SPEAKER 1: When Fabry Disease was first described, there were no therapies for it. So we did the best we could with symptomatic management. Then the idea was developed for enzyme replacement therapy. That was approved by the FDA in about 2003. And more recently, the FDA has approved a chaperone therapy, that's an oral medication.

Now the enzyme therapy is appropriate for use in any patient with Fabry Disease. The chaperone therapy is not as universally applicable. So the mutations have to be responsive to the chaperone therapy. And there has to be some ability for the body to make some of the protein that is the enzyme.

So for example a frame-shift mutation, where you have a deletion or duplication of part of the gene, will generally produce no functional protein. And is unlikely to be amenable to the chaperone therapies. Most of the time, if a mutation is a missense mutation it's more likely to be a candidate for a chaperone-- for uses of the chaperone therapy.

So that testing is done using a chemical assay. So they will take the enzyme produced using the given mutation, and add the chaperone to that. And then measure the enzyme activity. And if you get a response with an increase in the amount of enzyme activity, then that mutation is considered amenable to the chaperone. And then patients with that mutation would be considered candidates for treatment with chaperone.