

PROSPAX and TREAT-ARCA Annual Meeting July 2022

On 6-8 July 2022, the PROSPAX and TREAT-ARCA consortia held a joint annual meeting in Berlin, Germany. Researchers, clinicians, and patient advocacy organisation representatives from Euro-ataxia and the Ataxia Charlevoix-Saguenay (ARSACS) Foundation attended the meeting.

The PROSPAX project aims to study the progression of Autosomal Recessive Spastic Ataxia of Charlevoix-Saguenay (ARSACS) and SPG7 over time; from the molecular to the clinical level, including brain imaging, markers of progression and animal models. TREAT-ARCA is a preclinical research project focused on two rare ataxias: ARSACS and COQ8A-ataxia. As there is some overlap between the PROSPAX and TREAT-ARCA projects, it was useful to have a joint meeting to share knowledge and ideas.

The PROSPAX project has produced many resources on ARSACS and SPG7, such as data, samples, devices, and protocols. One aim of the meeting was to develop ideas on how these resources can be used for additional projects.

To facilitate this, the PROSPAX resources were split into different categories:

1. **Clinical resources** (hosts: Dr Rebecca Schüle, Dr Ruby Wallis)
2. **Human biosamples** (host: Professor Matthis Synofzik)
3. **Brain imaging** (host: Professor Filippo Santorelli)
4. **Digital motor** (hosts: Professor Bart van de Warrenburg, Dr Andreas Träschütz)
5. **Mouse models** (hosts: Professor Bernard Brais, Dr Francesca Maltecca)
6. **Genetics** (hosts: Professor Nazlı Başak, Professor Stephan Züchner)



As a result of brainstorming sessions on the different subjects, five projects were shortlisted to discuss in more detail the following day. The project ideas developed are now being worked on to further research into the spastic ataxias.

Look out for our future blog posts where we will describe these resources in more detail!

There was a presentation by Dr Ruby Wallis (Euro-

ataxia/Ataxia UK) on the contributions of the patient advocacy organisations working on the PROSPAX project, and discussion on how the lessons learnt from the PROSPAX project could be applied to TREAT-ARCA. Dr Bianca Habermann presented her work to develop a new web tool called ataxiaXplorer to analyse data on the mitochondria (the cell's energy producers), which is of interest for both TREAT-ARCA and PROSPAX.). Data generated on

ataxia can be added and analysed to see which molecular pathways change during treatment with potential therapies.

There were also presentations on the progress that had been made on the different areas of the TREAT-ARCA project. You can learn more about the aims of TREAT-ARCA by reading the overall project summary [here](#). Dr Francesca Maltecca and Dr H el ene Puccio presented their work on testing repurposed drugs (drugs which are already approved for another condition) in mouse models of ARSACS and COQ8A-ataxia. There were also presentations from Dr Julie Schmitt and Dr



Francesca Maltecca on testing new drugs in an

ARSACS mouse model. Dr H el ene Puccio, Dr Francesca Maltecca and Professor Matthis Synofzik showed how they were collecting samples from humans and mice to discover new biomarkers for ARSACS and COQ8A-ataxia. Biomarkers are a way of measuring whether a treatment is working during a clinical trial. When preparing for ataxia clinical trials in the future, it is crucial that neurologists have reliable biomarkers.

Overall, this meeting was a fantastic opportunity for researchers around the world to meet, discuss ideas, and progress this exciting research on ARSACS, SPG7 and CoQ8A-ataxia.

