

SPEAKER: In treatment of Fabry disease, most of the early studies have been done on men. And there's a reason for that. This is in X-linked condition, and the outcome measures in the predictable pattern of the disease is better understood in men and women. The assumption has been that if a therapy or an approach to therapy works for men that it will almost certainly work for women.

However, there are some reasons to wonder if that is actually true because the pattern of the disease is different between men and women. So in men, about 60% of the men will have as their initial serious manifestation that's potentially life threatening of Fabry disease be progressive kidney disease. So the kidney disease is by far the leading life-threatening complication for men, followed by progressive heart disease as the second most common. But heart disease is the leading cause of death in Fabry disease for men.

Compare that to women, where, in women, 50% of the time, the first potentially serious complication of Fabry disease will be related to the heart. The second most common serious complication is stroke in women. And the third most common is kidney disease. Only about 10% of women will progress to end-stage renal disease, although many more than that will have some evidence of kidney involvement.

I think that this difference in the pattern of the disease means that the response to therapy may be different and the strategies used, both for monitoring and for intervening, may need to differ between men and women. So I think that it would be wise to design studies, either with minimal numbers of both men and women or to have separate studies done on men versus women in trying to optimize intervention strategies and to increase the understanding of the disease.