

Appendix 4 – Comments from Surveys

Q. How important would improvements in different aspects of their health and daily living be / have been if these could be / have been affected by a drug treatment?

Adults with SMA responses

Very important to maintain my current abilities and not deteriorate.

For myself any improvement would be fantastic. I have weak neck muscles, and while I can support my head most of the time, I really want to be able to maintain this ability. It is so important for so many everyday functions. As important, is improving or maintain my breathing and swallowing.

Breathing, swallowing and communication for the SMA patient are high functioning at the moment, as such the questions above are not applicable

All of the above are extremely important. I have severely reduced lung capacity and anything to improve that or prevent it from deteriorating would save my life. It could extend my life expectancy and no words can convey how important that is to me. It would also ensure I can maintain the milestone of sitting up in my wheelchair and ensure I can maintain the ability to type, use my phone, and put on my make-up. I would love it if I could one day open a packet of crisps. I thrive off independence and I would be so much less reliant on people if I could open a pen to write, open a door, or open a bottle of water. I am terrified of losing my ability to swallow and communicate, treatment will stop that.

Improved mental health.

Walking with aids (Crutches) is not easy and there is always a danger of falling down and breaking a leg or arm. It will be impossible to do anything myself if that happens.

General mental wellness

It's very important to maintain as much independence as possible, both for physical & mental health. It's one thing using an electric wheelchair because you cannot walk, it's totally different losing the ability to use your arms. Maintaining strength for independence and mental health is vital.

The SMA has a general negative effect on life, from mental health to work, leisure time and domestic abilities

Ability to move in bed, possibly go to the toilet or make a cup of tea would be amazing

To have my independence back. I live alone. And cannot even go to the toilet alone I need caregivers to help. To be in charge of my own life.

Every single bit of improvement matters.

Maintenance of ability to self-drive electric wheelchair, which is becoming increasingly difficult

Although breathing eating and communicating are not currently a problem, it's not abilities I wish to lose.

The ability to drive again.

Parents of young people age < 18 years responses

Breathing, swallowing and communication for the SMA patient are high functioning at the moment, as such the questions above are not applicable

Walking 5 independent steps is by far not the most valuable this and should not even be in the MAA criteria. Improvement in back and neck strength, the ability to transfer, cut up food is of far greater importance for a Type 3a.

Ease for me her mum as it is hard work looking after her everyday. Quite exhausting and also limits social interactions. It is important for her to lead a normal life and be able to interact, engage and participate in activities with other children of similar age.

Self-confidence and mental health would improve dramatically with treatment as well as my daughter's general belief of self-worth, which she has very little of currently because of her SMA condition!

To be more consistent and not having 1 illness of a simple cold - lose a function she has worked so hard to gain and maintain.

Ability to be more independent and stay healthy being able to fight off infections

To have a general overall increase in health and ability to be the same as their peers

Upper body strength to write, do hobbies, self-care with personal hygiene. Especially arm strength, brush hair, teeth, lift cutlery, cups, use computer

Other responses

I think her mental state would be very much improved.

Can now roll over independently in bed at night so carer does not have to administer assistance.

the prevention of loss of upper body strength would have been a huge improvement.

Improved use of arms cannot hold anything heavier than small cup of tea and knife and fork. Cannot cut food. Cannot sit herself up. Turn over move legs in bed.

As my sister gets older, she will lose the mobility that she has, and her independence will decline.

Q. Please rate whether you consider each aspect to be an advantage / disadvantage of this treatment.

Adults with SMA responses

I think the main advantage of this treatment is that it can be taken orally, as a liquid. This is fantastic for someone like myself, who has difficulty with swallowing, and may not be suitable for other treatments that are more invasive.

The non-invasive nature of this treatment (and accessibility for those with fused spines and contractures) gives it strong advantage in all categories, especially in Covid times. There are fewer complications with oral medicine, and it is easier and safer to use.

Could be different to store when on holiday

I would prefer tablet form as I can take this independently, and preferably something that does not require refrigeration.

Refrigeration could be a problem if you frequently travel, otherwise it's not really an issue

It doesn't need to be taken at home - people often buy small portable refrigerators. The biggest disadvantage is possible side effects of the drug, not necessarily any of the points mentioned above.

