

**Press release – [16 July 2014]**

### **International effort to fill gap in Friedreich's ataxia research**

Three ataxia patient groups joined forces to push research forwards in the progressive neurological condition, Friedreich's ataxia. The charities, FARA, Ataxia UK and GoFAR (respectively from the US, UK and Italy) launched an international call for research proposals to address an identified unmet need. The aim was to develop new non-invasive approaches for evaluating how nerves in the brain and spinal cord are affected in people with Friedreich's ataxia. This is an important step in providing much needed new tools for use in measuring the effect of treatments on the brain and spinal cord in clinical trials.

We are pleased to announce that two research teams have now been awarded grants. Dr Mark Baker and his team at Newcastle University in the UK are planning to test the feasibility of measuring changes in the spinal cord in people with Friedreich's compared to 'control subjects' by using a portable electro-diagnostic screening test. This test was developed by the Newcastle research team and has shown some promise in other neurological conditions. 'We are now excited to explore its use in Friedreich's ataxia, says Dr Baker, 'If successful this could be an easy-to-use tool for trials allowing us to measure the effect that potential disease-modifying drugs could be having on the nervous system in patients. Being painless and non-invasive it would be particularly suitable for children and it is also easy to use making it ideal for trials.' Patients will be recruited via the Ataxia UK accredited Specialist Ataxia Centre in Newcastle, run by Professor Patrick Chinnery, a co-investigator on the project.

In the US, at the University of Minnesota, Drs Christophe Lenglet and Pierre-Gilles Henry are using a different approach to developing sensitive measures of brain and spinal cord degeneration. They plan to use two techniques: diffusion magnetic resonance imaging (MRI) and magnetic resonance spectroscopy (MRS). These techniques provide complementary information on structural and neurochemical changes in the brain and spinal cord. The team already have some data using these techniques from patients with Friedreich's ataxia and they now need to get information over a longer period of time to assess change over a one year period. They also wish to extend this study by including people at an earlier stage to see if any changes measured using these techniques can be seen earlier in the course of the condition. "We hope that these novel neuroimaging techniques will help improve our understanding of the disease, and eventually assess the efficacy of potential treatments in clinical trials." say Drs Lenglet and Henry.

In a joint statement Jen Farmer (FARA), Julie Greenfield (Ataxia UK) and Mina Ruggeri (GoFAR) say 'We are delighted to be working together in this important initiative that we hope will lead to new disease relevant ways of measuring the effect of treatments in Friedreich's ataxia trials. Having consulted with researchers in academia, industry and regulators it became clear that there was a gap that needed some attention. We were pleased with the response the call generated and are grateful to the researchers' constant

efforts in developing new research tools for the Friedreich's ataxia community, taking us one step closer to developing a much needed treatment for this currently incurable condition.'

Ends

### **Notes to Editors:**

#### Information on Friedreich's ataxia

- Friedreich's ataxia is a progressive neurological condition affecting about 1-2 in 50,000 people
- People of any age may be affected, from children to adults. It is an inherited condition and 1 in 85 carry the faulty gene causing the condition but do not have the condition.
- There is currently no cure or treatment to stop the progression of Friedreich's ataxia.
- The most common symptoms are a staggering gait, balance problems, loss of co-ordination, slurred speech, trouble with swallowing, hearing problems and visual impairments. Many people have weakening of the heart, scoliosis and diabetes too. Symptoms may lead to total physical dependency.

#### Information on charities

- Ataxia UK ([www.ataxia.org.uk](http://www.ataxia.org.uk)) is a registered charity providing services and support for people with ataxia, their families, friends and carers. We fund world-class research to develop safe, effective treatments. Ataxia UK Helpline: 0845 644 0606. Contact: Julie Greenfield, [research@ataxia.org.uk](mailto:research@ataxia.org.uk).
- The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. [www.CureFA.org](http://www.CureFA.org) .Contact: Jen Farmer, [info@curefa.org](mailto:info@curefa.org), 484-879-6160
- GoFAR ( [www.fagofar.org](http://www.fagofar.org) ) is a registered (n.7603044400-1, To Italy) non-profit charitable organization dedicate to facilitating and promoting scientific research leading to a cure for Friedreich's ataxia. Contact: Mina Ruggeri, [minagofar@gmail.com](mailto:minagofar@gmail.com).