What is Spinal Muscular Atrophy (SMA)?

SMA is a genetically inherited neuromuscular condition which can cause irreversible loss of children's ability to crawl, walk, breathe and swallow.

SMA Type 1 – children are unable to sit without support or roll over. Without intervention for breathing difficulties, most live for less than two years.

SMA Type 2 – children always rely on wheelchairs for independent mobility, need significant support with many daily activities and are vulnerable to chest infections. The majority live long lives.

SMA Type 3 – children walk, but as they get older many need wheelchairs for independent mobility and increasing support with daily activities. Their life expectancy is normal.

What is Spinraza™ (nusinersen)?

Manufactured by Biogen, this is the first possible treatment for those with SMA Types 1, 2 and 3. In early clinical trials, significant numbers of individuals treated with the drug showed improvement, including:

- achieving physical milestones that they would not have reached without treatment
- maintaining physical milestones that they would not have maintained without treatment
- surviving longer than expected considering the typical course of their condition

Spinraza is given by lumbar puncture (intrathecal injection) – four injections in the first two months then once every four months for life.

Clinical trials for children with SMA Type 1 were ended early due to sufficiently positive results. In autumn 2016, Biogen opened its global 'compassionate use' Expanded Access Programme (EAP) – committing to continue to provide the drug free unless the family and their clinician consider it appropriate to stop.

Time is running out

Gemma, age 41, SMA Type 3

Finley, age 5, SMA Type 2

Haris, 6 months, SMA Type 1

Lily, age 12, SMA Type 2

Access achieved for all in Scotland

7th May 2018: Scotland agrees to fund the treatment for those with SMA Type 1.

13th February 2019: Scotland announces that, through its new ultra-orphan pathway, Spinraza will be routinely available for people with SMA Types 2 and 3 from April 2019, subject to successful sign-off.

Still no access in England, Wales and Northern Ireland

Spring 2017: Biogen's EAP starts to roll out in the UK.

1st June 2017: European Medicines Agency (EMA) licence granted.

18th January 2018: NICE announces pathway will be a Single Technology Appraisal **(STA)** – used for common diseases – **not** a Highly Specialised Technology (HST) evaluation – used for rare conditions – as NICE assesses it doesn't meet the HST criteria.

14th August 2018: NICE announces it does **not** recommend funding by the NHS and releases consultation paper. NICE states Spinraza's:

- **Clinical effectiveness is not yet proven** – but NICE indicates this would not block possibility of a three- to five-year Managed Access Agreement (MAA) between Biogen, NICE and NHS England (NHSE)
- **The price is too high**.

October 23rd 2018: NICE's second committee meeting reviews all responses to its consultation.

1st November 2018: SMA community devastated as Biogen closes the EAP for SMA Type 1 to all new infants. More than 80 children remain on the programme, **but since this time infants have been diagnosed with SMA Type 1 and cannot access this life changing treatment.**

6th March 2019: NICE's third committee meeting will take place.

PLEASE TAKE ACTION – SEND LETTERS TODAY TO NICE, BIOGEN AND NHS ENGLAND

Dawn, age 55, SMA Type 3

George, age 8, SMA Type 2

Andi, age 45, SMA Type 2

Chris, age 22, SMA Type 3

Matilda, age 4, SMA Type 3