Infinitely better than a lumbar puncture which may not even be possible.

the biggest disadvantage would be side effects and what other activities it would limit

The strong advantages are significant.

Parents of young people age < 18 years with SMA responses

Major advantage is that it is none invasive, so not side effects from the lumbar puncture. No theatre space required.

This is a small sacrifice for a potential gain in strength

For my daughter, having Spinraza every 4 months is quite a disturbing experience, as she doesn't feel very comfortable being around people she doesn't know very well and who does put a needle on her back. Also for me ,as a mother (even that I trust the doctors and all the people who treat my daughter), is a very hard time to see her crying and scared, so yes , a drug that can be given at home, it is a very important advantage.

Others' responses

in my opinion my sister would try even the most painful procedure if there is a hope that she can be more independent. However, Risdiplam seems like very easy and effectful so I am really hoping that she can be able to get it.

In our case, the syrup will be administered via feed tube. Hugely advantageous due to spinal curve which currently entails the involvement of a radiologist as well as the neurologist for administration of Spinraza.

This 1 to 5 scale does not really work for me. There are NO strong disadvantages in the list but there are things which make no difference e.g. refrigeration.

The lack of requirement to have a surgical procedure with risk of infection is a plus. Loss of school days for visiting hospital 150 miles return in a day is of enormous benefit. At a time when hospital visits are only possible in emergency cases home treatment and administration is a definite positive to reduce risk of catching COVID 19 on journeys and in hospital. It releases clinicians to do other essential work

The risks associated with regular lumbar puncture in my opinion are too great for my sister. She has reasonable mobility in her lower body and the risk of damage to the spinal cord is too high. Therefore, taking an oral medication would be much more suitable for my sister.

Q. NICE will assess what impact this drug has on aspects of 5q SMA. Considering what is known so far about risdiplam (see clinical trials summary), what are your views on this

Adults with SMA responses

It is looking extremely encouraging in trials so far, especially improvements with motor skills, breathing and swallowing.

I need to do further research into impact on fertility.

There doesn't seem to be enough data to confirm/deny any side effects caused by the drug.

If you choose to take this medicine, then you take any risk of side effects. That's why I've answered the way I have.

I'm not sure if there is definitive proof about impact on male and female fertility, I think it is currently not advised to have children whilst on this drug but I am unaware of a significant study done that would indicate whether this was more than "safety first" rather than something that is proven to be a definite risk.

It would be useful to know the side effects from all trials, especially those with older people rather than children.

If given a real choice between Risdiplam and Spinraza - I would choose Spinraza in a heartbeat. I am going to start Risdiplam on EAMS because I am exhausted but if side effects occur, I will stop taking it. I will have to stop taking it at some point to have my own children. I feel MY personal concerns are not a reason to delay this drug - EVERY SINGLE PERSON with SMA should have the option to take Risdiplam. It has been amazing and life- saving for the many. We need every option on the table for every single person with SMA and we needed it yesterday.

Until there are results from the Jewelfish group, it is difficult to tell what impact the treatment may have on me.

Parents of young people age < 18 years with SMA responses

SMA is degenerative as such all stabilisation and improvements are extremely positive.

Although the fertility issues come with cons this is something, we would discuss but as a 6-year-old we would choose quality of life as there are other means of fertility available

At this stage in my daughter's deterioration (i.e. a constant, marked and gradual weakening), even a stopping of progression without a gain in strength would be vital to her. Every day that passes she loses a daily function that is vital to independence. I.e. unable to press a lift button, unable to stay warm at school (jumpers limit movement), unable to eat outside home for fear of choking, unable to cough anymore. Unable to manage school or working day due to poor stamina. A bright, clever child about to put back in society who within a year will not be able to do that and will require society to look after her.

Q. Consider the information about risdiplam treatment as a whole, please rate how acceptable it is in your view as a treatment option for 5q SMA.

Adults with SMA responses

I feel it would be a great treatment, the ease of taking it, is a real bonus.

It's non-invasive and can be self-administered at home without medical professionals. That's a milestone.

I would like more info about the side effects; however, the pros seem to outweigh the cons.

