Is There a Cure for SMA?

There is no cure for SMA, but since 2016 drug treatments have gradually been introduced worldwide and, in the UK, there are now NHS-funded drug treatments for eligible children and adults; see our website for the latest information. Any drug treatments must still be combined with the best supportive care and management of symptoms for each individual.

Who are SMA UK?

Established for over 35 years, we're a charity providing accurate information and a wide range of support services, while working to improve access to the best care, services and drug treatments today and funding research projects that can change tomorrow.

Our services are UK-wide and free. We don't provide medical services/advice, which must come from your medical team.

How You Can Help Us

We don't receive government funding and rely on the generosity of our supporters to maintain our services. If you can help us, please go to:

smauk.org.uk/donate

How to contact us

Spinal Muscular Atrophy UK
Unit 9, Shottery Brook Office Park,
Timothy's Bridge Road
Stratford-upon-Avon, CV37 9NR

Phone: 01789 267520

- Mon – Thurs (8.30am – 4pm)
- Friday (8.30am –1.00pm)
- Closed on public holidays.

Email: office@smauk.org.uk

Sign up for mailings:

smauk.org.uk/sign-up-for-mailings

www.smauk.org.uk

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Registered in England and Wales

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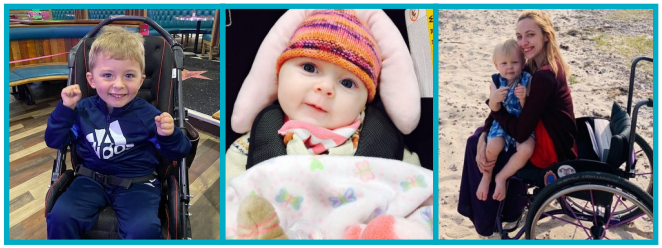
About Spinal Muscular Atrophy

Key Information

spinal muscular atrophy uk
Help for today, hope for tomorrow

What is Spinal Muscular Atrophy?

5q Spinal Muscular Atrophy (SMA) is a rare, genetic neuromuscular condition. It causes progressive muscle wasting (atrophy) and weakness leading to loss of movement. This may affect crawling and walking ability, arm, hand, head and neck movement, breathing and swallowing.



5q SMA is passed from parents to their children through a 'faulty' version of the Survival Motor Neuron 1 or *SMN1* gene.

- Approximately 1 in 40 of us carry this 'faulty' gene – that's around 1.7 million carriers in the UK. Carriers do not have SMA.
- If two carriers of the 'faulty' gene have a baby, there is a 1 in 4 chance their baby will have SMA.

How many people have SMA?

Studies suggest that worldwide, approximately:

- one in every 10,000 babies born have a Type of SMA.
- between 1 and 2 children, young people and adults in every 100,000 have a Type of SMA.

Types of 5q SMA

5q SMA is grouped into clinical 'Types' 1, 2, 3 & 4, based broadly on the age symptoms begin and the physical 'milestones' a person would be expected to achieve if their SMA followed its 'natural history' without drug treatments or interventions for breathing difficulties.

5q SMA—Childhood Onset Types 1, 2 and 3

SMA Type	Age symptoms usually begin
Type 1 (most severe)	0—6 months
Type 2	7—18 month
Type 3	18 months—18 years

5q SMA is often described as a **spectrum**. The severity of the condition varies from person to person, both within and between 'Types' - each child and adult is affected differently.

How anyone responds to treatment is also very individual. What the outcomes are, or will be, for each individual will vary, depending on factors such as how early treatment was started and how severe the impact of SMA was at the time.

Many children, young people and adults now living with SMA ,though doing so much better than they would ever have done in the past, are still impacted by varying degrees of muscle weakness. Some have breathing and swallowing difficulties. Many rely on powered wheelchairs to get around, may have difficulties keeping up with daily activities and need specialist equipment, plus significant care and support during both childhood and adult years.

5q SMA - Adult Onset Type 4

Symptoms begin in adulthood and include mild to moderate muscle weakness in the arms and legs, and some difficulty walking. NHS treatments are not currently available.

Rarer forms of SMA

There are other rarer forms of SMA which have different genetic causes. They include: Adult Onset SMA, Distal SMA, Kennedy's and SMA with Respiratory Distress1 (SMARD1).

For more information about SMA, visit: smauk.org.uk/information