And if -- you can imagine that if you have a result that is so close to superiority, that you will have subgroups where we clearly

pass the hurdle of superiority and are superior. And although it's early days, as you mentioned, I think when I mentioned that we are very confident in this data, when we look at the totality of the data, that is also driven by the strong results of PEPAXTO in some of the key subgroups.

So looking at subgroups, yes, we think that will further support our case when it comes to regulatory interactions, but also when it comes to the footprint of this drug in the clinical treatment landscape of multiple myeloma in the relapsed/refractory setting. So we are basically -- what I'm saying is that we are very encouraged by what we're seeing in some of the most important subgroups as well. I hope that answers your question.

Operator

And the next question comes from the line of Patrik Ling from DNB Markets.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

Yes. Thank you, guys. Could I ask -- I mean when you started this trial, you had, as one of the preconditions, that the patient should have failed lenalidomide pretty soon before entering the trial. And then you relaxed those inclusion criterias. Did you see actually any difference in the patients' outcome, whether they were very, very soon that they failed len, very, very rapidly before entering the trial or if it took some longer time since they had failed on len?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Patrik, thanks for the question. I'll turn this one over to either Jakob or Klaas regarding this change.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. I'm happy to start there. I think, Patrik, very relevant question. To start with, I can say that the vast majority, and then I'm talking about 90% of patients, had lenalidomide as their last line of treatment. So it's only a very small group that we're talking about. We have not done the analysis yet to see if there are any differences in between those patient groups. But looking at the totality of the data in the inclusion, we feel that the protocol change did not have a material impact on the study data. So the relaxation of the inclusion criteria did not lead to a relative over-recruitment of patients that failed len longer ago. So we couldn't see that trend back in the data.

Jakob, is there anything you want to add there?

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

No. I think you said it all, Klaas, and we're going to do further analysis and look at both the duration of len treatment as well as the time point. But ultimately, it's a very homogenous population in terms of time point. So there might be something on duration.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

Okay. So just so I understand it correctly. Around 90% of patients failed lenalidomide within 60 days of entering the trial because 100% of patients are len failures?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. You could take that as a reference point, yes.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

Okay. Great. Then my second question. I mean when you talk about scientific meetings, do you have any feeling for when you could present something?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

We -- our goal is to do it as soon as possible. And we need to do some more analysis to make sure that we present a full and clear picture of the results. But clearly, I think we may have [flex like] the second half or Q3 of this year. I think that's still the base case, but we are looking into opportunities to do it even faster, given that we think we have a truly good result to share with the scientific community.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

Okay. Great. Then last question. When you evaluated the patients now in the trial, did you have to do any corrections or amendments for some sort of impact from COVID-19?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. That's a good question. So COVID-19 has played a role in every clinical trial in the COVID era. We did necessary sensitivity analysis. And I can say that it did not -- it seems but -- it seems that COVID-19 did not materially impact the result or the quality of the trial. So it's a very robust trial where COVID did not have a significant impact in terms of the results. Of course, in terms of the execution of the study, it was sometimes hard, especially last year, to enroll the right number of patients, but it didn't swing the result in any direction. So no material impact of COVID.

Operator

The next question comes from the line of Andreas Buchbender from Medical Strategy.

Andreas Buchbender - *Medical Strategy GmbH - Analyst*

Thanks for taking my question; it's rather naive. When you think about the data regarding PEPAXTO, you see it's definitely noninferior to Pomalyst, and to just scrape the border to superiority. The safety signals were similar, you said. When you think about the commercialization, what do you think -- why would doctors prefer PEPAXTO over Pomalyst?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. It's a good question. Well, I think this comes down to -- it can come down to subsets, particular patient characteristics. I think in the case here, what we're seeing is a higher overall response rate. We're seeing a stronger progression-free survival. We're seeing a regimen that is used once a month and is an IV formulation. In a lot of cases, the management of older patients, let's think about the population that we're talking about here, taking a pill on a daily basis and relying on a patient to remember to take a pill on a daily basis is much different than the compliance you know you get as a physician in controlling that point-of-care by having them come in every -- once a month and receive their dose of PEPAXTO.

