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Released: 12/15/2025
Valid until: 12/15/2026
Time needed to complete: 1h 01m

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# Clinical Evidence in Focus: Interpreting Data on Systemic Therapy Targeting CSF1R

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**Dr. Gelderblom:**
Hi, this is CE on ReachMD, and I'm Dr. Hans Gelderblom. Today I'll review clinical data on systemic therapy targeting CSF1R in tenosynovial giant cell tumor, or TGCT.

The first registrational study was the ENLIVEN study. This was a randomized study with pexidartinib vs placebo. The randomization was 1:1, and the first part was 24 weeks. And after that, all patients had access to pexidartinib, so the placebo patients could cross over.

The primary endpoint was overall response rate according to RECIST at week 25. And this primary endpoint was met with 39% versus 0%. But most importantly, all the secondary endpoints were also reached. Amongst them, range of motion, pain, and PROMIS, which is a specific quality of life questionnaire for this disease.

Of note, 3 patients had cholestatic hepatotoxicity, 1 lasting more than 7 months and confirmed by biopsy. So this was a major thing in this study.

The long-term results of ENLIVEN were published in *The Oncologist* this year, and you can see the deepening of the response after longer follow-up. The toxicity you can see on the right-hand side. Specifically for this drug, the off-target KIT inhibition causes hair color changes, and there's also rash and pruritus, some nausea, diarrhea, and vomiting and fatigue. So it's important to realize that these drugs don't come without any toxicity, but there are very few grade 3 and 4 toxicities. The PROs improved and were maintained over time.

The follow-up study was a discontinuation study for patients that wanted to have a break. And in 6 of the 11 patients—so more than 1/2 of the patients—in a treatment-free retreatment cohort, had progressive disease according to RECIST after a mean PFS of 22.8 months. Three of them were retreated and all with good effects. So this may be an opportunity, although you have to realize that there will be PD in between.

Emactuzumab is a monoclonal antibody. The first study was published in 2015, a French study, and you can see that the drug is quite active from the spider and waterfall plots on the right-hand side. You can see also that edema was a common side effect with this drug.

So the long-term data that were published 5 years later showed that the effect was sustained, as with many of these drugs, but the


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toxicity was also, well, common, let's say. You can see that from the left-hand side: pruritus, asthenia—70% and 62%—face edema 49%, and general edema 44%.

So finally, this drug was bought by another company, and a randomized study is now currently underway. It's called the TANGENT study. Results, I would expect to be at ASCO 26, maybe a press release before that. Emactuzumab 1000 mg IV every 2 weeks for 5 cycles are randomized against placebo infusions in a 2:1 randomization. And patients have the opportunity to be retreated if they experience progressing in the follow-up time without treatment. So this is an important study because this is the only study with a treatment break and also with a monoclonal antibody.

So my time is up, and I hope this brief overview of 2 completely different studies were of interest to you, and thanks for listening.

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