

14th June 2019

To NICE and NHS England

Re: NICE /NHSE communications re: access to nusinersen treatment and the impact on the SMA Community

Having participated in the NICE/NHSE meeting with the Patient Advisory Groups (PAGs) on 30th May, we are writing to you with our observations on the communications there have been in the last few weeks of the decisions by NICE / NHSE with regard to the provision of nusinersen by the NHS. Throughout the process we have made it clear how important it is to carefully manage communications with the SMA community who have been desperately waiting for news about access to this treatment. To be clear, this is not a formal appeal, but we believe we have a responsibility to highlight the detrimental impact this has had on the SMA community we represent and support and ask you to give our comments due consideration.

The First Communication

On 15th May NHS England made a public announcement via a press release. As patient groups we were given less than 24 hours' notice of this statement and no clarity of the detail of the agreement despite our repeated cautions that it was vital that the community was updated sensitively given the hopes that have been pinned on this treatment. The update state that:

'For older babies, children and young adults with less severe symptoms (SMA types 2 and 3), the NHS will begin to provide nusinersen shortly after NICE's guidance is published, once the services to deliver them are established. This is in line with the approach for making other brand new treatments available in a fair way for patients across the country, and is not expected to take more than a few weeks. This is one of the most comprehensive deals in the world, meaning all relevant SMA patients (SMA 1, 2, 3a and 3b), including adults and siblings who are yet to show symptoms, will be able to benefit from this treatment.'

The SMA Community was elated. Many families were in tears having waited so long for access and were asking 'Is this true?' A further press statement from Biogen's Head Office was released stating

'The positive recommendation is for the treatment of infants, children and adults with 5q spinal muscular atrophy (SMA), including pre-symptomatic and symptomatic SMA Types 1, 2 and 3'

Another community update came from Biogen UK stated:

'An agreement has been reached to recommend the funding of nusinersen on the NHS for the treatment of infants, children and adults with 5q spinal muscular atrophy (SMA), including presymptomatic and SMA types I, II and III. This decision is a momentous occasion for SMA patients, their families, and the patient organisations who support them, who have fought tirelessly for access to this treatment. It has been achieved through intensive and collaborative working between the SMA community, the National Institute for Health and Care Excellence



(NICE), NHS England, and Biogen. Everyone involved should be applauded for their commitment to achieving a resolution'

There was no further communication from NHSE / NICE to suggest that there would be significant limitation of access, except the use of the word '**relevant**' SMA patients. Suffice to say that, at the time, these communications were taken at face value and so, for the first time, many families told their children that they would be able to receive a new treatment. Many were still careful not to raise expectations of outcomes. Many adults were both amazed and delighted to at last have this outcome.

The Second Communication and our Meeting with NICE/NHSE

On 24th May we received confidential copies of NICE's final guidance and the draft Managed Access Agreement.

On 30th May 2019 we attended a meeting with you when we were given an opportunity to give you feedback on what we, at that stage, believed was a **draft** Managed Access Agreement (MAA), given it was stamped as such. We advised you of the points that were of great concern which we will not repeat here as we have clearly documented these.

At this meeting, we urged NICE/NHSE not to publish the NICE guidance and draft MAA on June 3rd as was planned without first establishing if there was a possibility of the MAA being amended in favour of addressing these concerns. We also asked for a lay document to be published at the same time as any release to answer the most critical of the many questions that would arise. We had already submitted lists of these concerns and questions but, following your request, consolidated the Q and As into a single document and emailed them to you the next day. We also made strenuous efforts to assist with wording possible amendments to the MAA, not only to address the criteria but also to ensure greater clarity of meaning.

We received an email on 1st June from Sheela Upadhyaya stating '*We are in the process of seeking clarity on the areas raised at the meeting and will update you next week*'.

Publication of the guidance and draft MAA

Despite our request, the guidance and what was still called a **draft** MAA was published on June 4th without any acknowledgement of our requests. There was no clarity in this release as to whether people could comment and, if so, how they should do so. There was only information about how to appeal.

All the PAGs spent the next few days trying to calm what was the beginning of alarm within the SMA Community, reassuring people that we had already expressed the concerns they were raising to NICE / NHSE, that clinicians were also expressing the same concerns and that we were hopeful we would have a positive response later in the week.

Communication that the MAA was in fact final not a draft

To say the least we were shocked with the communication we received on 7th June that closed the door on the possible amendment of the **draft** MAA.

Our subsequent work with clinicians and the SMA Community

Since this time, we have been working hard with clinicians to find a positive way forward while at the same time trying to manage the huge distress this has all caused in the community. To give you a glimpse of this, here are the reactions from the parents of a child and an adult who the MAA excludes from treatment because they were clinically classified as Type 3 'walkers' but are now non-ambulant:

Parents of B (age 4, SMA Type 3): has made contact several times by email and phone. The mother has had to take time off work she is so distressed, *'Yes I'm struggling with this whole situation. I don't get how we can have come so far, campaigned so hard to be then told she was never in the running. How can B and others like her be left to rot over the next 5 or so years? How are we expected to watch others stabilise and regain whilst she declines? The thought of this I find soul destroying. Her condition will only get worse, she has no other complications at present but more than likely will have at the end of the agreement in 5 yrs. time. What's worse is that on paper she's the ideal candidate. The impact of this is beyond devastating.'*

They didn't tell their daughter about the treatment until after the announcement on May 15th at which point the media contacted them for a reaction and asked if they would be prepared to do a follow up interview at some point following treatment. They of course agreed as they were so excited. Now, the mother reports she just didn't know what to say when last night she was fitting her daughter's callipers and her daughter asked, *'When will I get my medicine mummy?'* A bright little girl who has kept asking why she can't walk any more.

Example from non-ambulant adult with SMA Type 3 given in actual letter – not published here.

Many have asked, *'When will we know?'* and are finding the waiting unbearable as shown in this reaction from **Parent of child C (age 10, SMA Type 2)** who would be included in the current criteria, *'Having anxiety attack due to state of son's bent up back with the threat of appeal that would delay the treatment he needs. We've managed 10 yrs. with little to no support & now we're supposed to be part of a "community"'*

This also highlights the damaging impact these decisions and the way they have been communicated have had on what is a close community. Today we heard from **Adult D** who was diagnosed with SMA Type 3 as a young teenager and is now in her forties. She said she has never seen families more distressed as she is now witnessing on social media and how worried she is, especially for the children and young people who are losing their mobility and muscle strength as she did. She asked if we could set up additional helplines to provide emotional support. Sadly, we simply don't have the capacity to do so but will of course continue to do all we can to support families and adults.

We refer you to all the awareness raising that has taken place this year with regard to mental health and the need for support, especially for young people. The All-Party Parliamentary Group for Muscular Dystrophy report on *'Access to psychological support for people with neuromuscular conditions'* contains many powerful testimonies from people with SMA about the additional impact such a condition has on the mental health, not only of the people with SMA but also their families. There is all party agreement that more must be done. We also remind you that the Patient Experts and people who made submissions to NICE also



highlighted this many times and that this was a key theme that emerged at the recent Impact HTA workshop on MAAs

In Conclusion

We urge you to remember how vulnerable the people in our community are when you are making your next decisions and are communicating what these are and why and how you have made them. We want to be able to work with you on the next steps of the MAA. We ask you to hear and respect the suggestions we make available to you and our motivations for doing so.

Kind regards

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