

SPEAKER 1: The most common mechanism for obtaining long-term outcome for treatment of Fabry disease has been using disease registries. There have been two disease registries active for the last 15 years. And they are both ERT-based. And they have indicated that there is a clear benefit.

One of the most interesting studies looked at risk for life-threatening events, related to Fabry disease, based on the time on therapy. And what we found with that one was that once you start a patient on Fabry treatment, it took approximately six months before you could see a difference in the risk for major complications. And the major complications that were tracked were renal failure requiring renal replacement therapy-- which is either a kidney transplant or dialysis. Heart disease that progressed to the point of requiring a new medication, or implantation of a pacemaker or a defibrillator. Or stroke or death related to Fabry disease.

So once patients were on treatment, they had a similar rate of complications for about the first six months. And then we saw a drop off to about half of the risk, for those major life-threatening, medical events, compared to untreated patients. But the risk for life-threatening events remained well above the general population risk.

Now the population treated in that study had an average age of onset of treatment around age 40. So it was relatively late in the course of the disease. We think that if we start treatment earlier and we treat more aggressively, that we can get an even better impact of the disease.

The chaperone drug is also being studied using registries. However, that drug is relatively new in the market. So the long-term outcome studies are still in process for that. The long-term follow-up studies are being done actively, for both ERT and for the chaperone drugs at this time. And are asking some overlapping questions. For example, the response to kidney disease.

But some of the things are being asked more specifically. So for example, one of the key questions for the chaperone registry is impact on GI symptoms. And also impact on the neuropathic pain. Those can and may be addressed in the ERT, but have not been definitively answered at this time.