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EDITED TRANSCRIPT

ONCO.ST - Oncopeptides AB (publ) To Present Updated Ocean Results Call

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PRESENTATION

Operator

Welcome to the Oncopeptides Audiocast Press Conference 2021. (Operator Instructions) Today, I'm pleased to present CEO, Marty J. Duvall. Please go ahead. Your line is open.

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

Great, thank you. And good morning, everyone. And pleased to provide, today, updated results on the OCEAN trial and also a communication of a partial clinical development hold for our lead product, melflufen. So joining me on the call, Slide 2, will be Dr. Klaas Bakker, our Chief Medical Officer; and also Jakob Lindberg, our Chief Scientific Officer. And on Slide 3, we will be making forward-looking statements. So I ask everyone to look at our submissions and representations for complete fairness and accuracy that you'll find on our website and in other filings.

So looking at Slide 4. So what are the key takeaways here? And this is mostly associated, of course, with the OCEAN trial. And recall that this is a bold head-to-head study versus pomalidomide, so 2 very different mechanisms of action in patients with third- and fourth-line relapsed refractory multiple myeloma. So we are pleased to report today that the Independent Review Committee has reassessed and the trial now meets its superiority on the primary end point of progression-free survival across the intent-to-treat population. We'll provide a little bit more detail on that process and how it came about, but now meeting superiority on the primary end point in the intent-to-treat population.

We also see, in the trial, mixed overall survival results. So we'll describe to you how that's the case and where those mixed results do reside. And also, we are targeting a particular meeting, the IMWG Meeting in September in Vienna. We'll be submitting a late-breaking abstract. We obviously don't control the acceptance of that abstract. But we just wanted to make everyone aware that, that was our target for providing the full data set and full data disclosure. And we're also working very hard on a publication of the OCEAN trial results.

So as it relates to clinical development, because of those mixed for all survival results, the trials that we have ongoing, which are in those early lines of multiple myeloma, are now placed on partial hold. And we will describe some of those trials and the rationale behind that. And please note that we are committed to work with the FDA expeditiously to get these trials back up and running so that we can further study our drug in this important patient population. And finally, I do want to communicate that we -- our PEPAXTO commercialization in the U.S., there is continuation of the marketing based on the HORIZON label.

So with that, I'll turn it over to Dr. Klaas Bakker.

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

Yes. Thank you, Marty, and good morning, everyone. If we just go to Slide #5, just a short background, the study design of OCEAN, which is, as Marty stated, a head-to-head study of melflufen plus dexamethasone against pomalidomide plus dexamethasone, 2 very different drugs from a mechanism-of-action perspective and a quite unique study in that it is not often that you see 2 drugs against each other where you basically can really identify the strength and weaknesses of both drugs. The primary end point was PFS as assessed by the Independent Review Committee, and we'll talk about that more a little bit later, and 2 key secondary end points being overall response rate and overall survival.

Go to Slide #6, please. If we look at what happened, the top line results were disclosed on May 25, as you may all remember. Today, on the 8th of July, we disclosed the final Independent Review Committee PFS results. As Marty stated, in Q3, hopefully, at the IMWG, we will present the data to a broader audience at the scientific meeting. And at the same time, we're working on a manuscript. These time lines are all unchanged. So with that, we also still have -- because we have a superiority trial, we still expect to file for an expected sNDA around year-end or early next year.

Next slide, please. So the top line results were communicated on May 25 last year. After that, there was a deep dive on the data. And this is normal because you need to prepare data for your clinical study report, the CSR. But for regulatory interactions, you need to look deeper into the data to prepare your file with the FDA. During that deep dive in the data, we became aware that some of the imaging results were not provided to the IRC at the time of their assessment, and this has been done by our statistical vendor, who is actually the responsible body to make sure that all the data from clinical database is actually shown in worksheets to the IRC members. So while all the information is there in the clinical database, it's always an extraction of the database that comes in front of the IRC. Note, this is a process completely independent of Oncopeptides.

Because of this finding of initially 1 or 2 patients where we could see that imaging had been lacking, we asked the CRO to do a full review of all 495 patients to see if there was any discrepancy between what was provided to the IRC and what was in the clinical database. Based on that, we landed on 29 patients where a reassessment had to be performed because of changed data points in the clinical database. Please note that after May 7, the clinical database has never been opened. So this is only about what is extracted from the database and put in front of the IRC members. There were no data points changed, and the database remained locked throughout this process. Also, we made the FDA aware on a very early notice that this was going on, and this was happening in the background, and we had an ongoing dialogue with the agency throughout this process.