This is an opportunity for me to finally access a treatment after a lifetime of nothing! That's 64 years, a long time! It would be 100% acceptable for me!

Not only is it acceptable, it is vital.

I accept everything, I just want to stop the progression of my illness and give me more strength

I have to say I don't believe that repeated lumbar punctures are a safe method of administering a drug over a prolonged period. Oral medicine kept at home and able to be dispensed direct to the patient via postage/courier makes much more sense in terms of cost effectiveness as well as patient safety. In addition, I believe that a measured dose every day will be more likely to provide consistent results over a long period rather than the "peaks and troughs" experienced by those taking other medication spaced out over a longer period of time

If my doctors are recommending it for me, then I would be very keen.

I believe that, for the majority of the SMA population, the advantages will far outweigh the possible risks.

Parents of young people age < 18 years with SMA responses

Would like to see comparisons of different treatments, improvement and side effects.

It is easy to administer. As it will be taken orally at home. I don't have to wake baby up early to get her ready to travel to the hospital. She will be free from lumbar puncture pain.

There is no other treatment for her as she is non ambulatory type 3.

Which of the following groups do you think should have access to risdiplam?

Adults with SMA responses

Ideally, I would like this treatment to be available to all with SMA. Everyone deserves the chance or to improve their condition, enabling them to be healthier and more independent. However, as type 0, 1 and 2 are life threatening, cause individuals to be so reliant on others, and have serious disability, I feel that they should definitely have access to the treatment.

all should receive the hope of more independence!

All those with SMA should be eligible.

We should all have this treatment

At all age groups. Very specific tests should be set up to make sure the drug is providing improvements.

I think everyone should have the option to access the medication and decide if this is the right option for them.

SMA affects us all very differently. It's a devastating disease no matter what type you are. It steals your abilities & in turn steals your life. Every person with SMA should be given treatment.

Everyone should have the opportunity

And SMARD too if it will also have a positive impact.

As someone with SMA, I believe everyone has the right to access treatment where available in order to get the highest quality of life possible. My primary concern will always be the wellbeing of someone with SMA rather than the value for money of any treatment.

Treatment for all

I know several people on trials of this drug, from many different "type groups", nearly all of them have experienced minor to major improvements (major in the case of children), I do not know anyone personally from any type that would not benefit from at least having the SMA stabilised so that they can get on with their life without SMA affecting it (obviously, other things may affect it but if this can be eradicated then that would be a massive advantage to everyone).he

I believe everyone affected should have the option to the drug if they want. I understand that different types have differing deterioration rates/abilities to do things i.e. sitting up, walking etc. However, all types see deterioration of some sort that does affect the person and their families.

Are you getting the message yet NICE? EVERYONE.

I would like to choose more than 1 type, I believe all treatment should be available to all types, no discrimination please

Anybody who is eligible should be allowed to trial

I am type 3 but everybody deserves the chance for an improvement in their lives.

Anyone of any type whose clinician believes it would benefit

Really all should be given the opportunity to make their own decision

Q. Other comments

Adults with SMA responses

Having SMA myself, it affects my everyday life and I rely on carers for everything. I am confined to a power chair and am extremely weak, with very limited mobility. I am unable to do so many things like lift my arms to brush my hair, but I am still able to brush my teeth, which is so important for me. It impacts everything from getting out of bed, using the toilet, eating, going out, and even just getting comfortable in my chair or bed. Most important is the difficulty I have with my respiratory and swallowing functions which causes most frustration and worry. I hope that potentially the treatment would provide some improvement with my physical strength and movement, or even help to maintain my current level. It would mean a lot to be able to continue to support my neck and head as this is so important for safe eating and swallowing. It is so important that my muscles maintain as much strength and stability possible to make breathing and fighting illness easier. I really want to be able to keep my independence

and carry on using my hands to drive my wheelchair, hold my toothbrush, use my phone, write and use the computer for as long as I possibly can.

I have been depending upon my family the whole of my life! all I want is to be able to live more independently with my day to day activities! I really would like to have a hope!

