

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, 23 November 2021

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

14:30 Scene setting & basic science. Case: 8 month old infant has been referred to neurology for floppiness and impaired motor development

Dr Min Ong & 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?

16:15 Refreshment break

16:40 Different SMA types

Dr Min Ong & Dr Channa Hewamadduma

- How will different SMA types present
- Will SMA type change your approach to discussion of the diagnosis, disease modifying treatments and other management aspect of the patient?

18:00 Meeting close

Dr Min Ong

19:15 Dinner and networking

21:00 Close

Day 2: Wednesday, 24 November 2021

08:30 Registration

08:45 Welcome

Dr Min Ong

09:00 Service / pathway for SMA patients (considerations, education of different groups/professionals) e.g. respi / ortho / local therapists / genetics / ICU / hospice / GPs

Dr Vasantha Gowda & Dr Channa Hewamadduma

10:30 Refreshment break (final check out)

11:00 Respiratory management in SMA

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

11:45 Orthopaedic management in SMA (management / discussions)

Dr Ed Bayley, Consultant Spinal Surgeon, Sheffield Children's NHS Foundation Trust,
Kay White, Paediatric Neuromuscular Physiotherapist, Sheffield Children's NHS Foundation
Trust & Robert Muni Lofra, Consultant Physiotherapist, Newcastle University Translational
and Clinical Research Institute

- How medication affects scoliosis presentation, and how scoliosis impacts medication use
- Available options to reduce complication from scoliosis development
- Scoliosis surgery in SMA

12:45 Lunch

13:30 Adults with SMA

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

14:30 Refreshment break

14:45 Metabolism, nutrition in SMA Amino acid diet, hypoglycemia, growth

Dr Giovanni Barranello, Clinical Associate Professor in Paediatric Neurology/ Neuromuscular
Disorders, University College London Hospitals NHS Foundation Trust

16:30 Quiz

Dr Min Ong

16:45 Depart – coach leaves for station