FIRST CLINICAL TRIALS BEST PRACTICE WORKSHOP

As part of its mission to bring effective therapies to patients in a timely & sustainable way, we have started to expand Europe's current capacity to run clinical trials for potential SMA therapies.

One of the activities undertaken to achieve this goal was the organisation of a workshop to inform neurologists, physiotherapists and research nurses interested in running SMA clinical trials in their countries about clinical trials best practice.

Renowned European experts in SMA clinical trials were recruited to organise and run this workshop:

- Dr. Mariacristina Scoto, Consultant in Neuromuscular Translational Research and Honorary Lecturer at the UCL Great Ormond Street Institute of Child Health in London, UK
- Dr. Giovanni Baranello, Clinical Senior Lecturer in Paediatric Neurology/ Neuromuscular Disorders, UCL Great Ormond Street Institute of Child Health in London, UK
- Professor Laurent Servais, Professor of Paediatric Neuromuscular Diseases, Oxford University, UK and vice-Chair of SMA Europe's Scientific Advisory Board
- Professor Eduardo Tizzano, Director of the Genetica Clínica y Molecular Area of the Hospital Universitari Valle Hebrón, Barcelona, in Spain and a member of SMA Europe's Scientific Advisory Board
- Hinal Patel, Clinical Trials Coordinator at the Great Ormond Street Institute of Child Health in London, UK
- Charlotte Lilien, Senior Research Physiotherapist at Oxford University, UK
- Allyson Gray, Senior Paediatric Research Nurse at the UCL Great Ormond Street Institute of Child Health in London, UK

Left, top to bottom
Charlotte Lilien, Hinal Patel and Mariacristina Scoto
Right, top to bottom
Giovanni Baranello, Eduardo Tizzano, Allyson Gray and Laurent Servais

vanessa.christie-brown@sma-europe.eu | Research Programme Manager

SMA Europe | www.sma-europe.eu

The workshop

This first workshop took place on Tuesday 4th February in Evry, France. Eleven Neurologists and physiotherapists from hospitals in Portugal, Russia, Serbia, Switzerland and Ukraine participated.

In order to take part in the workshop, participants were asked to review a clinical trial protocol on a fictitious gene therapy product and answer some questions pertaining to:

- Any practical or ethical concerns observed in the protocol
- The timeline, manpower & equipment needed and budget to recruit patients
- The adequacy of safety and efficacy assessments.

All preparatory work was reviewed by our experts ahead of the workshop.

The workshop programme consisted of four sessions, each including brief presentations and interactive exercises:

- Setting up a clinical trial, including feedback on the preparatory work
- Patient recruitment
- Endpoints, outcome measures and safety
- Audit, monitoring, protocol deviation and patient retention.

vanessa.christie-brown@sma-europe.eu | Research Programme Manager

SMA Europe | www.sma-europe.eu

The day ended with patient advocates feeding in the patient's perspective and with members of the pharmaceutical companies with programmes for SMA (AveXis, Biogen, Cytokinetics, Roche and Scholar Rock), who came to find out about the European arm of this initiative.

This workshop was extremely well reviewed by all stakeholders. We are now working on our next endeavours.

Participants from Portugal, Russia, Serbia, Switzerland and the Ukraine, together with the trainers and SMA Europe's Clinical Trials Project Team.

vanessa.christie-brown@sma-europe.eu | Research Programme Manager

SMA Europe | www.sma-europe.eu