



UCL INSTITUTE OF CHILD HEALTH

Great Ormond Street  
Hospital for Children

NHS Foundation Trust

**INFORMATION SHEET FOR PARTICIPANTS AGED 16-18+ YEARS****Chief Investigator: Professor Francesco Muntoni****Recording information on the management of your Spinal Muscular  
Atrophy in the UK – SMA REACH UK Database  
in association with the Neuromuscular Clinical UK Network**

You require the expert care of medical and therapy teams for the long-term management of your Spinal Muscular Atrophy. We would like to collect and record information, which will help us to deliver the best care to all children with SMA in the UK.

This leaflet explains why we are asking your permission to record clinical information into a database; the SMA REACH UK Database.

**What is the SMA REACH UK Network?**

The SMA REACH UK Network is a national and international partnership between doctors and therapists involved in the care of children with Spinal Muscular Atrophy. This Network is supported by the SMA Trust.

**What is SMA REACH UK Database?**

The SMA REACH UK Database is an internet based system which can save information about your diagnosis, assessment and management of SMA. Into this new database we aim to put clinical and genetic data from two existing databases (SMARTnet and the SMA registry). The data collected would be jointly managed by the Dubowitz Neuromuscular Centre and MRC Neuromuscular Centres in London and Newcastle.

**Why have I been invited to take part?**

You have been invited to take part in this research study because you have SMA and we would like to study how your condition changes over time. All children with SMA who attend clinics in Great Ormond Street Hospital in London and in Newcastle will be invited to take part.

**Do I have to take part?**

No, it is entirely up to you to decide. If you do decide to take part, your doctor or physio will ask you to sign a consent form. By signing the form you are agreeing to take part in the study. You are free to stop taking part at any time during the research without giving a reason. If you decide to stop, this will not affect the care you receive in any way.

### What will happen to me if I take part? What will I be asked to do?

We would like to collect and save information each time you are assessed in clinic. You will be asked to come to hospital every 6 months as you do for your routine clinic appointments. The only difference will be that some of your physiotherapy assessments may be a little longer. The study will last for 2 years but may be extended in the future.



### What information will we collect?

We would like to record:

- Your NHS number
- Name and date of birth
- General information about your condition for example your age at diagnosis, any results of gene testing, and problems resulting from SMA  
Results of muscle, heart, breathing, growth and general health testing from medical assessments
- Some extra physiotherapy assessment measures.

We will also ask you for your permission to videotape/take photos of you while the physical assessments are carried out. The photos will be used to make the instructions that physiotherapists use to do your physical assessment. The videos will be used to help teach other physiotherapists to do the correct assessments for SMA. They will also be used to test that the physiotherapists are scoring properly - these are known as reliability studies. Some of the results from the reliability studies may form part of a MSc project.



You can still be registered on the database if you do not wish to be videotaped/photographed.

### Why are we collecting this information?

We will use the information we collect to help us:

- Collect accurate details about SMA and how it changes over time
- Monitor medical and therapy care to make sure it is always up to date.
- Plan and develop services for better management of SMA
- Try out new assessment tools with the aim to develop more sensitive SMA specific scales.
- Undertake reviews and produce reports that will improve our knowledge of the natural history of SMA
- Improve and monitor the standards of care
- Start to prepare for clinical trials.
- Compare information on SMA from this database with data from other international sites



### Who collects the information?

The hospital staff at the clinic will collect this information. This will usually be your doctor, physiotherapist or nurse or may be one of the designated research team: a doctor, physiotherapist or study coordinator. A designated database manager may also help with recording information.

### **When and how will you collect the information?**

The information will be collected and updated at every clinic visit. We will collect the information from the medical and therapy records.

We will also invite you and your parents/carer to be involved in one or two group sessions in the coming months. These sessions will allow you, your parents/carer, researchers and doctors to discuss the most useful assessment tools for families.

### **Who will see the information?**

All information will be stored on a secure system and password protected. Only the NHS staff that care for you will see your details. There are strict regulations controlling who has access to personal information like your name, date of birth or NHS number. By law, everyone who works for the NHS must keep all personal information confidential and the trust has strict confidentiality and security procedures in line with the data protection act (1998). Only anonymised data will be shared with other institutions (SMA Registry, MRC database) and international centres.

### **What is the consent procedure?**

If you are happy for your details to be stored on the database and used for clinical care and research purposes please give your permission on the consent form. A signed copy of your consent form and a copy of this information sheet will be given to you for your information.

### **Can I see the records on the database?**

**Yes**, you can get a copy of the information we have about you. To do this, please ask the doctor in charge.

### **Are there any benefits or disadvantages?**

You may not directly benefit from the database system; however it might help to improve the standards of care for SMA in clinics in the UK and may benefit children with SMA in the future. This research could also help to prepare for and design clinical trials for SMA in the future.

### **What if there is a problem?**

You may contact one of the study team by email or telephone using the contact details at the end of this leaflet. If you are not happy about your treatment and you wish to complain, you should contact the PALS service at Great Ormond

Street By phone: 020 7829 7862 (direct line) or by email: [pals@gosh.nhs.uk](mailto:pals@gosh.nhs.uk) so that they can advise you about the steps to take as well as being able to give you the contact details for the appropriate people in the hospital.

### Who is organising and funding the research?

This study is funded by the SMA Trust.

### Who has reviewed the study?

Before any research is allowed to happen, all research in the NHS is looked at by an independent group of people, called a Research Ethics Committee, to protect your interests. This study has been reviewed and given favorable opinion by the London- Bromley Research Ethics Committee.

### How can I find out more about it?

Please talk to the doctor in clinic if you:

- Need more information
- Have any questions or concerns



Or contact one of the study team by email or telephone:

Dr. Mariacristina Scoto: [m.scoto@ucl.ac.uk](mailto:m.scoto@ucl.ac.uk)

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Or visit our website [www.SMAREACHUK.com](http://www.SMAREACHUK.com)