Ataxia UK partnership with Pfizer & university researchers

As part of our **2020 Vision initiative** to find treatments and cures for one or more of the ataxias by 2020, we are pleased to announce the launch of a significant collaborative drug discovery project between Ataxia UK, three major academic Institutions and **Pfizer** in Friedreich's ataxia (FA). This is the result of our ongoing strategy to actively engage and collaborate with pharmaceutical companies. Over the last two years, Ataxia UK has been engaged in a regular and productive dialogue with members of Pfizer's **Rare Disease Research Unit (RDRU)** to identify the optimal way to collaborate. As Pfizer were new to this disease area, initial discussions were directed towards helping Pfizer better understand the research landscape and in particular the researchers and specialists it would be beneficial to engage with to help shape the development of future therapies.

An FA drug development project launched

In November 2013 Pfizer initiated the Pfizer Rare Disease Consortium: www.rarediseaseconsortium.co.uk

Initially signed with six founding universities (Oxford University, University College London, King's College London, University of Cambridge, Imperial College London and Queen Mary University of London), this agreement was created to facilitate interaction in drug discovery in rare diseases. Ataxia UK therefore saw the opportunity this created to bring together a consortium of UK Friedreich's ataxia experts to work with Pfizer on one such drug discovery programme. Ataxia UK brokered the creation of this consortium consisting of researchers with complementary expertise from **Oxford University**, **University College London** and **Imperial College London**, and supported them in designing a programme of research to propose to Pfizer. This proposal is to develop a new drug or therapy that, if successful, could be tested in clinical trials. We are now delighted to announce that Pfizer has agreed to fund this significant 3-year programme in three universities which involves the following academic researchers:

Dr Paola Giunti (University College London) brings her clinical expertise from running the **London Ataxia Centre** and research studies on the functionality of the frataxin protein in cell and animal models.

Professor Richard Festenstein (Imperial College London) has expertise on frataxin gene regulation and has recently completed a clinical trial of nicotinamide in FA patients with Dr Giunti.

Dr Michele Lufino (Oxford University) has developed methods to study the effect of drugs in cell and animal models of FA and has recently shed new light on the mechanism of frataxin silencing seen in FA.

Over the next three years these scientists will work with Pfizer scientists based both in the US (**Cambridge MA**) and in London, at its new **Genetic Medicine Institute** facility to identify and test new potential therapies designed to address the needs of Friedreich's ataxia patients.

Ataxia UK has had a pivotal role in supporting the development of this research programme. In addition Ataxia UK Trustees have also agreed to provide some financial support to this programme to optimise the effectiveness and maximise its chance of success.

In addition to this consortium grant, it is encouraging that Pfizer have also funded **Dr Wyatt Yue** at the University of Oxford on a separate project on Friedreich's ataxia.

Collaboration is of paramount importance

Pfizer's Head of Rare Disease Alliances (RDRU), **Dr Michael Skynner**, and **Ataxia UK's Research Projects Manager, Julie Greenfield**, spoke at the **Oxford Rare Disease Conference** in May. This was an opportunity to highlight the importance of collaborations in drug discovery, in particular between pharma and patient groups.