So I think it's -- those factors help to drive the choice of regimen. And again, here being on the border of a superior and equivalent result, we're thinking more bullish about what we've demonstrated and believe that there'll be many situations in which PEPAXTO would be selected over pomalidomide based on the results here. So stay tuned for more details regarding the subsets and additional full body of evidence from the trial.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

Could I add something there, Marty? Is that okay?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Absolutely.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

Yes. I think also the -- as you know, the relapsed/refractory multiple myeloma market is very fragmented, but pomalidomide is seen as the best drug there is for these patients. And if the fragmentation stems from all these comorbidities, refractory status, tolerability issues, et cetera, that these patients have. So it is a bit of an illusion to say that all these patients have so many options because in reality, they don't. So regardless of the real statistical outcome here in the end, even though we feel strongly that we'll see what this ends up as ultimately in the eye of the regulators, right? That there is a huge group of patients for whom pom is not an option. It is considered the strongest performing drug in this segment, and we are clearly same or better than pom. That's a solid position.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

And Jakob, it's Klaas here. I would also like to add one thing that I haven't mentioned yet. We have shared this data with some of the investigators on the trial to get their perspective. And I can only say that they were highly impressed by the data, looking at the curves, some other details. And I think going back to the question, why do you think that we could replace Pomalyst in the market, I think the early signals that we have gotten from physicians on the study who have seen the results are very encouraging.

Operator

The next question comes from the line of René Wouters from Kempen.

René Wouters - *Kempen & Co. N.V., Research Division - Research Analyst Life Sciences*

I just wanted to confirm that there are no major or key differences in patient characteristics between the arms of the trials.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. That is the case, René.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Marty?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. Yes. Please expand upon that, Klaas.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

No. I think the short answer is no. It's very well balanced as you would expect with a randomized trial here, but we were pleased to see a very well balanced study between those arms.

René Wouters - *Kempen & Co. N.V., Research Division - Research Analyst Life Sciences*

Okay. And then second question, please. So can you remind us about the filing strategy in Europe? And how these data fit in there?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Of course. Absolutely. Yes. I'll let Klaas comment on the regulatory cadence here.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. So for the U.S., it's pretty straightforward. We will file for an sNDA later this year, and we believe we have a strong case based on the totality of the data to, one, get full approval, but also to get a label update, hopefully, to earlier lines of treatment. When it comes to the EU filing strategy, this study is intended to be used as a confirmatory study of our HORIZON trial, which is currently under review for conditional approval in the EU. So that's, in short, our strategy when it comes from a regulatory point of view. So it will be used for both geographies.

René Wouters - *Kempen & Co. N.V., Research Division - Research Analyst Life Sciences*

Okay. And congratulations on the results.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Thank you very much, René.

Operator

The next question comes from the line of Christopher Uhde from SEB.

Christopher Winston Uhde - *SEB, Research Division - Analyst*

So when I turn this around, I guess, there's sort of an outside chance of turning this into a superiority demonstration. But I guess, as it stands, when you've got noninferiority as what you've demonstrated, how do you see this affecting your ability to get reimbursement, particularly in the EU and to get reimbursement in the U.S. in earlier lines of therapy? I guess, Bristol Myers is going to be lobbying KOLs pretty hard to limit the strength of the language on -- in any guidelines. How do you see -- I mean you're David against 4 Goliaths currently with a bunch of other mechanisms. I mean, I guess, a score of drugs in development coming up behind you. So is the risk -- is there a risk that you get squeezed at the back end and are unable to penetrate earlier lines of therapy? I guess that's my question.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. So maybe I'll start off just thinking about the U.S. I mean, the current label is after 4 prior lines. So we're pushed pretty far back. And thanks for the question, Chris. Really appreciate it. We're pushed into the area of high unmet medical need based on the products that are available today. So that pushes us back to fifth line plus. Clearly, based on the OCEAN trial, we're looking at third and fourth-line patients. So this is a significantly

larger patient population. I would not anticipate there to be a much challenge at all regarding guidelines and the authorities on those guidelines to widen the scope as it relates to PEPAXTO based on this extremely strong evidence in direct head-to-head comparison versus pomalidomide.

