TREAT-ARCA project update at the Euro-ataxia Annual Conference 2023

The Euro-ataxia Annual Conference 2023

Euro-ataxia, of which Ataxia UK is an active member, is the federation of 20 European ataxia patient organisations. Every year, Euro-ataxia organises a research conference, to enable ataxia patient groups from across Europe to share their research updates. The 2023 Euro-Ataxia Annual Research Conference was held on the 20th of May, in Greece. The Greek patient organisation (HEFAA) and Euro-ataxia organised the event, with support from Ataxia UK. The event was a great success; over 50 participants attended physically, and over 20 followed the event online. The participants included patient organisation representatives, people with ataxia, researchers, clinicians, and pharmaceutical company representatives.

The research presentations covered a wide range of topics, including improving diagnosis, emerging and existing therapeutics, and improving the quality of clinical trials. Additionally, a panel of people with ataxia described their experiences, and interviews were conducted with patient group representatives, researchers and clinicians, to gain their perspective on important issues facing the ataxia community. The perspective of people with ataxia will help researchers conduct studies that answer the important questions.

At the conference, Dr Hélène Puccio gave a presentation which included an update on the TREAT-ARCA project. The remainder of this blog will explain what the TREAT-ARCA project is and summarise Dr Puccio's presentation. You can find the full conference agenda, alongside slides from some of the other presentations, on the Euro-ataxia website: www.euroataxia.org/research-overview/.

The TREAT-ARCA project

The TREAT-ARCA Project is a global, preclinical research project focussing on developing therapeutic strategies for two rare autosomal recessive cerebellar ataxias (ARSACS and COQ8A-ataxia). The project began in June 2021, and is due to last three years. The project is funded by the EU's Horizon 2020 research and innovation programme.

The TREAT-ARCA research project aims to identify potential treatments for ARSACS and COQ8A-ataxia, which could then be tested in clinical trials. The research could also pave the way for similar work in other recessive ataxias.

In her conference presentation, Dr Puccio explained that the TREAT-ARCA project is a global effort. Dr Puccio leads the project, and is based at the Université de Lyon, France. There are research partners who actively work on the project in Italy (Dr Francesca Maltecca), Germany (Prof Matthis Synofzik) and Canada (Dr Bernard Brais). Additionally, patient advocacy organisations represent people with ataxia at every stage of the research. The organisations involved are Euro-ataxia (with support from Ataxia UK), the German patient group DHAG, and the Ataxia Charlevoix-Sagneunay Foundation (based in Montreal).

Dr Puccio then summarised the objectives of the TREAT-ARCA project, that were originally set out in 2021. The first objective is to test different therapeutic techniques on animal models and cell models of ARSACS and COQ8A-ataxia. Animal and cell

models reproduce certain characteristics of a disease, so that treatments can be tested in the laboratory. The researchers aim to test new drugs, repurposed drugs that have already been approved for other conditions, and gene therapy on such models. Gene therapy involves the deletion of the faulty copy of the gene in cells, and the insertion of a healthy gene copy.

The second objective of the project is to identify and validate biomarkers for ARSACS and COQ8A-ataxia. A biomarker is a measurable indicator of a condition. The development of biomarkers is vital for the success of future clinical trials in people with ARSACS or COQ8A-ataxia. Biomarkers are used to assess how well a condition has responded to treatment.

The progress and future aims of the TREAT-ARCA project

Dr Puccio presented detail on mouse models and cell models that have been developed for ARSACS and COQ8A-ataxia.

The researchers will now validate the different therapeutic strategies in the mouse models. Dr Albre has been identifying potential drugs to test on the models. Dr Puccio and her team have previous experience of gene therapy in mouse models of Friedreich's ataxia. Her team will apply gene therapy to the mouse models of ARSACS and COQ8A-ataxia.

In addition, the research team have a plan to identify biomarkers for ARSACS and COQ8A-ataxia. They will do this by using samples from humans, and the mouse and cell models.

Finally, Dr Bianca Habermann, a specialist in data processing, has started to develop a web-based platform for ataxia research data. This will allow a wide range of ataxia researchers to input their research data into a singular, free platform. The aim is to compile data to answer common questions in ataxia research more effectively.

Want to learn more about the TREAT-ARCA project?

You can read our previous blog posts on the Euro-ataxia website (/www.euroataxia.org/research-overview/). You can also get involved with an educational webinar series that will explain aspects of the TREAT-ARCA project in more detail. Register for the first webinar on gene therapy here: https://us02web.zoom.us/meeting/register/tZcvc-GvqjopHdAU5dOlYGt-p0inBwIXkdU8.

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