

### 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, Consultant Neurologist and Honorary Senior Lecturer Sheffield Teaching Hospitals NHS Foundation Trust

- 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



## SMA Basecamp 1

23 & 24 November 2021, Halifax Hall, Sheffield

**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 Depart – coach leaves for station**



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