We think the totality of the evidence from a regulatory perspective is going to be strong. But I'll turn back to what Klaas was saying regarding clinician reaction. Again, it's early days, right? We just have these data now. And certainly, I think, we'll see this play out even greater as we're able to present this data to more clinicians and they're exposed to it in a peer-to-peer setting. But we believe that they'll be highly motivated to select our drug. So from a reimbursement perspective in the U.S., don't see any challenges. From a regulatory or reimbursement perspective in Europe, of course, it's kind of early days to begin thinking about that.

Certainly, pomalidomide is a widely used drug in Europe as well. And it's a drug that has a pretty decent price point as it relates to it. So being able to obtain a price point similar to pomalidomide would be excellent and then driving -- we believe the data then will stand on its own regarding our ability, as Jakob and Klaas have articulated, into a patient population that has very different needs. It's very much a -- not -- 1 drug is not appropriate for every single patient. We believe that the totality of the data from the OCEAN trial opens up many opportunities for PEPAXTO to be a very significant and foundational treatment in relapsed and refractory multiple myeloma, independent of the geography.

Maybe I'll also turn that back to Klaas and Jakob for their comments and thoughts.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

I think you said it all, Marty. I think 1 comment on the EU strategy, where a non-inferiority result would stand, regardless, on its own. And I think the fact that we're so close to superiority will make us having a stronger case. Needless to say that, of course, we -- our filing strategy in Europe is first focused on HORIZON, which will be the starting point for our reimbursement discussions, where OCEAN will be then used as a confirmatory trial. But right now, I don't see any true impact of the OCEAN trial on the planned reimbursement strategy for Europe.

Christopher Winston Uhde - *SEB, Research Division - Analyst*

Okay. So you mean -- so I mean, in general, when we have new cancer drugs coming onto the market and trying to go head-to-head with an incumbent, particularly very well entrenched incumbent with best resources, we typically see that they have to have a lower price. Can you elaborate on why you don't believe that would be the case here in myeloma?

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

Could I just add, Marty?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Sure.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

So, yes, it's not on the price as such, Christopher. And I think your questions are really well put. But I think you need to look at the activity of the drug for these patients. In add-on trials, in relapsed/refractory multiple myeloma, the average hazard ratio is around 0.65, and they include PomDex at the bottom. If you translate that, that means that you're adding half unit of pom in activity for these patients. That's your treatment effect, half of pom.

We have a hazard ratio of 0.8, statistically noninferior, but if you look at the point estimate, we are adding 1.2 units of pom activity to these patients. And that is ultimately the bucket of activity that we need to provide. So I think in a way, you're a little bit stuck in the framework of add-on trials

when interpreting the hazard ratio here. And obviously, this will be an in-depth discussion. But the activity level we have shown for these patients is a very steep mountain to climb, and we have really over-delivered. And I need to stress that.

Marty, sorry for that. Yes, your point, sorry.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. Yes. No, no. Thank you for that. Also, Chris, in the only market that we have the drug currently available, when we look at the landscape and pricing, we're approved as a triple class refractory agent in relapsed/refractory multiple myeloma. We looked at the price points for the 2 most recent entries. And in particular, that was selinexor and belantamab at the time. And of course, ide-cel has since launched, or BCMA, at a way different price point. But we price with the future in mind and with our vision in mind. We price with a vision that this OCEAN trial would be the success that we see it as today. So we price with pomalidomide as a reference point. So from that standpoint, we've kind of taken that into account. We feel very, very confident in both the pricing, the benefit-risk profile and our future opportunities related to PEPAXTO uptake and commercialization.

Look, we're out there in the market today as are others with data that is not robust head-to-head data, that is not data head-to-head against the most widely used drug. This is a very significant opportunity from a commercialization perspective when you show these kind of results.

