# The Use of Formal Expert Opinion Elicitation to Inform Model Structure and Inputs in Rare Diseases

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### **BACKGROUND AND OBJECTIVES**

An economic model is composed of two main elements: the model structure, typically identified by investigation of the care pathway, and model inputs, extracted from quantitative data. High uncertainty in both elements could confer a high risk that the economic model mispresents the true cost-effectiveness of the new intervention.

### **METHODS AND RESULTS**

Figure 2: Methodology used to produce the model structure and inputs for economic



High uncertainty is particularly prevalent in rare disease areas, when the diagnostic pathway is usually complex and variable due to a lack of clinical guidelines, and/or accurate, cheap or non-invasive diagnostic tests to identify the population of interest. Clinical trials for these conditions are also expensive because of the complexity of patient recruitment, leading to a lack of robust evidence of treatment outcomes.

This research developed a robust approach to inform the implementation of a costeffectiveness model in adult-onset Still's disease (AOSD), a rare systemic inflammatory disorder.

### Figure 1: Care pathway for patients with AOSD



#### analysis of AOSD Two Systematic **Structured Expert Care Pathway Reviews and Meta-Opinion Elicitation** Analysis Methodology elements Analysis To establish the common To find the published inputs for To elicit 34 missing model elements and discrepancies in inputs with plausible ranges quality of life, resource use, the pathway following interviews and clinical outcomes [2] from five European experts with 11 European clinicians [1] 3. Workshop to obtain 2. Individual elicitation 4. Mathematical Sub-element final quantities after aggregation of the 1. Training of experts exercise piloted on an group discussions and distributions (linear external expert to minimise bias to validate care (Fig.3A) pooling) pathway Model structure, R function Upper plausible Statistical simulated 10k Facilitator limit explanation of the values for each Lower plausible Clinical experts Detail quantities to elicit input and pooled Health economists limit (SHELF, 3) them together. Reviewers Median (M) Explanation of the Mean and standard Upper tertile (T2) Interviewers individual elicitation deviation was Lower tertile (T1) calculated (Fig. 3B) exercise

Figure 3: Quantities from the individual elicitation presented at the workshop (A) and the final distribution (B)



**Key:** JS, SS, AT, AS, JR are the expert identifiers; T1, T2 and M are the lower tertile, upper tertile and median

### CONCLUSIONS

Whilst producing a cost-effectiveness model for AOSD, we developed a robust methodology that can be adopted by other researchers to provide information and data that are missing from the literature. This is particularly useful in the context of rare disease areas, where there can be a complex and variable care pathway and lack of robust evidence of treatment outcomes.



<sup>1</sup> NSAIDs considered during diagnostic workup

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