SPEAKER: So when we're talking about treatment of Fabry disease, there is no age restriction for treatment with enzyme replacement therapy. It's approved for all ages as long as the patients have confirmed disease. It is difficult to treat patients below age 5, just because of IV access issues with the ERT. But if they're symptomatic, we recommend that. Or at least I recommend that, anyway.

For the chaperone therapy, the drug is approved only for adults. However, there are currently treatment trials being done in pediatric populations. So we should have studies getting down to age 5 within the next few years. And we anticipate that the chaperone should work in that age group as well.

Another area of controversy regarding Fabry disease is how long to treat. So as I have previously mentioned, I recommend starting therapy if there are any symptoms regardless of age. Once a patient qualifies for treatment of their Fabry disease, I recommend treating lifelong.

However, some people have asked, at what point should they stop therapy? And there are no well-established guidelines for that. The way I have approached that in older patients is to ask them if they are still making plans for the future. And if they are making plans, and have things that they hope to accomplish, and it's going to be more than a couple of years into the future-- so if they are thinking about things they want to do more than two or three years into the future that they should stay on therapy, whichever therapy they have chosen or has been chosen with collaboration from their care providers.

So there have been questions about whether or not to continue therapy if a patient's presenting complaint was, for example, renal disease and, in spite of treatment, their renal disease progresses to renal failure should they then continue on therapy. My answer is that, yes, they should because Fabry disease is a multi-system disease. And if you stop treatment when the primary indication for starting treatment was progressive renal disease, you are leaving the patients at continued increased risk for progression, with high risk for stroke and/or heart disease, and also at risk for progression of GI symptoms, pain, hearing loss, or lymphedema, which are also important parts of the disease and also warrant therapy.

Similarly, if a patient has progressive heart disease and gets a pacemaker or some other cardiac intervention, that may help their immediate symptoms of heart disease. But if you discontinue the therapy to decrease the stored material, the disease itself is likely to progress and become more difficult to manage. So I recommend continued therapy for patients, even if there is changes in the management, unless they get to the point where the patient is starting to think about end of life issues and they are comfortable with allowing the disease to take its course. Then at that time, it is quite reasonable to consider discontinuation of therapy.