Now what did this lead to? If we go to the next slide, Slide #8. The primary end point, progression-free survival, as per IRC now has a hazard ratio of 0.792 with a confidence interval just below 1, thereby reaching statistical superiority with a p-value of 0.0311 with a relative median progression-free survival improvement of 39%. You will notice that the relative median progression-free survival for improvement is actually a little bit less than what was disclosed during the top line results and this all has to do with changing medians due to the reassessment.

The overall response rate stayed the same. This is logical since responses as such, is not changed, of course, during this reassessment. It was a reassessment of time-to-event data for the progression-free survival. So of note, this number didn't change. But because it's a secondary key end point, we actually like to show it here again with 32.5% for melflufen and 26.9% for pomalidomide. Very positive results.

Next slide, please. The overall survival data. As mentioned, this is a head-to-head comparison of 2 different treatment modalities. And hence, we saw striking differences between the performance of both drugs across different patient populations. On the ITT level, the overall survival hazard ratio was 1.1 in favor of pomalidomide for the full population. This is not statistical significant. But because it is other direction than the progression-free cycle, this of course leads to further analysis to see what explains this hazard ratio of 1.1. And what we and also the agency have seen is that in prespecified subgroups, there are large differences. So in some patient groups, PEPAXTO or melflufen does really well from an overall survival perspective. In other subgroups, pomalidomide does particularly well. This all adds up to a hazard ratio of 1.1. But we are currently further investigating this in close cooperation and collaboration with the FDA. This is all ongoing.

Now let's go to the next slide, Slide #10. So because of the striking differences in overall survival with some patient groups also being in favor of pomalidomide, as shown by the hazard ratio of 1.1, the FDA has requested Oncopeptides to put the program of clinical development in earlier lines of treatment on a partial clinical hold. The partial clinical hold means that we are no longer recruiting patients into our entire study program until we have a better understanding of the overall survival results and as such can determine which patients benefit most from melflufen. Patients on the study can stay under study, and this is subject to reconsent and assessment by the relevant investigators. So patients who derive benefit can stay on the drug.

This decision is effective immediately and impacts our LIGHTHOUSE study, which is the randomized study of melflufen, daratumumab, dex versus daratumumab; the ANCHOR study, which is the basket combination study with daratumumab and bortezomib; it's the PORT study, which looks at central versus peripheral administration; it's the BRIDGE study in patients with renal impairment; the ASCENT study in amyloidosis. And we also put -- and this is something that we did ourselves, temporarily suspended the COAST study with OPD5 as this is a very close analogue of melflufen.

And we first want to be absolutely sure, in cooperation with the agency, that we fully understand which patients benefit most from this drug and which patients do not benefit most of this drug. There will be a lot of speculation probably about how long a clinical hold will hold. We currently don't know, but it is reasonable to assume that it won't be a couple of weeks, but more in the time frame of months. But beyond that, it would be speculative to talk about time line. It's that, that we have a very active information flow ongoing with the FDA to resolve this issue as fast as possible.

Thank you. Marty, back to you.

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

Great. So Slide 11. So kind of in summary then, we see the OCEAN result. Very pleased to have met superiority on the primary end point of progression-free survival in the intent-to-treat population. As we look at, then, at the totality of the data, Klaas reminded on the objective response rate. In addition, we know the clinical benefit rate, duration of response are solid data favoring melflufen.

But it's in that overall survival secondary end point, important secondary end point, where the results are mixed. We look forward to Q3 and the IMWG meeting to present full data. As mentioned, clinical development, we are now on partial hold in those early lines of multiple myeloma. Treatment, partial clinical hold. And we hope to work and we will work closely with the FDA to resolve those issues as soon as we can. And we continue with the commercialization of PEPAXTO in the United States based on the HORIZON label.

So that wraps up the slide part. Now we can move into the Q&A and for that, I turn it back over to the moderator.

QUESTIONS AND ANSWERS

Operator

(Operator Instructions) Our first question comes from the line of Viktor Sundberg from ABG Sundal Collier.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

So first one on overall survival. So I'm a bit perplexed why the FDA is looking at the OS data already since it's premature at the moment. Could you give any more clarity into this? And also the rational for the big variations between melflufen and pomalidomide.

