# A picture containing text  Description automatically generatedINFORMATION SHEET FOR CHILDREN (11-15 YEARS)

# Principle Investigator:

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

Explanation (11-15 years old), Why are we doing this research?

You are receiving 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 improve and deliver the best care for all children with Spinal Muscular Atrophy in the UK.

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

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

The SMA REACH UK database is a way that we can save all the information that is collected about your SMA in one place. The data collected would be jointly looked after by the Dubowitz Neuromuscular Centre and MRC Neuromuscular Centres in London and Newcastle.

What is the SMA REACH UK Network?

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

Furthermore, The SMA REACH Network is part of an international SMA Consortium (ISMAC) in partnership with two prestigious Networks: the PNCRN in the United States and the Italian SMA Network. We will ask you to give permission for fully anonymised information (which means all your personal details will be taken out) and collected to be shared with an international platform. This can then be accessed by Biogen and be shared with third parties (pharmaceuticals and academic institutes) in a strictly anonymised form, to look at SMA changes over time and the effect of different SMA specific drug treatments. This information will only be collected upon patient consent. If patients choose not to share data as part of this collaboration (or are participating in clinical trials), then they can still be a part of SMA-REACH and data will not be shared with third parties.

**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 neuromuscular clinics in the UK will be invited to take part in this study. Furthermore, all other UK sites open to SMA-REACH will have the opportunity to invite children with SMA to participate in the study.

**Do I have to take part?**

No, it is entirely up to you to decide if you want take part. If you do decide to take part, your doctor or physio will ask you to sign a form called an assent form and your parents/guardians will need 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?**

You will be asked to come to hospital every 6 months as you do for your normal 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. At some point you might be asked to do an interview to give us your point of view about how management and time may have affected you.

**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 and problems resulting from SMA
* Your medical history and additional medical information will be recorded as part of the initiative with ISMAC (only applicable for patients who consent to take part in this initiative)
* Results of muscle, heart, breathing, growth and general health testing from medical assessments
* Some additional physiotherapy assessment measures including some as part of the initiative with ISMAC (upon consent).
* If you consent, we may contact you by phone for short interviews/surveys on SMA (only at GOSH)

We will also ask you for your permission to videotape/take photos you while the physical assessments are carried out. This will allow another physiotherapist to view the recordings. You can still be registered on the database if you do not wish to be videotaped.

**Why are we collecting this information?**

We will use the information we collect to help us:

* Collect accurate details about SMA
* 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 and develop new SMA assessment tools
* Create reviews/audits and reports that will improve what we know about SMA and the current standards of care
* Compare information with data from other international sites
* Prepare for clinical trials

**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.

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

Information will be collected from the medical and therapy records and updated at every clinic visit. We will also invite you and your parents/carer to attend one or two group sessions in the coming months. This will allow you, your parents/carer and researchers and doctors to discuss the most useful assessment tools for families.

**Who will see the information?**

Only the NHS staff who care for you will see all the details. There are strict regulations controlling 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 GDPR. Only anonymised data will be shared with other institutions (SMA Registry, MRC database, ISMAC IT Platform).

**What is the consent procedure?**

If you are happy to be a part of this study you will be asked to sign an assent form. You will be given a copy of this information sheet and an assent form to keep. Please talk to your doctor if you would like to withdraw your consent for providing data to SMA Reach.

**Additional information for those enrolling onto the nusinersen (SPINRAZA®) Managed Access Agreement (MAA)**

If you/your child are enrolling onto the nusinersen (SPINRAZA®) MAA then in addition to providing consent for the SMA REACH database you will also need to sign up to the managed access patient agreement. Your doctor will provide you with this.

People enrolled in the MAA will have their personal pseudo-anonymised data from the SMA REACH database shared with the following institutions:

* NHS England- To monitor patients treatment start and stop criteria as per the terms of the managed access agreement. To monitor case ascertainment in the SMA REACH database.
* The National Institute of Health and Care Excellence- To ensure compliance with the data collection terms of the managed access agreement i.e. to monitor data completeness of mandatory data fields. To monitor case ascertainment in the SMA REACH database.
* University of Strathclyde- To allow matching of clinical and PROMS data.
* Biogen (the company that makes SPINRAZA®)- To enable the company to analyse the clinical and cost effectiveness of the technology and present a submission of the evidence to NICE for a health technology appraisal.

This is not an exhaustive list and it may be necessary during the course of the MAA to share your/your child’s data with other institutions but your/your child’s data will always be pseudo-anonymised before it is shared.

Pseudonymised personal data means replacing characteristics of personal data with a pseudonym, a value that does not allow the person to be directly identified without the use of additional information, provided that (a) such additional information is kept separately, and (b) it is subject to technical and organisational measures to ensure that the personal data cannot be attributed to an identified or identifiable individual.

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

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

**Are there any benefits or disadvantages to taking part?**

There are no direct benefits to you for taking part but we expect that the research will help to improve the standards of care for SMA, and may also benefit children with SMA in the future.

**Who is organising and funding the research?**

This study is funded by a charity called the SMA UK and MDUK.

**Who has reviewed the study?**

All research in the NHS is looked at by a 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 the study?**

Please talk to the doctor in clinic if you:

* Would like more information
* Have any questions or concerns
* Visit our website <http://www.smareachuk.org/>
* <insert site details>

Thank you for taking the time to read this information sheet