

## **Appendix 9.**

### **Who should have access; views on which groups might have priority; other comments for NICE.**

#### **1. People with SMA- comments about priority suggestions / other comments for NICE**

**Twenty-four commented that it should most definitely be available for all who want access**

##### **Type 2 age 3- 4 years parent**

From research that we have read, the more earlier you have access to the treatment is of greater benefit however from other people's personal experiences, even with older children and young adults it has helped them to achieve more muscle strength and maintain their skills and make them less fatigued which can make life more fulfilling if you are not tired all the time.

##### **Type 2, age 13-17 years young person**

Have only read about very young children being treated with nusinersen and the very positive effects this has had. If it could be used for all forms of SMA and have a positive effect then this would certainly be life changing for anyone with SMA and provide good value for money for the Government.

##### **Type 3 age 13 - 17 years young person**

Why would nusinersen NOT be given to those with SMA? Price is the only barrier at the moment as far as I can see and it is immoral to price probably 90% of sufferers out of receiving the drug!! There must be some common ground between Biogen and the NHS which works for both in terms of cost? Why would Biogen put the effort into creating a treatment that is not being fully utilised?

##### **Type 2 age 13 - 17 years young person**

SMA is a very cruel condition to have - it affects not only the sufferers in their everyday life but also their families. It is awful not to be able to do the simplest of things (such as scratching your nose, lifting your head or your arm) on your own and needing someone to be with you at all times! There is NO privacy, any moment of day or night. I believe that nusinersen will give me a bit of control of my body and therefore some form of independence and privacy as and when I need it. There is potential to transform my life; I have real academic capability but there is a great challenge to make the most of it. Nusinersen must be available to everyone with SMA; it is discriminatory to give it to a minority because drug companies charge a large amount of money for it. Many countries around the world provide nusinersen to all types of SMA. Why is England (NICE) being left behind?

##### **Type 3 age 18 - 25 years adult**

I understand that the drug may not have as much of an impact on older patients, but the support or provides would still be invaluable

### **Type 3 age 18 - 25 years adult**

I feel everyone who has SMA should be entitled to this treatment and I feel it's unfair for those who has a less severe type of SMA to be excluded from a treatment that can help improve muscle strength and maintain it.

### **Type 3 age 26 - 35 years adult**

I have seen the benefits first hand from people who have been treated with Spinraza/Nusinersen on Facebook. Many people who have had the injections have reported strength increases and have been able to do things they previously haven't. This is from being involved in the SMA groups of Facebook and seeing Americans and German's who have access to the drug.

### **Type 2 age 26 - 35 years adult**

It should be available for everyone who wants it. The cost will be offset by less NHS / social services interventions.

### **Type 2 age 26-35 years adult**

The UK is known for its prominent role in the research community, I hope it will also be known as being one of the countries to embrace orphan drugs and for allowing open and equal access to nusinersen.... SMA is time-sensitive. Nusinersen received EMA approval many months ago and I understand that NICE is not likely to announce a decision until November. Please bear in mind that every day that goes by, we are losing function. It is heart-breaking to know that there is a drug out there that could at the very least slow progression, and not be able to access it. There are no other interventions at the moment, no other way to try to fight the condition.

### **Type 2 age 36-45 years adult**

There has never been a drug for SMA. This is a game changer for us with the condition. I am nearly 40 and have been aware of research being done for a drug most of my life. Now that it is found it seems crazy not to allow access to it. There aren't many of us with SMA and we've not got any other options. Anything is better than nothing. I have two healthy young children who need their mum and I want to be the best I can be for asking as possible. When you're as weak as me everything helps. It might not be much to an able-bodied person but being able to swallow or cough better is everything to me. Too many people are dying and I believe the drug should be made available. I lost my brother and the loss is unbearable-please help us get access to this drug.

### **Type 2 / 3 age 36-45 years adult**

All types of SMA should have access initially to see how it affects each individual. The longer-term benefits can then be assessed on a case by case basis.

### **Type 2 age 36 – 45 adult**

I'm desperate to try this drug. Even a bit of strength or slowing down the atrophy process would make a huge difference to my life and my family (who help me day to day).

### **Type 2 age 46-55 years adult**

I think nusinersen should be an option for everyone with SMA if there is real possibility of improving strength/function. Although it is a very expensive treatment it must have a cost cutting effect if people with SMA need less social care, less healthcare and could contribute much more to society if nusinersen is an effective treatment.

### **Type 1 / 2 age 46 - 55 years adult**

It really should be merit and those who will benefit the most in the immediacy and from a clinical point of view, I would perhaps start looking at how much somebody's care and therapies are costing just to see whether they could project into saving or being a slightly more cost-effective procedure. But, you cannot put a price on a life, or happiness, or health.

