**Please nominate 1 category that best fits your submitted abstract:**

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| [ ]  Paediatric and/or congenital diseases[ ]  Maternal and/or prenatal health[ ]  Cardiometabolic diseases[ ]  Chronic diseases[ ]  Healthy aging[ ]  Cancer[x]  Neurodegenerative diseases[ ]  Public health[ ]  Other |

**Please nominate 2-5 subject areas relevant to your submitted abstract:**

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| [ ]  Aged care [ ]  Allied health [ ]  Animal models [ ]  Biochemistry [ ]  Bioinformatics [ ]  Biomarker research [ ]  Biotechnology[ ]  Cardiovascular research[ ]  Cancer [ ]  Cell biology [x]  Clinical research[ ]  Commercialisation [ ]  Computational biology and/or statistics [x]  Consumer advocacy[ ]  Dentistry [ ]  Developmental biology[ ]  Drug discovery[ ]  Drug target identification and validation[ ]  Education and training[ ]  Endocrinology[ ]  Environment[ ]  Epidemiology [ ]  Genetic counselling[ ]  Genetics, epigenetic or small RNAs[ ]  Healthcare  | [ ]  Health economics [ ]  Health policy[ ]  Health promotion[ ]  Imaging and computing[ ]  Immunology[ ]  Indigenous health[ ]  Industry [ ]  Invisible illnesses[ ]  Medicinal chemistry[ ]  Microbiology[ ]  Molecular biology[ ]  Neuroscience [ ]  Nutrition[ ]  Pain management[ ]  Pathology[ ]  Personalised Medicine[x]  Rare diseases[ ]  Physiology[ ]  Psychology[ ]  Public health[ ]  Reproductive biology[ ]  Technology[ ]  Tele-health[ ]  Virology[x]  Other (please specify): **Gene Therapy, Qualitative Research** |

**How great a risk do you take? A qualitative study exploring attitudes of individuals with Friedreich ataxia towards gene therapy**

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**Background and Aims:** Scientists and pharmaceutical companies are working towards delivering gene therapy for Friedreich ataxia (FRDA). Understanding the views of people with lived experience of FRDA and their parents toward gene therapy is essential to inform trial design and identify potential barriers to participation in clinical trials. The goals of this study were to identify the attitudes towards gene therapy held by individuals with FRDA and parents of individuals with FRDA, and to explore how these may impact future trials for this condition.

**Methods:** Audio-recorded, semi-structured, qualitative interviews with nineteen Australians explored experiences of FRDA, knowledge about clinical trials, views on gene therapy including risks and benefits, and potential barriers to participation in trials.

**Results:** Participants included thirteen individuals living with FRDA aged between 15-43 years, and six parents of children with FRDA aged 4-12 years of age. Thematic analysis of the interviews identified six main themes. Findings from this study indicate there is strong desire for information regarding gene therapy in FRDA however the current level of uncertainty around gene therapy makes decision making challenging. The desire to maintain functional status and avoid additional risk of deterioration from an investigational treatment was apparent.

**Conclusions and Significance/Impact:** People with FRDA and their parents can offer many insights into their preferences around gene therapy clinical trials, which can be used to aid in the design and planning for these trials. Further research is required to identify if attitudes and perceptions differ according to geographical location and disease stage.

(300 words max.)

**Lay Title:** How great a risk do you take? What people with Friedreich ataxia think about gene therapy

(100 characters max.)

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**Lay Summary:** This study aimed to find out what people with Friedreich ataxia and their parents already knew and thought about gene therapy. We interviewed 19 people, and asked about their experiences of FRDA, knowledge about clinical trials, views on gene therapy including risks and benefits, and potential barriers to participation in trials. We found that people want more information about gene therapy, and that current symptoms drive considerations of what they want a therapy to target. We hope to use the results of this study to inform future research to explore how widespread these views are.

(100 words max.)