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

Yes. Good question. So it is immature. And certainly, we'll be following it longer over time. But with that, I'll turn it over to Jakob for comment.

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

Thank you, Marty. This is Jakob Lindberg, Chief Scientific Officer. I think it just underlines the uniqueness of the result, Viktor. First to have a head-to-head comparison between 2 different modalities. And as a sponsor here, we have actually failed to find precedent for a successful clinical trial where you have OS hazard ratios in large prespecified subgroups going from around 0.5 to around 1.5. And given the large differences, it is

clear, and it's also clear to us as a sponsor, that the hazard ratio 1.5 signals, for example, that those patients shouldn't be in our trials. And they should be excluded from any potential label. And there, we are in complete alignment with the FDA.

And given the very large OS differences, it is also clear that there is a very clear patient population that benefits from melflufen treatment. 0.5 that -- around that range is a very, very good number in the other end. I understand the agency's need to delineate and understand this data before we can continue with our clinical trial program, both that those patients that can benefit will receive the drug as well as those patients that do not actually receive pom. So I think it's important to understand how big the differences are in this material, which is a very unique clinical data set that, of course, from a scientific point of view, is extremely intriguing. But of course, as a sponsor of a study, you would rather have an easier trial result, but this will, for sure, be discussed in academic settings.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

Okay. And are there any risks that there will be a restriction on the current label that you have a fourth-line-plus setting, even if they don't -- or is it also related to this difference with pomalidomide?

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

Yes. We don't foresee that at this time. Of course, that's a different patient population, under accelerated approval, fifth-line-plus triple-class refractory patients. And a reminder, about 90% of those patients had pomalidomide.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

Yes. And how many of the 29 reevaluated patients have their results change when they looked at imaging, yes, once again?

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

So I'll turn that to Klaas for comment.

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

We don't know because we get the data in a blinded way originally. We will look into that to better understand that. But we don't exactly know in what arm the patients were, and we also don't know right now in which directions they have changed. Overall, of course, because the numbers have changed towards superiority, it be will in favor of melflufen. But whether that is based on 10 patients that have been like another result or 15 or 20, that would be speculation this time. We don't have full view of that.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

Okay. Sure And was the IRC committee made up of the same clinician that's in the first data readout or?

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

It's the same IRC as the -- no, the IRC is the same IRC.

Operator

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

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

A couple of questions. I mean when you talk about overall survival varying from 0 -- with the hazard ratio from 0.5 to 1.5, I mean, do you have a feeling for -- I mean you know your subgroup. So do you have a feeling for how large proportion of the sort of market that the OCEAN trial addresses that seems to be less suitable for melflufen versus the ones where you can say that it seems to be more suitable?

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

Yes. Good question, Patrik. And obviously, we'd be speculating a little bit there since it's probably not as straightforward as one might think. But let me turn over it to Jakob for comment.

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

I think we should be very careful in speculating exactly where, for example, the FDA's assessment will end here. But as you can understand, with these differences and the superior PFS results and an ITT OS hazard ratio of 1.1, we're talking about significant patient populations on both sides of the fence, so to speak. And then we should have to wait for their assessment. But we're not talking about the minority versus the majority. We're talking about 2 very large groups on different sides of this border.

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

Okay. On the OCEAN trial, you also have a special protocol assessment. And as you've communicated about it before, it was primarily based on the primary end point of progression-free survival that you needed to reach superiority. You've done that now. So could you see this analysis from the FDA as some sort of early label discussion really for OCEAN to really find out what subgroups are suitable or not?

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

Yes. Well, certainly, the dialogue is going to be very active and has been. It's been continuous since the first release of the data. And as we've kind of discussed over the past year or so, looking at inferiority or superiority with some regulatory bodies, it's particularly important for a superiority outcome. So we're pleased that in the intent-to-treat population, any final IRC results that we have that superiority on progression-free survival. But I'll turn it over to Klaas, as he thinks about some of the regulatory interactions and how this might proceed.

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

Yes, sure. And please note that SPA is still valid. So that means that if you reach superiority, you kind of fulfill the first requirement to sit on the table with the FDA, so to say, to talk about a label expansion. The point is that overall survival is also considered to be a safety end point because ultimately, the overall survival event is leading. And it is the regulatory body's responsibility, before taking any actions on approving a drug in a certain patient population, to make sure and to be absolutely sure that there is harm to patients before you enter that stage. So although the SPA is still leading, from a regulatory perspective, the first action that always needs to be taken is around safety.