As it relates to Europe, I mean, there's still -- again, it's work in progress. We're early days. We're a day removed from having this top line data. As you know, our pressures are to get it out as soon as possible. There's a lot of work to be done on understanding the full data set. But again, we believe the totality of the data provides -- will provide for a very robust opportunity in Europe and other geographies.

Operator

And we have a follow-up question from the line of Peter Welford from Jefferies.

Peter James Welford - *Jefferies LLC, Research Division - Senior Equity Analyst & European Pharmaceuticals Analyst*

So just very briefly, just with regards to the FDA, just so I can understand here -- the different scenarios here. Is the -- does the full approval scenario with regard to that -- does that necessitate getting OCEAN data on the label? So I guess what I'm saying is, are we really saying here that -- do OCEAN data get on the label as a new label indication? Or do they get on the label as just in the clinical data section? Is one of those required for full approval? Or can [this convert] get full approval with no mention of OCEAN data because the FDA doesn't regard this trial as a success to put on the label? And that there is sort of conversion to full approval scenario.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. Good question, Peter. And I believe one of the potential outcomes, and we believe that it's not the ultimate outcome, could be a full approval with the data not being in the label. Obviously, as Klaas mentioned, we believe that the totality of the data and the results here support a full approval and inclusion of the OCEAN data set and expansion of the indication into earlier lines of therapy. And that's what we will be seeking, but there is a case where what you're describing could happen.

Klaas, any further thoughts and reflections on that?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. I think you said it well, Marty. Of course, we don't have a crystal ball. But based on the totality of the data, again, and I cannot stress enough how much we are looking forward to share the data at a scientific meeting. I think we have a very, very good shot at also changing the indication

based on the OCEAN trial. Of course, I have to put a disclaimer there because we don't know. But based on the totality of the data and what we hear from KOLs, but also from our regulatory people, we feel confident that we have a good chance there.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

[To add to Klaas comment] also, Peter, and that is -- it's 2 separate processes, the confirmation and the label. They, of course, merged in terms of package, but actually the rules and regulations around them are separate. The confirmation requires randomized data. The label is, of course, the regular path. So it is actually technically 2 different topics, as per your question.

Operator

And we have just 1 more question from Patrik Ling from DNB Markets.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

Just a short follow-up question. I mean, have you looked into where the physician assessment and the Independent Review Committee assessment -- where they differed? And why they ended up in slightly different outcomes?

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

That's a great one. I'll turn that over to Klaas and Jakob both for comments.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes. The short answer is yes. We looked at these discrepancies. And it's not without a reason you have an IRC because they have an independent look at the data. We believe that you will see -- as you saw, the results are very close, whether it's from an investigator assessment or an IRC. And there are always judgment calls involved. It's not easy to say, one is right or one is wrong. And I think this is also important when we consider the fact that the FDA will look at it from an individual patient level, where it go -- could go either way in being superior or not. But then, again, regardless of where we land on superior or not, I think, the totality of the data will still be the leading part of the discussions with the FDA.

Patrik Ling - *DNB Markets, Research Division - Senior Analyst Healthcare*

And given the tightness of the data, are we talking about many patients where the IRC and the physicians differed in their opinion?

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

It's what you would expect.

Jakob Lindberg - *Oncopeptides AB (publ) - Chief Scientific Officer*

Yes. Normal.

Klaas Bakker - *Oncopeptides AB (publ) - Executive VP & Chief Medical Officer*

Yes.

Operator

And as there are no further questions, I'll hand it back for any closing remarks.

Martin J. Duvall - *Oncopeptides AB (publ) - CEO*

Yes. Thank you, and thanks to everyone for joining us this morning and digging in. Excellent questions. Just want to reiterate how proud we are of the data set, how excited we are to bring this forward to conferences first, to clinicians and decision-makers on prescribing -- prescribers secondly and, of course, regulatory authorities. It will be -- will also be critical here. So we look forward to providing you updates on that. Again, thank you for your participation and look forward to talking to some of you very soon. So thanks very much. Take care, and have a good rest of the day.

Operator

This concludes our conference call. Thank you all for attending. You may now disconnect your lines.