Spinal Muscular Atrophy Support UK
Help for today • Hope for tomorrow

Cabinet Secretary for Health and Sport
The Scottish Government
St. Andrew's House
Regent Road
Edinburgh
EH1 3DG

8th May 2018

Dear Ms Robison,

Re: Access to spinal muscular atrophy treatment called Spinraza for all types of spinal muscular atrophy

I am writing to you regarding access to Spinraza, the first and only treatment for 5q spinal muscular atrophy (SMA).

5q SMA is a rare inherited neuromuscular condition which includes childhood onset Types 1, 2 and 3. It causes progressive muscular weakness and loss of movement due to muscle wasting. This may affect crawling and walking ability, arm, hand, head and neck movement, breathing and swallowing. Without intervention, life expectancy for infants with SMA Type 1, is less than two years. Children with SMA Type 2 are never able to stand or walk, and live with a very complex and challenging condition that has a huge impact on both them and their families. Over time, those with SMA Type 3 also experience increasing difficulties with walking and standing. We estimate there are 110 children, young people and adults living with 5q SMA in Scotland and up to 1,300 in the UK overall.

On 31st December 2016, Spinraza was recognised as having the potential to change the lives of all those with 5qSMA and was licensed in the USA. On 1st June 2017, it was granted a licence by the European Medicines Agency. It has now been approved for treatment of those with all types of 5qSMA in many other countries in Europe and the rest of the world.

You can imagine the high hopes of the SMA Community in Scotland when we heard that Spinraza was to be considered for provision here. Imagine now how we feel having heard the news on May 7th that the Scottish Medicines Consortium (SMC) has approved it for use on the NHS in Scotland, but that this is **only** for children with SMA Type 1. Whilst we recognise this is a positive step forward and a lifeline for these children and families, it is hugely disappointing that children, young people and adults with Type 2 and Type 3, who wish to access and could potentially benefit from this treatment, still don't have this opportunity. We, along with SMA charities, have been calling for equitable access for those with all these types of SMA and this was made very clear in all the charities' patient submissions and representations to the SMC.

There is, though, a way forward. In December 2016, the Montgomery Review on Access to New Medicines recommended new appraisal processes for ultra-rare disease treatments. SMA is designated an ultra-rare disease which would mean that Spinraza would therefore have a much greater chance of approval for **all** childhood onset types of 5qSMA under such a system.

I understand that these recommendations have been accepted by the Scottish Government and are due to be implemented in Spring 2018. It is now May.

I would be very grateful if you would do everything in your power to ensure the implementation of the Montgomery recommendations as a matter of urgency so that Spinraza can be reassessed as a matter of priority for those with SMA Types 2 and 3 under the new appraisal process.

I would also be very keen for you to show your support more generally by finding out more about the urgency of access to new treatments for people with muscle-wasting conditions and other rare diseases and discuss how to remove the barriers to this by attending:

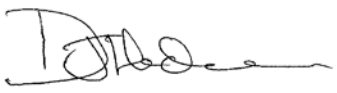
Muscular Dystrophy UK's Fast Track to treatments MSP drop-in event: Wednesday 30th May, 6pm to 8pm, Fleming Room (Committee Room 3), hosted by Jackie Baillie MSP.

I will be attending this meeting and hopefully we can meet there to discuss this further.

For more information and any queries about this event, please contact Jonathan Kingsley at Muscular Dystrophy UK at j.kingsley@musculardystrophyuk.org or call 020 7803 4839.

Thank you for your support and I look forward to hearing from you shortly.

Yours sincerely,



Doug Henderson
Managing Director
Spinal Muscular Atrophy Support UK