**Cannabinoid therapeutic testing of a Friedreich ataxia mouse model**
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## Scientific abstract

We have recently developed a GAA repeat mutation-based transgenic mouse model of Friedreich ataxia (FRDA). The mice exhibit age-related somatic instability of the GAA repeat, with prominent expansions detected in the cerebellum, accompanied by a corresponding decrease in frataxin expression. The neurological phenotype of these mice includes a progressive coordination defect, as measured by decreased rotarod performance, and vacuolar pathology within large neurons of the dorsal root ganglia.
The cannabinoids ∆-9-tetrahydrocannabinolm (THC) and Cannabidiol (CBD) are potentially therapeutic compounds produced by GW Pharmaceuticals. The antioxidant activity of cannabinoids indicates that they may be effective in preventing and/or treating the development of neurodegenerative disorders. Therefore, the aim of this project will be to test the potential neuroprotective effect of cannabinoids on our FRDA mouse model. Such pre-clinical testing should help the design of future clinical trials using cannabinoids as therapeutic compounds for FRDA patients.



## Lay summary

Medicines that are derived from the cannabis plant are currently being developed by the company GW Pharmaceuticals for the treatment of a number of human diseases. The medicines contain two main compounds, called “cannabinoids”, that can be mixed in different ratios to give potentially different therapeutic effects. Both of these cannabinoids have been shown to protect nerve cells from dying in a number of cell culture and animal model studies, including studies of neurodegenerative diseases similar to Friedreich ataxia (FRDA). However, there is currently no information available on how useful these cannabinoids may be for the treatment of FRDA or other ataxia disorders. This project, which will form the basis of a PhD studentship, aims to address this question by carrying out drug testing on a mouse model of FRDA that we have recently developed, using different cannabinoid preparations provided by GW Pharmaceuticals. The results of this project will determine whether cannabinoids are likely to be a useful treatment for FRDA, enabling the progress into clinical trials either to proceed on a more solid basis, or to be treated with caution.



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