



Case Study: Rare Pediatric Cancer

Study sponsor: A patient group

Condition: Rare pediatric cancer

- The current standard of care is chemotherapy with limited effectiveness and several side effects, including nausea
- Regardless of whether a child undergoes chemotherapy, the mortality rate is high
- There are both child and parent preferences and valuations, patients may have varying treatment priorities
- Some patients may be unable to communicate their preference

Motivation:

The patient group would like to spur medical product development by better understanding what outcomes are most important to patients and their parents. An additional challenge is recruitment in clinical trials. The patient group would like to also explore:

- What parents and children are willing and not willing to accept in a clinical trial protocol
- To see if preferences vary between the parents and the children