And overall survival is such an important end point. It is, in the end, the golden end point. And that leads now to this, I would say, interjection by the FDA to actually, first, understand that better before we move on. But as said before, reaching superiority is very important, will get us a seat at the table. We think we may still have a good shot at a label. It's just that we first need to sort out to see which patients do benefit from melflufen

and who do not. And currently, the focus is at least to make sure that we do not provide melflufen to who actually do not derive benefit from melflufen. So in a way, they are kind of separated from each other, the SPA and the superiority and this investigation that is based our drug candidate.

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

I think, Patrik, it's also fair to say -- and Klaas, that OS is obviously a very, very critical end point, as Klaas mentioned. In a trial like this in earlier lines of therapy where people -- where patients will live beyond their third or fourth line, there's a confounding factor of subsequent therapy, and all of that needs to be worked out in this analysis as well.

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

Okay. But when it comes to the difference in overall survival, you can -- based on the analysis that you've done up until today, you can be sure that it's not due to an unexpected side effects or anything that has been caused to the patient by your drug?

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

I can't comment on that.

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

Or is that something completely different?

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

No, I can't -- and thanks for asking that question. The answer is you are correct. And there are no -- as far as we can see now, there are not unknown adverse events or new safety signals as we have also already mentioned in the top line results update. That still stands. We have not identified a new safety signal in terms of toxicological terms. It's only the overall survival that we need to understand. But so far, we haven't identified a classical safety issue, so to say, that is associated with this overall survival.

What we really look at is various layers of efficacy. So one patient group seems to be more benefiting from this drug than other patients. And some do benefit really well from melflufen. Some do not really. But this has nothing to do, as far as we can say now, with safety as such. So when I say the safety measure, that's what I mean. The FDA just wants to understand who to expose this drug to from an efficacy perspective but not because they are concerned about more adverse events here.

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

Okay. Last question. Do you see any correlation between the variation in overall survival with overall response rate and PFS? Do you see the same type of variations for the same type of subgroups for the other measures?

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

I don't...

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

For some, we...

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

Yes. Go ahead, Jakob.

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

So for melflufen, there is a very consistent path between increased response rates, increased PFS, increased OS. So the entire target end point system, so to speak, from an efficacy point of view, makes perfect sense in the melflufen part. It is much more tricky in the pomalidomide arm, where the PFS is basically very stable across all subgroups, but they always varies tremendously across the prespecified subgroups, which makes this analysis a bit tricky. But for melflufen, your statement is absolutely true, the efficacy end points and the OS end point goes hand in hand.

Operator

Our next comes from the line of Christopher Uhde from SEB.

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

Okay. So it sounded like -- maybe I'll just ask this one first, since you sort of alluded to it earlier, but just to be clear, so bearing in mind the OS finding, would you say your confidence in full approval and/or label expansion is increased or decreased compared to when you first announced top line? And do you see any difference in how you view the FDA and EMA processes?

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

Yes. Maybe I'll turn that one -- thanks, Christopher. I'll turn that over to Klaas for the first comment.

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

Right. And thank you, Christopher. We -- and from the beginning, also on the top line results, always have said that based on the totality of the data, we feel very confident in our interactions with regulatory authorities. The fact that this now has changed to a superiority result had even further increased the chances of regulatory success. So when you speak about full approval, I would say there is a substantial possibility that it will not be a label because that covers the full ITT population. Because if you have a hazard ratio, and as Jakob already said, sometimes going to 1.5 on overall survival and that turns out to be true, it's hard to kind of include these patients in a label. So we are not -- it's not the base case anymore that we will get a full approval on the full ITT population based on these results.

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

Okay. That's helpful. Then my next question is -- so it initially struck me, but I guess your last comment there on -- related to unexpected side effects, whether there are any that suggests that's unsafe. But I was immediately thinking of venetoclax initially. Are -- is there -- so first of all, I guess, is it correct that infections are not even part of the problem here in terms of the OS, let's say, deficit in some subgroups for melflufen? And then is the next thing to do with t(11;14)?

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

Okay. Yes, I can answer that question. This is completely different situation when compared to venetoclax. Regarding infections higher in certain subgroups, that is not the case. And we don't see a delineation across subgroups according to t(11;14) that you just mentioned. So it's -- that is not the case. And we look at a completely different situation than venetoclax here. Jakob, anything to add there?

