

SPEAKER 1: It's not as simple as gee I'm amenable to the chaperone therapy, therefore I'm going to take that drug. There are pros and cons to each of the approaches to therapy. So I will illustrate that with a history of a family that I have treated.

So as a family with four affected family members with Fabry Disease-- now in a family you expect all of the people with Fabry Disease to have the same mutation. So in this family, the four affected individuals-- there are three men and one woman. The men had started on treatment with enzyme replacement therapy at the time of their diagnosis, because that was the only option.

But the lifestyles of the three men were quite different from one another. So we had one who was a medical student. He had a very unpredictable schedule and usually did not have blocks of time that he could use, which made getting the enzyme infusions very difficult. So when the chaperone therapy became an option, he actually called us and said am I a candidate for this? We looked up the mutation, and his mutation was found to be amenable to the chaperone therapy. With his lifestyle, being able to take an oral therapy-- which only takes a moment-- and then get back to studying or working or other demands on his life, was very important to him. And he wanted to switch almost as soon as the drug became available.

He had a brother who has a very predictable pattern to his day. Works regular shifts and whose boss was quite supportive of time off for healthcare needs. So in his case, when we talked to them about the possibility of switching to a chaperone, he asked about pros and cons. And wanted to know which one is more effective. And the bottom line is that the chaperone-- we didn't have enough experience to be able to predict how it would do compared to the established therapy. And for that individual the predictability and years of experience with the enzyme therapy, he felt was more desirable. So that brother opted to stay with the enzyme therapy.

And the other two family members decided that they would give them a few months, and then ask questions, and decide which to do. So they used the two brothers that had made the decision as guinea pigs. And have gone on and on and decided which option to pursue. At this time, the woman has opted for the oral therapy, because it's just more convenient. And the fourth individual is currently on enzyme therapy, but is wanting to try the chaperone therapy in the near future.