

# UK SMA Patient Registry

## Who should register?

Adults and children with SMA and a confirmed mutation in the SMN1 gene who live in the UK or Ireland.

SMA is a rare condition, every single person counts!

## How do I register?

You can register online and create an account so that you can view and update your information at any time. If a person is under 16, a parent or guardian must create an account on their behalf. Register online at:

[www.treat-nmd.org.uk/registry](http://www.treat-nmd.org.uk/registry)

If you cannot register online, contact us at the address on the back and we will send you a registration form.

Registration is voluntary and at all times the information remains your property. You have the right to withdraw your information at any time.

## For further information about Patient Registries and the TREAT-NMD Network:

### UK SMA Patient Registry

John Walton Muscular Dystrophy Research Centre,  
Newcastle University, International Centre for Life  
Newcastle upon Tyne, NE1 3BZ

+44 (0)191 241 8640

[registries@newcastle.ac.uk](mailto:registries@newcastle.ac.uk)

[www.treat-nmd.org.uk/registry](http://www.treat-nmd.org.uk/registry)

[www.treat-nmd.org](http://www.treat-nmd.org)

[www.treat-nmd.org/patient-registries/](http://www.treat-nmd.org/patient-registries/)

### Registry principal investigator:

Dr Chiara Marini-Bettolo



For further information about spinal muscular atrophy, support and services:

### Spinal Muscular Atrophy UK

40 Cygnet Court, Timothy's Bridge Road Stratford upon Avon, Warwickshire, CV37 9NW

01789 267 520

[office@smauk.org.uk](mailto:office@smauk.org.uk)

[www.smauk.org.uk](http://www.smauk.org.uk)



For individuals living with Spinal Muscular Atrophy and resident of the United Kingdom or Ireland



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## What is a patient registry for SMA?

A database of genetic and clinical information (data) about people affected by SMA.

## How is the information used?

Promising new treatments for SMA are becoming available. More treatments are in development and need to be tested in clinical trials. However, SMA is a rare condition and it can therefore sometimes take years to find enough patients for a clinical trial. The Patient Registry can help speed this up.

The Registry also helps specialists gain more knowledge about the condition and the number of people affected by SMA. In addition, it helps capture information from individuals now receiving treatment. This information helps to develop and improve worldwide standards of care for people with SMA.

## What information is collected?

- Personal details, such as name, address, date of birth and gender, so that individuals can be identified and contacted.
- Clinical and genetic information to inform researchers in their development of treatments, to aid recruitment to clinical trials and to assist access to new treatments.



## What other benefits are there?

If you register, you will be told about clinical trials and other studies you may wish to join, but you are not obliged to do so.

You will receive information relevant to SMA about latest research developments and about TREAT-NMD activities.

Collected information will help improve standards of care for people with SMA and will help inform on the impact of new therapies.

## Is my data safe?

All the information is stored in a secure server which is protected in a similar way to online bank accounts. Only specially appointed registry staff can look at your information.

The UK SMA Patient Registry is part of the TREAT-NMD Global SMA Registry, which collects medical information from national SMA registries worldwide. Only anonymised data is shared with the TREAT-NMD Global SMA Registry. You can be sure that your contact details are safe.

The UK SMA Patient Registry also collaborates with SMA REACH UK, a research database that collects longitudinal clinical data from children with SMA. SMA REACH UK collects patient data entered by doctors and is operated by Great Ormond Street Hospital and the MRC Neuromuscular Centres in London, Newcastle and other neuromuscular centres across the country. This compliments data provided by patients themselves in the UK SMA Patient Registry. Consent for the exchange of limited and specific patient data between the registry and database is requested at registration.

## How is Spinal Muscular Atrophy UK involved with the UK SMA Patient Registry?

Spinal Muscular Atrophy UK funds the UK SMA Patient Registry. In doing so, it is supporting clinical research and ensuring all people with SMA in the UK and in Ireland can be informed about research, clinical trials and best-practice care.

Information you provide to the UK SMA Patient Registry is not shared with Spinal Muscular Atrophy UK.