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

Yes, I would just echo what you said. Actually, when it comes to infections, it favors melflufen in this study as will be shown at a future conference. No, I mean, we have done a very thorough job of analyzing this. And just like you, Christopher, our first thought was that this was adverse-event driven, and it wasn't. From my interpretation point of view today, this is just a stunning difference in efficacy between 2 different treatment modalities across different patient populations that we are seeing. So we're seeing true efficacy differences between these 2 drugs. And once again, from a clinical scientific point of view, I think it's a treasure trove of information. But obviously, this may create a complication for us in the regulatory interactions and to determine exactly how to define these 2 patient groups, those that benefit and those that do not.

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

Okay. That's very helpful. And I guess my last question is just a clarification. Since you mentioned that the database has been locked throughout since May, is the OS change, I guess, due to the then reappraisal of the existing data from -- coming out of the IRC look again? Or is it to -- actually to maturing data?

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

And I'll turn to Klaas.

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

Yes, that's a good question. So the IRC reassessment has nothing to do with overall survival. That was -- that's such a hard end point that you don't need to reassess that. It's -- from a clinical database perspective, you look at a certain date and then you extract the data. That hasn't changed, and we have not seen other OS data today that we have seen on the very first day. What we did understand that, the first day, that it was very immature, the overall survival end point, and very difficult to read. So we needed further analysis to better understand what we were seeing at that time. But no, there has been no update in overall survival results.

Operator

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

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

I've got 4 questions and perhaps we'll take them in turn. So firstly, just with regards to the overall survival again. So I appreciate you say there are no new safety signals. But can you confirm that this is not related to the existing, for example, hematological toxicity and it's not the hematological toxicity that is causing this difference, and equally, that it's not related to duration of therapy because, I guess, duration of therapy obviously -- I guess, I'm saying it isn't -- it's not the cumulative effects of those heme tox events over time that's leading to this OS difference?

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

Yes. Thanks, Peter. I'll let Klaas touch on that one.

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

Yes. Thanks, Peter. And 2 very relevant questions and 2 relatively short answers. No, we don't see this being related to heme tox. And it's not due to any differences in duration of treatment.

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

If I could add one. Jakob here. Peter, you actually -- if you look at the OS while on therapy and within 30 days of the last dose, we have a positively trending OS and you have a larger amount of patients receiving subsequent therapy in the melflufen arm compared to the pomalidomide arm.

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

Okay. And then the next -- the second question is just with regards to disclosing this subgroup. I mean I guess you obviously are approved HORIZON -- the HORIZON indication and you also have open access programs in Europe. And I'm thinking what is your thinking regarding the time line of disclosing these subgroups. I'm thinking ahead of September even given presumably, there are potentially patients who may be later lines who perhaps should not get the drug anymore based on these subgroups or am I misinterpreting?

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

Yes. No, I don't think you're misinterpreting. I think our initial look and certainly, of course, the communication with the agency has been that the current label is not impacted. So based on our look, we don't see later lines of therapy being impacted. But of course, analysis will continue, but that's not where we stand today. Maybe I'll turn it to Klaas also for comment here.

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

Yes. I think for now I can only echo what you say, Marty. The patient population of HORIZON is so materially different from OCEAN, much later-line patients with limited clinical treatment options, and that the subgroup results that we see, based on the analysis that we have been doing, quite extensive analysis, we don't see a repetition of that in HORIZON, so to say. So at this moment in time, we feel comfortable with the patients who are currently getting melflufen within the HORIZON label. We have no concerns about that.

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

And let me repeat, so we are -- so we're comfortable with a target of a September disclosure on these subgroups and the full data.

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

Got it. And then the third question is just with regards to the comment that was made on the confidence in approvals but not for the entire population. I guess I understand the thinking. But if you were to exclude, I guess, the patients that have unfavorable OS, I guess, how is your confidence then that the PFS benefit is still going to be meaningful, and ideally statistically meaningful, if you just take that subgroup of the population? Because, I guess, to get approval for just the subgroup would then rely on essentially lowering the aim of the study.