### **Type 3 age 46 - 55 years adult**

the nature of SMA and the associated atrophy in my view means that once you reach adulthood means that the body, particularly the skeleton, will have adapted due to the physical demands of managing the condition. To then seek to reverse this in adulthood would be of less value than to those who are younger and as such their bodies are better able to adapt as a result of the treatment

### **Type 3 age 46-55 years adult**

People with Type 3 have some strength and ability to live more independently. This should be supported and they should be given the chance to avoid dependency through physical deterioration and decline.

### **Type 3 age 56 - 65 years adult**

Having read many peoples experiences of nusinersen, I have not seen A SINGLE negative report. Babies & young children do see results faster, and these are life changers, but many adults have also been amazed by the gains they have made; whether its strength, more energy, regaining lost abilities such as feeding themselves, brushing teeth etc, we all deserve and in my opinion, are entitled to QUALITY OF LIFE.

## **2. Unpaid carers - comments about priority suggestions / other comments for NICE**

**Twenty-two commented that it should most definitely be available for all who want access.**

**Twenty-seven made strong statements about the need for urgent access - a representative sample is included.**

### **Type 2 age 0 - 2 years mother**

After extensive research on the impact of Spinraza in other countries, we have noticed that the under 5 group seem to have the most positive response. Type 2 have a significantly greater strength than type 1, having travelled further down the gross motor development pathway, Spinraza seems very

effective at maintaining these gains. The under 5's could be accessed before they reach the peak degenerative period which appears to be age 5-15.

### **Type 3 age 3-4 years father**

As a Type 3 SMA person if my daughter gained access to Spinraza the likely improvement would see her as not strong enough to be considered normal, but strong enough to have a normal life! which could be achieved with this drug. Conversely, more severe types of SMA such as type 1 may see an increase in functionality or life expectancy, but still have a very limited life with the requirement of huge support systems and medical interventions. This would not be the case for milder forms SMA in patients. The cost benefit balance would see a type 3 require little or no assistance from people or the state if given this drug, in the long term it is cheaper to provide this drug than all the other support services. However, if the drug was given to type 1, even if there was an increase in functionality that patient would still require a great deal of financial and state assistance. I believe this drug is best placed at children under 16yrs or younger with milder forms of SMA, such as type 3.

### **Type 2 age 5-12 years parent**

There is currently nothing at all to try and mitigate against the effects of SMA. This is soul destroying as a parent and a terrible fact to have to share with a growing child who has questions about whether there is anything that might help. Small improvements in muscle strength have a disproportionately huge impact on quality of life. So, going from not being able to pick up a drink to being able to do this, for example, is a really big deal. Anything that can increase muscle strength will be life changing for children with SMA, and potentially life-saving if it keeps the respiratory muscles a bit stronger. Watching some of the younger SMA children in the USA hitting milestones and achieving mobility having been treated with Spinraza is really emotional. I wish we could have done that for our son when he was little.

### **Type 2 age 5-12 years mother**

If my son were to receive Spinraza, he may be able to stand up or even take a step. This would be lifechanging. However, if this were never to happen, Spinraza could still massively enhance his quality of life by enabling him to do many day to day tasks which we all take for granted. Such as: Manual dexterity/strength - Taking off a pen top, opening a birthday or Christmas present, opening an envelope, opening a packet of crisps or sweets, pushing a straw into a carton, opening a bottle or jar. Pressing buttons e.g. for using a pedestrian crossing, pressing a lift button or doorbell, turning on a light switch or lamp, pulling the trigger on a water pistol, building lego unaided, using a pair of scissors. Personal care and dignity - Dressing himself, buttoning up a shirt, zipping up his trousers or jacket, tying his own shoe laces, brushing his own teeth, squeezing a tube of toothpaste, styling his own hair, cleaning himself after using the toilet, pressing a soap dispenser to wash his own hands, being able to scratch his own itches, cutting up his own food with a knife and fork. Health and Wellbeing – Having stronger mastication muscles and swallowing reflex to chew and eat normal amounts of food without the risk of aspiration or choking, catching a cold without the risk of gagging and vomiting with every weak cough, having to use a cough assist machine or a home suction machine to remove secretions from your own child's throat and seeing the look of utter horror on his

face at the mere sight of the machine, having endless courses of antibiotics and hospital visits for chest infections, which could have serious or even fatal consequences. 2/2 Core strength benefits – Having stronger core muscles to support his own spine, preventing scoliosis and the need for spinal surgery. Maintaining good posture benefitting his respiratory function, reducing the amount of hospital admissions and therefore saving the NHS time and money. Being able to turn himself over in bed, repositioning or sitting himself up if he loses balance, empowering him and giving him a huge increase in independence.