To have a non-invasive treatment within reach but not quite accessible is tantalising. SMA is unpredictable and can progress at any speed, at any moment. Anything to delay that progression, or maintain existing strength, will do the world of good to people's physical and mental health. It's not fun thinking you've reached middle age at 15, and society has a lot to do to make the world more inclusive and accessible, but Risdiplam will also help with the barriers like progressive breathing and swallowing challenges and motor functions. Let's not make disabled people fight for a good quality of life anymore.

My condition has deteriorated considerably over the last ten years & I cannot overemphasise the effect this has had on me. Ten years ago, I was completely independent and an active member of the community but now have to have help everyday and my activities are becoming increasingly limited. I find my situation extremely distressing and I fear the discomfort and misery that old age currently offers. I have been excluded from accessing Nusinersen despite evidence showing it could prevent any further deterioration. Risdiplam is the only hope I have of maintaining my current level of mobility.

I've been on drug 10 months now and doing very well, I'm 59 and this is the first treatment for me, I've had no illnesses, no hospital stays and no pneumonia since starting the drug so amazing for me

Tiny margins of increase or halting decrease would have a huge impact on all areas of my life.

I would hope that when NICE are making their decision about the use of this drug, they will take into not only the possible cost savings of people living more independently, also the availability and cost of care.

As long as the drug company aren't looking to rip the NHS off, considering the information provide, it seems a no brainer to supply Risdiplam now, why is any time being wasted if no further data is going to be considered. Put very specific tests in place to make sure improvements are real and then it can be stopped if needed.

I probably won't get Spinraza due to spinal deformities and fusion so Risdiplam is my best option to alleviate my condition or at least minimise further progression.

Any treatment that will improve my mobility will be absolutely wonderful and will immensely improve the quality of my life. I am living with this condition for over 20 years and would like to see some help from medication that would improve my mobility.

I believe Risdiplam should be considered as a suitable treatment for SMA as it would open the market to different medications and allow those with the condition to make informed choices as to which treatment is suitable for them.

I've had restrictive SMA since diagnosed at the Radcliffe Hospital when I was early 40's, I am now 73. I have seen a slow decline in my condition, from walking straight legged, through stages - walking with a stick, to two sticks, to walker frame, to frame and wheelchair. I have been told (specialist at Salisbury hospital) that the years of additional leg use knowing that some muscles in my upper leg don't work have put additional strain on my knees especially my left knee which now bends backward.. It would be great to walk again instead of the wheelchair, but at my age I believe the available treatments should go to the young.....

As an older adult with SMA I feel completely forgotten about. We have no feisty parents fighting for our every need. I'm sick of struggling, every time we lose an ability it hits so hard, and this happens on a regular basis. It hits us physically, but also mentally, and it's very frightening. To receive a treatment like Risdiplam would be an absolute miracle, to have the hope of no further progression, or the hope that we could actually improve and maintain our strength, our health & independence would be beyond anything I could even wish for. Also, to receive treatment would mean so much to our families, those that live with us, that love us, that can see daily just how much living with SMA does to us, the ones that watch us struggle & cry. I know for them just what this medicine would mean.

Treatment for all

I have type 2. I am "lucky" enough to have been to uni and am still, aged 47, in full-time employment. This treatment gives me hope that I can continue to do my job, contribute to society and pay taxes like everybody else gets the chance to do

With the potential increase of mobility and breathing functionality, I think it will significantly improve the quality of life of myself and many others that are in the same or similar situation. This could possibly remove the need of ventilators and potentially increase the chances of us gaining employment. Not only that, it could reduce the reliance on the NHS equipment which should help make it more cost effective. Additionally, with it being administered at home, and how simple it is to take, I think that it will allow many of us to have the opportunity to increase our quality of life, as I know myself and many others are unable to participate in some of the other drug trials due to spinal rods / fusions etc..

I am 53 years old; I need access to this treatment to prolong my life and to also give me more ability hopefully...

It is extremely difficult for our mental health, to know there is a treatment available, but it is out of reach. Risdiplam is a medication we could only have dreamed of when I was growing up, especially due to the non-invasive administration. I personally would prefer tablet form but am more than happy to take a liquid medication if it means I do not have to take time off work for hospital administration of a drug (like nusinersen).