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

Yes. I think -- and I'll hand this to Klaas for comment as well. I mean Jakob made one point about the overall directionality of the secondaries of overall response, depth of response, duration of response as it relates to PFS. And I mean -- and Klaas made mention of the fact that in the

intent-to-treat population, the PFS meeting superiority makes this a positive trial. And with the trial being positive, then the analysis of subgroups and the meaningfulness of subgroups becomes more important. So we kind of see that sequence of events. I would speculate you'll find directionally the numbers to line up. But maybe I'll turn it over to Klaas and Jakob for their comments as well.

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

I just want to echo what Jakob mentioned earlier. For melflufen, it's quite stable, as we say here. And what I mean by that is that when there is a clear PFS benefit, that also translates into an OS benefit and the reverse. When the PFS benefit is not really there, the overall survival is not going into another direction. It's really pomalidomide which behaves differently here. Going back to your question, we have the firm belief that we see a benefit on the primary end point for melflufen that also translates into good overall survival results. So we have full confidence that some major subgroups, actually everything goes in the right direction, so to say. So we don't see, for melflufen, specific patient populations where the PFS number doesn't hold in the OS analysis anymore. So that makes us pretty confident on that level.

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

It's Jakob here. I could just add one layer more. Under the assumption that your end points move in the same direction, which I think, Peter, you have heard that they really do in this trial for melflufen, then it means that you have proven benefits by reaching your primary end point and in this case, a superiority on PFS. So what the regulator would do, presumably and our speculation, is -- and there is a lot of precedent for this, is that you exclude subgroups based on risk, and the bar you need to reach to exclude the subgroup based on risk is, of course, much lower than to prove benefits. But you have proven benefit on the ITT level already, which means that the exclusion is based on risk assessment rather than benefit assessment. So -- there are -- there is precedent for this in multiple trials, actually, this exact way of thinking. Thank you.

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

That's interesting. So final quick question then, it's just with regards to the core, I guess it's the event that started this, really. Just trying to understand what the cause of this was. I mean it seems as though the IRC had, I guess, and the sequence of events, I mean, they have time to do this and since when the study was fully enrolled and then the events occurred and happened. So I guess just curious what the cause is of this error and who's -- I guess, to cut this short, who's to blame for this because clearly, something, somewhere -- I mean, I've done this a long time, and if someone, somewhere clearly makes a mistake in the analysis of this?

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

I'll turn that to Klaas for comment.

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

Yes, sure. That -- as always, it's an addition of individual small things that add up to a result like this. What we can say here is that the process that has been followed here is a sequential read by the IRC of overall survival results in batches. So when -- every 3 months, there is a review of the event and the IRC reviews that. So that they don't review all 495 patients at the same time, which would be an awful lot of work. This is common practice.

Now between the last IRC meeting and the database locked, where data, of course, is cleaned, added subtracted to make sure that everything is correct, there was not a new IRC meeting after that to look at these changes. The IRC is managed by one of our vendors that's guided by us. So it's a combination of parties who are involved here. But to be -- this is as transparent as I can be. And just that because of the not looking at everything at one time at the end, but instead looking at it at various time points and then not later on look at patients where things have changed, that's, I would say, the main driver here of the fact that the first IRC read was not complete.

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

Yes. Just for a slight clarification, that was on PFS, Peter.

Operator

We have a follow-up question from Patrik Ling from DNB Markets.

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

Just a short question. Do you think that the FDA analysis and the partial clinical hold will be over by the time you present the data in September?

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

I think that might be a little tight. I'd be speculating a little bit there, but I'll ask Klaas to comment.

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

Yes. I think it's really speculative, so I wouldn't feel comfortable to specifically comment on that. As I said earlier, I think it won't be weeks. And then how many months is really speculative. The only thing I can say is that we work as fast as we can with the agency, and the agency is working very hard here as well to get this resolved ASAP because that is in the interest of patients and all parties involved. So no one benefits from a delay here. So we're just doing everything we can to get this clinical hold lifted as soon as possible.

Operator

(Operator Instructions) We have a follow-up question from Christopher Uhde from SEB.

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

So just following on, I guess, from a couple of your comments. The first one in terms of the bar to exclude being much lower than the bar for benefit. Does that -- it sounds like you're saying -- I mean given that you said these are 2 roughly equal very large populations. I mean does that -- it sounds a little bit like -- are you saying that 50% of the addressable -- of the what we would have considered the addressable market previously would be potentially excluded? Or is it more or less? And also, how do you see this affecting reimbursement in Europe?

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

Yes. So maybe I'll turn it over to Jakob to talk about the first part of that question.

