**Supplementary Material 1**

**Interview Script For Health Technology Experts**

Measuring outcomes, in general:

What outcome measures do you think are the most important for researchers to include in research studies designed to potentially establish improvements or benefits?

Tell us about the details/features of outcome measures that you see as important.

Could you provide us some examples of outcome measures that you consider to be well done in experimental research?

Could you provide an example of outcome measures that are poorly done?

What suggestions do you have for researchers about ways for them to better use outcome measures in experimental research involving patients with rare diseases?

Role/value of patient-reported outcomes:

How important do you think it is for researchers to include patient-reported outcomes?

What qualities or features do you look for in patient-reported outcomes that are used in research?

Are there any patient-reported outcomes that you particularly favor? If yes, why is that?

What do you consider to be limitations or challenges of using patient-reported outcomes used in studies designed to investigate the benefit of treatments for patients with rare diseases?

Issues of research in rare diseases in general:

If we were to focus on research for patients with rare diseases in general, how well do you think it is done? What could be done better?

What do you see as challenges or limitations to the research involving patients with rare diseases?

Do you think that the “rules” or expectations for establishing benefit for rare diseases should be different in any way from research in other more common diseases?

Advice to researchers:

What advice would you have for researchers who are designing studies to investigate the benefits of treatment in patients with rare diseases?

What suggestions would you have for researchers working in the field of rare diseases in regards to the study design or use of instruments that would lead to a positive review of the proposed study?

Conclusion:

Is there anything else you would like to say or add about this topic before we end?