**Supplementary Material 2**

**Interview Script for Researchers**

Research using Patient-Reported Outcomes: General

Please tell me about your experience with use of patient-reported outcomes (PROs) in clinical studies in patients with lysosomal storage diseases.

Which patient-reported outcomes do you most commonly use? Are there any others?

Based on your patients, what are the symptoms that that you would be particularly interested in following?

Research using PROs: Benefits

What do you see as the value or benefit of using PROs in clinical research to identify or sort out treatment benefits or harms in lysosomal storage diseases or rare diseases in general?

Would you say that certain patient-reported outcomes were more useful than others?

If so, what makes them more useful? Can you explain why you think that?

Have you had any particularly good experiences using PROs, and, in particular, to help identify or sort out treatment benefits or harms?

Do you think there have been any missed opportunities in using PROs to help identify or sort out benefits or harms of treatment?

Have you ever had the experience that patients with lysosomal storage diseases are feeling appreciably better as a result of treatment? If yes, were PRO measures able to detect the improvement? If so, what about the instruments do you think made them successful?

Research using PROs: Challenges:

What do you see as the challenges or obstacles to incorporating PROs in clinical research to identify or sort out treatment benefits or harms in lysosomal storage diseases or rare diseases in general?

Have you had any unfortunate experiences in trying to using PROs and in particular to sort out treatment benefits or harms in lysosomal storage diseases or rare diseases in general? If yes, can you tell me about a specific example?

Do you have suggestions about how research could deal with these problems?

Wrapping up

Is there anything else you would like to say or add about these instruments?