### **Type 2 age 5 - 12 years mother**

It's very hard watching your child suffer when you know that there is something that can be done to prevent this from happening. It seems unethical not to provide them the opportunity to a treatment.

### **Type 3 age 5-12 years mother**

For teenagers and adults, it's all about maintaining what they've got which is huge because being independent is the most precious thing and will cost less to the state as a whole. How much do you pay for your independence? We have no time to wait around, days can make a difference between life and death for the babies type 1. We have no time to wait around, weeks can make a difference between whether or not a type 2 child will lose an ability to brush teeth, scratch head and survive a flu. We have no time to wait around, months can make a difference between whether or not a type 3 child will lose an ability to walk and be forever confined in the wheelchair. We have no time to wait around, months can make a difference between whether or not a type 4 adult will lose all of their independence and therefore dignity.

### **Type 2 age 5-12 years mother**

We are sensible and realistic. We do not expect that upon some treatment/drug our daughter would get out of the wheelchair and walk. It would be fantastic if it just prolonged their life expectancy and improved quality of life (like hand functionality / joints contractions, better muscle strength, etc - so they are able to perform more everyday tasks independently and for longer. I think that is the most important aspect of this: the improved quality of life and more independence. That, in turn, would enable many parents to return to work at least part-time and make their contributions too (to society and the tax office), realise their potential and be a good role model to their kids.

### **Type 2 age 5-12 years mother**

Had this treatment been made available to my son over a year ago as it was to many others there is a strong chance he would still have the ability to perform tasks like sitting up himself or washing properly independently

### **Type 2 age 5 - 12 years father**

Even if Nusinersen does not provide the desirable results for all patients, clinicians can learn from the results. It will help to develop better drugs that work on wider across SMA spectrum and improve drug delivery mechanisms.

### **Type 2 age 5 - 12 years mother**

The costs associated with having a disabled child are extremely high. With treatment this would be dramatically reduced. Money would be saved on hospital stays, equipment and care. It could also help the economy as it would stop parents / carers having to take time of work /stop working.

### **Type 2 age 5 - 12 years mother**

SMA is devastating and we grieve every day for the life my Daughter could have had without SMA. She is suffering terribly with her mental health and would love any improvements in strength. The impact SMA has on her everyday life is massive and every day is exhausting physically and emotionally.

### **Type 2 age 18 - 25 years father**

Negotiate a better price with the pharmaceutical company. The price for care for a person with SMA per year will justify the treatment-never mind the change in life

### **Type 3 age 18 - 25 years father**

SMA is not a lifestyle condition brought upon by excessive smoking, drinking, eating, drug taking etc. As such I feel very strongly that this drug should be prioritized above treatments for such lifestyle conditions.

### **Type 3 age 46 - 55 years partner**

Plenty of funds go to self-inflicted illnesses but not enough to people born with imperfect genetic makeup.

### **Type 4 age 46-55 years partner**

My husband's condition affects all aspects of his life and in turn affects my life, our children's lives and others in our family. He has continued in his profession despite this becoming increasingly difficult for him. More and more this is looking like something that will not continue for much longer. Once he becomes unable to work, this will have a much greater effect on our lives not just financially but also emotionally. We may not be able to stay in the family home nor provide for our family as we have been doing. However, if by taking nusinersen his energy levels improve, this means he will be able to maintain financial independence and equally important be able to feel good about himself as he contributes to the family and to the society in general. His work not only provides for us, but also provides employment for others. I believe that as we all contribute to the development of new drugs, so should we all benefit from them.

### **3. Other relatives - comments about priority suggestions / other comments for NICE**

**Twelve made statements calling for access with six advocating for treatment for all. Examples of comments:**

#### **Type 3 age 3-4 years grandparent**

I don't believe it would be either ethical or fair to treat only type's 1 and 2 only as we have read. Our understanding is that type3 is rarer so wouldn't it cost less to treat this group as well

#### **Type 3 age 13-17 years grandparent**

NICE members need to experience a day/week in life of those who have SMA and those who care for them.... walk a mile in their shoes/wheelchair!....to see how Much of a support the improvement in physical improvement would be in physical, social, emotional and educational access. I ask them to give serious consideration to extending access to nusinersen. Thank you for this opportunity to comment

#### **Type 2 age 18-25 young adult**

Having added strength would be life changing, and would give me a massive part of my independence back, something which I along with all other SMA'ers deserve.