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Emerging Evidence in Focus: Interpreting Data on Next-Generation CSF1R TKIs

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Dr. Gelderblom:
This is CE on ReachMD, and I'm Dr. Hans Gelderblom. Today, I'll be reviewing emerging clinical data on next-generation CSF1R inhibitors, TKIs, in a disease called tenosynovial giant cell tumor, or TGCT.

The study I would like to discuss now is vimseltinib. The registrational study is called the MOTION study and was published in *The Lancet* in 24. The study randomized vimseltinib 30 mg twice weekly vs placebo 2:1, and there was an open-label period after.

The response rate was 40% altogether after 24 weeks. And you can see from the waterfall plot that there were no responders in the placebo arm. So these were meaningful differences regarding response. But most importantly, the study was also positive on all secondary endpoints, including active range of motion, PROMIS performance, worse stiffness, and the pain and quality of life, which is, I think, very important for our patients.

The 2-year follow-up data were just presented at ESMO 2025, and Figure 1 shows very nicely the design of the study with a half-year double-blind period, followed by a half-year open-label period, and then the extension periods. The overall response rate increased, as you can see, to 48%, and there were 19 complete responders and 21 partial responders. So there was some deepening in the response, as you can see with all these studies upon prolonged treatment.

What you can also see, that the common side effects like edema, pruritus, some arthralgia, asthenia, fatigue, etc., were seen with the drug, but they were mostly of low grade.

The MANEUVER study is an interesting study because this is the only study that's being done partly in China but also in Europe and in North America. The design was very comparable. Again, a 2:1 randomization for pimicotinib 50 mg per day vs placebo, and then crossover to active treatment for all patients, and then an open-label extension period.

From the waterfall plots, you could see there was, surprisingly, a response in the placebo arm, but you can see the activity that is quite pronounced in the whole group, and in the figure, you can see that. The overall response rate by RECIST 1.1 was 54 versus 3.2% at week 25. The tumor volume score—and I talked about this earlier—is probably a better measure of outcome of the size of a tumor—the irregular, long-shaped tumors—that we see in this disease. The TVS response rate was 61.9% versus 3.2%.

The long-term data—and this is not the last presentation, but this study started later than the other ones—were presented recently at the ESMO Congress. And you can see the deepening of the response here as well. The overall response rate is 76.2% now according to RECIST and 74.6% according to TVS. So this is really nice to see. The typical side effects, as we see with all these kinds of drugs.

So specifically for the US situation, pexidartinib has been available for quite a while already, vimseltinib as well. And I would expect, with the current results that we have seen now, that pimicotinib will also be available there. In Europe, pexidartinib is not available, but probably the other 2 drugs will be. So it's good to have a choice.

And how to choose between them—I think it's very difficult to give you advice here because it's very difficult to compare between studies. You can see here that the response rate was a bit higher with pimicotinib. It's been tested on Asian patients, but again, RECIST might not be the best way to measure this disease.

I think it's very difficult. I'm truly happy that there will be drugs available so we can avoid these repetitive operations. And if you get your hands on these drugs and they are reimbursed in your country, I would certainly advise you to centralize treatment, have multidisciplinary decisions together with the patient. And I hope for the best for the patients in the future.

Thank you very much. My time is up, and I hope you enjoyed this session.

Thank you.

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