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

I don't want to speculate in exactly because we need to finalize the assessment in collaboration with the agency regarding the exact delineation here. But as we have stated, we are talking about 2 very material groups, right, and looking at the data. But then it's up to the agency also to make their assessment and exactly where they will come out on that. We don't know. And I just want to highlight that your question about reimbursement will then be linked, of course, to what the final data set is in those patients that are selected in a potential label.

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

To add on that, Jakob, I think it's not unreasonable to state that the European payers, in general, do like medicines that are effective in almost all patients that are part of the group. So when you get approval, and this is highly speculative, if you get approval for a certain patient group where you derive a lot of benefit -- well, who derive a lot of benefit, the bar for reimbursement will be lower than going for full ITT population with not a benefit for all patients. So from a USP and payer perspective, it's always more interesting to look at a smaller group with higher efficacy.

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

Thanks. And I think that -- and maybe stepping back, Christopher and others, as we think about this, I mean, you think about an intent-to-treat population here, 495 patients and on the primary end point, as we've described, meeting superiority on PFS and directionally across the ITT population, favorable secondaries on overall response rate, clinical benefit rate, things like that. It's the overall survival that's confounding. So what that means is that, and as Jakob mentioned, we've got efficacy results on the melflufen side across the primary and secondaries that follow one another. So clearly, there are pockets of very large benefit. Again, getting back to the stunning efficacy differences I think is the way it was described, and this really is a treasure trove of information.

And tying that back into the point that Klaas is making relative to reimbursement on the European side and really, where we're at today in oncology and other deals, if we can define the population that benefits, that's the best place we can be, right? The highest-priced drug is the one that doesn't work. So the better we can define the population that the drug works in, the better off we are. And of course, our commercialization will be more crisp in having more solid results from a benefit/risk perspective in those populations.

Operator

We have a follow-up question from Viktor Sundberg from ABG Sundal Collier.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

Yes. So going back to the multiple study that you also referred to where you showed that overall survival depended a lot on the lenalidomide-free period. Is there anything there that's driving any difference in overall survival trends? Of course, that's for median overall survival but just curious about that point.

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

Yes, I'll turn that to Jakob and Klaas for their comments.

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

We don't want to disclose data today, but the answer is yes, Viktor. So you can find a lot of patterns in how extensive the lenalidomide use has been and how that impacts -- those patients that have used a lot of lenalidomide, those groups that favors melflufen strongly. Yes. But we will most likely disclose this at some point. I'm not sure at the IMWG if we get that presentation, but that's a very interesting set of data as well.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

But does that goes both ways, I guess, that if you had a longer lenalidomide-free period, you have better outcome or worse outcome from melflufen or?

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

Yes, yes. It goes bidirectional. That's correct, yes. I mean if you have a longer period and little lenalidomide before that, it benefits -- it favors pomalidomide. If you have a lot of lenalidomide use and short period, it favors melflufen, that's correct, yes.

Viktor Sundberg - *ABG Sundal Collier Holding ASA, Research Division - Research Analyst*

And just a final question. On the subgroups that we are talking about, do you expect to show any statistical significant, yes, benefit of the subgroup? Or will this be showing trends to the FDA? Or how should we think about that?

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

I'll turn that over to the -- to Klaas and Jakob.

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

I cannot -- short comment. One should be careful by talking about statistically significant when you don't -- they didn't have a null hypothesis around that. But what you can claim is that there's still 95% confidence interval is either larger than 1 or smaller than 1, right? And I mean those differences already exist in the materials.

Operator

Thank you. We have no more questions from the line. I will hand you back to our speakers.

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

Okay. Great. Well, thank you, everyone, for participating. And again, I think the key points here are, as stated, this meeting superiority on the primary end point of progression-free survival across the intent-to-treat population is very important. We see overall directionality positively in melflufen's favor across the intent-to-treat population as it relates to response rate, clinical benefit rate and others. It's the overall survival results that are mixed.

We are actively cooperating with the FDA to understand those results better. And in the meantime, our trials in the earlier lines of therapy are on partial clinical hold while we continue to market PEPAXTO in the United States based on the current HORIZON label. We certainly look forward to the IMWG meeting, where we're submitting a late-breaking abstract and targeting a full disclosure of the OCEAN data sets, and we look forward to that.

So with that, I'll wrap up. Thank you very much, and we'll talk soon. Bye now.