# 0253 FRDA Drugs

*Small-molecule modulation of sphingolipid metabolism as a potential treatment for Friedreich's ataxia*

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## THE OPPORTUNITY

Friedreich's ataxia is a progressive inherited disorder associated with reduced frataxin, mitochondrial dysfunction and oxidative stress. The project addresses the limited treatment options by targeting dysregulated sphingolipid metabolism with two small-molecule candidates.

## 1 TECHNOLOGY

The treatment strategy rebalances sphingolipid signalling using XY-14, an inhibitor of lipid phosphate phosphatase, and K6PC-5, an activator of sphingosine kinase. The approach is designed to increase pro-survival signalling, frataxin expression and mitochondrial function.

## 2 APPLICATIONS

- Disease-modifying treatment for Friedreich's ataxia
- Treatment of FRDA-associated mitochondrial dysfunction
- Potential combination with antioxidant treatment

## 3 KEY BENEFITS

- Increases frataxin expression in FRDA models
- Improves mitochondrial and oxidative-stress markers
- Small-molecule route compatible with chronic treatment development

## EVIDENCE & DEVELOPMENT STATUS

- Stage: preclinical validation in human cell models and YG8sR FRDA mouse models.
- XY-14 significantly increased frataxin protein expression and improved aconitase activity in mouse cerebellum tissue.
- Both compounds improved selected mitochondrial, antioxidant and iron-regulation markers across FRDA models.
- Next milestone: long-term in vivo efficacy, functional outcomes and translational biomarker validation.

## IP & OWNERSHIP

- IP Protection: US patent application.

## PARTNERSHIP SOUGHT

- Rare-disease drug-development expertise
- Preclinical and translational funding
- Licensing, investment or spin-out collaboration

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