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Analyzing Clinical Trial Data: Part 1

Dr. Goldberg:
Revakinagene taroretcel was recently approved for the treatment of idiopathic macular telangiectasia type 2, or MacTel. What are the phase 3 data that led to its approval? This is CME on ReachMD. I'm Dr. Roger Goldberg, and here with me today is Dr. Thomas Albini.

Dr. Albini:
It's great to be here, as always, Roger.

Just to start the discussion of the clinical trials, there were two phase 3 pivotal clinical trials that were performed: NTMT- 03-A and NTMT-03-B. We'll call them the A and the B trials. And in both studies, patients aged 21 to 80 years diagnosed with MacTel were included. They needed to have an ellipsoid zone break between 0.16 mm2 squared and 2 mm2 and a best corrected visual acuity of 54 ETDRS letters or greater. These patients were randomized to either a sham surgical procedure or the actual implant. And the primary endpoint was the rate of change from baseline in EZ area loss through month 24. As we can see here, looking at the baseline characteristics, the arms in both studies were well balanced in every category, including baseline visual acuity and EZ area and the degree of foveal involvement.

The outcomes, the primary outcomes for the study showed in the A trial, that there was a 55% reduction in ellipsoid zone loss, and in the B trial, there was a 31% reduction of ellipsoid zone loss over 24 months. And these were highly statistically significant.

Remarkably, this implant was well tolerated for up to 9 years, and a comprehensive look at the safety of revakinagene taroretcel was evaluated across 6 clinical trials. Charlie Wykoff and Dean Eliott will be discussing those details in the next episode of the series. Most of the adverse events that were seen were transient and mild. The few serious adverse events that occurred were primarily related to surgery. As we discussed in our last episode, it's really important that the prescribed surgical steps are followed and careful attention is given to handling of the sutures and of the conjunctival closure.

There was also a post hoc analysis that assessed baseline features to determine which patients may benefit most from the treatment. Roger, can you tell us about these findings?

Dr. Goldberg:
Sure. This was a post hoc analysis to see if we could try to identify which features and facts would be most predictive of those patients best suited for treatment and most likely to respond and have kind of an outsized benefit.

And it turned out 3 factors really rose to the top of the pile here. One was the baseline lesion area or EZ area loss. If it was less than 0.5 mm2, there was a marked increase in the likelihood of patients having both a 20% or 50% reduction in disease progression. Younger age was also associated with better response to NT-501. And having the fovea not involved—so nonfoveal involvement—was also associated with a better response. And those 3 things really point to the importance of intervening early.

When you mentioned here the primary results in terms of trial A and trial B, and how there seemed to be a difference in the outcomes, I think it really speaks to this baseline EZ area loss. NT-501, NTMT-03-A, the A trial, tended to have more patients with smaller lesions, and therefore it showed a bigger effect, over 50% reduction in EZ loss over 2 years.

Trial B had more larger lesions, greater proportion of larger lesions, particularly in the NT-501 treated arm, and so therefore it showed still a significant effect, but not quite as large as was shown in trial A.

And again, I really think this speaks to the importance of intervening early in this disease.

Dr. Albini:
Well, Roger, you've shown us really nicely that early intervention seems to be potentially a key to successful treatment response. Like you said, the differences in the treatment response between the two phase 3 clinical trials may be related to the differences in the baseline lesion size. The implant was super well tolerated, and I think it is going to be a really exciting option for our patients in clinic.

Dr. Goldberg:
Yeah, I think you've summed it up beautifully. Well, that's all the time we have for today. Thank you to Thomas for being here. Thank you to our audience for listening.

Dr. Albini:
My pleasure. Thank you.