

Course objective

The SMA Basecamp course is the newest of our educational offerings and has been developed to educate paediatricians, paediatric neurologists and general neurologist trainees earlier in their careers to cultivate an interest in spinal muscular atrophy (SMA). It will also support consultant paediatricians and neurologists who would like to develop their expertise in SMA.

We hope that exposing healthcare professionals (HCPs) to modern SMA management earlier, more clinicians will be confident in the management of infants, children and adults with SMA or may be motivated to specialise in the condition. There is limited understanding around SMA at present and we hope that, by providing a dedicated training and support programme, we can encourage an interest in specialisation for some and improved understanding for all.

Pre reads

Background reading and preparation – 6 hours (trainees to do at home)

1. International standards of care for SMA
2. landmark papers on drug therapies for SMA (nusinersen, gene therapy, risdiplam) and / or review papers of new drug therapies in SMA
3. respiratory management in SMA care papers
4. physiotherapy management and changes to phenotype with patients on drug treatment of SMA, including spines

Day 1: Tuesday, 5 July 2022

13:00 Registration & lunch

14:00 Introduction, who are you and welcome

Dr Min Ong, Paediatric Neurology Consultant, Sheffield Children's Hospital NHS Foundation Trust

14:45 Scene setting & basic science

Dr Vasantha Gowda, Paediatric Neurology Consultant, Guy's and St Thomas' NHS Foundation Trust

- What information from the consultation would be helpful to the diagnosis (history, examination)?
- What investigations would you perform and why
- If the diagnosis of SMA is already secured but family not informed what discussions should ensue and how will you prepare for this
- What disease modifying treatments are available and how will you discuss this with the family?
- What other aspects of management (non-drugs) need to be in place?
- Different SMA types

15:45 Refreshment break

16:15 Care pathways for SMA patients - paediatric & adult

Dr Vasantha Gowda

16:45 Paediatric & adult drug therapies for SMA

Dr Min Ong, Dr Vasantha Gowda & Dr Channa Hewamadduma, Consultant Neurologist and Honorary Senior Lecturer Sheffield Teaching Hospitals NHS Foundation Trust

17:45 Cases from paediatric & adult care - drug therapies in practice

Dr Min Ong, Dr Vasantha Gowda & Dr Channa Hewamadduma

18:15 Meeting close

Dr Min Ong

19:15 Dinner and networking

21:00 Close

Day 2: Wednesday, 6 July 2022

08:30 Registration

08:45 Welcome

Dr Min Ong

09:00 Treatment Options - case based discussions

Dr Min Ong & Dr Channa Hewamadduma

10:30 Refreshment break (final check out)

10:45 Adults with SMA

Dr Channa Hewamadduma, Neuromuscular Physician, Sheffield Teaching Hospitals NHS Foundation Trust

- Development of a pathway for adult SMA patients
- Accessing novel therapies in adult clinic
- Real world data on novel therapies in SMA: adults

11:45 Respiratory management in SMA

Lisa Edel, Senior Specialist Paediatric Respiratory Neuromuscular Physiotherapist, Great Ormond Street Hospital for Children NHS Foundation Trust

12:30 Lunch

13:15 Care management in SMA

Robert Muni Lofra, Consultant Physiotherapist, Newcastle University Translational and Clinical Research Institute

- Key role in SMA care and outcomes

13:35 Orthopaedic management in SMA

Mr Ed Bayley, Consultant Spinal Surgeon, Sheffield Children's NHS Foundation Trust

- Approaches to spinal management Paediatric & Adult

13:50 Hip management in SMA

Mr John Cashman, Consultant Paediatric Orthopaedic Surgeon, Sheffield Children's NHS Foundation Trust

- Approaches to hip management

14:05 Speech and language

Nicky Sedgwick, Speech And Language Therapist, Sheffield Children's NHS Foundation Trust

- therapist input into patients with SMA

14:20 Refreshment break

14:45 Growth and nutritional management, metabolic abnormalities

Dr Giovanni Barranello, Clinical Associate Professor in Paediatric Neurology/ Neuromuscular Disorders, University College London Great Ormond Street Institute of Child Health & Great Ormond Street Hospital NHS Foundation Trust

16:30 Depart