**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  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  Clinical research  Commercialisation  Computational biology and/or statistics  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  Rare diseases  Physiology  Psychology  Public health  Reproductive biology  Technology  Tele-health  Virology  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.)