PROJECT 0240

A Novel Stem Cell Therapy for Friedreich's Ataxia

A cell and gene therapy strategy with potential relevance to other mitochondrial genetic diseases

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THE OPPORTUNITY

FRDA is a hereditary genetic disease associated with movement and speech problems and reduced life expectancy. This project is developing a potentially disease-modifying autologous stem cell and gene therapy strategy.

1 TECHNOLOGY

Therapeutic frataxin is fused to peptides designed to enable secretion and cellular uptake. A lentiviral vector is used to engineer haematopoietic stem cells for transplantation and evaluation in an FRDA mouse model.

2 APPLICATIONS

- Friedreich's ataxia
- Potential platform relevance to other mitochondrial genetic diseases
- Future clinical translation, subject to successful preclinical studies

3 KEY BENEFITS

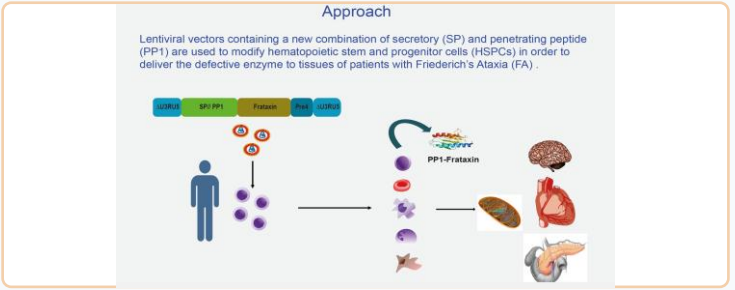
- Uses autologous stem cells, reducing transplant-rejection concerns
- Engineered cells are intended to secrete tissue-penetrating frataxin
- Strategy aims to restore frataxin function in target cells

EVIDENCE & DEVELOPMENT STATUS

- Stage: preclinical investigation in an FRDA mouse model
- Model: YG8XLR mice with approximately 800 GAA repeats
- Next milestone: assess whether engineered stem cells ameliorate disease symptoms

IP & COMMERCIAL ROUTE

- IP position: PCT patent application



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