I am 27, and with Risdiplam, this is really the first time in my life where treatment seems like a possibility and there is some chance to maintain my current level of strength, and quality of life (which is very high), and perhaps even to gain some strength and motor milestones. The delivery method seems such a great advantage compared to nusinersen. I am in no position to be able to offer any view of value for money for this treatment compared to the current cost of care for people with SMA but I can certainly guarantee the hope it offers with Risdiplam becoming available, and the clear benefits it could bring to my life.

I think everyone with SMA should have access to this drug. This is life changing treatment for people, even a slight improvement for someone with SMA is life changing

SMA progress has accelerated in the last two years. I cannot keep straight position, I cannot raise my arms, I have a very weak cough, I am starting to have breathing problems. I would like to stop the progress of SMA I hope for some more strength, more energy I would like to use my right hand I want to breathe by myself

I am 52 years old, I never believed in my life there would be any kind of treatment for SMA and now that there is, it is coinciding with a rapid decline in my functional ability. I would like the opportunity to, at a minimum, arrest this decline to enable me to continue to be a functional member of society rather than dependent on the state and/or charity for my ongoing well-

being. I have lost major functionality in my hands, arms and fingers, it has happened very rapidly, and it is now affecting my ability to drive my chair and operate a computer. Any opportunity to alter this, even to arrest it, would deliver a significant impact to my life and that of my wife and children. I would also hope that any potential improvements could even lead to me resuming my extremely successful professional career that has had to be interrupted due to my physical deterioration over the last 2 years.

Risdiplam would be a life changing treatment for me and others like me. I have watched myself deteriorate slowly over my life, getting weaker, struggling to eat food I love and participate in the independent and active life I enjoy. Anything that can prolong my life would be the best thing to ever happen. Making Risdiplam available to as many people with SMA as possible is the only right course of action.

In the past all research has been focusing on type 1 and 2 and 3. Nothing on type 4. Is type 4 not as important? Is my life over with nothing to look forward to except caregivers and an old folks' home?

As a 45 year old female with SMA type 2, I would benefit greatly from risdiplam as in recent years my strength, stamina, breathing etc have deteriorated and in order to allow me to continue to work in the future (I'm a self-employed Counsellor) some medication may be a great benefit.

I'm tired of filling out a million surveys explaining my view, my life, my experiences, when NICE and the SMC never listen. Why don't you educate yourself NICE? Why don't you read up on disability politics and stories already out there on the internet, in books and media? Why don't you do the hard work for once in your life? There is so much information out there- the level of ignorance on disability is unacceptable. If we were talking about any other minority - women, LGBT+, BAME, etc - there would be zero tolerance on ignorance. We're not here to provoke pity or repeat the worst parts of our lives to evoke emotions from the people in charge of deciding this over their morning coffee. Having every single treatment option available to every single person with SMA is a human right. My life is valuable and as a human being I deserve to benefit from any advances in medicine that make my life easier and more independent. Get your act together NICE.

The thought of an oral liquid treatment makes me very happy, as lumbar puncture is very frightening and I also struggle to swallow pills.

Risdiplam seems safe and effective. It's vital that all SMA patients have access to this drug ASAP as we are all deteriorating.

I'm 34 years old and at this present time I've received no help at getting any of the drugs that are available. I'm hoping that sometime in the future I will be able to have access to a drug that would provide a great improvement on my life. Nobody knows what an individual goes through with my condition. Any help at all would be very much appreciated. I read up on everything that is happening and the advantages that are being given by the new drugs on the market would provide such an improvement in people's lives.

I am slowly losing any independence I have left. I will need a full-time carer and a hoist if I continue without a treatment. This had put more reliance on my ageing mother who has her own health problems. The current rules for Spinraza don't make sense. If I never walked, I can get Spinraza yet if I was walking now, I could get it. This is unfair and I don't want this to happen to Risdiplam. Risdiplam has very good efficacy and is easy to take. It's a no brainer to use it. There isn't a lag period either like Spinraza has when you are waiting between doses. I just hope that the NHS/NICE come to the right conclusion and provide Risdiplam for all that

need it. I will lose faith with the NHS if they continue to withhold a much-needed treatment for myself and others like me.

Every opportunity to test therapies should be offered to all individuals with SMA. I was able to continue working until 60 years but on reduced 3-day week for the last 15 due solely to fatigue and difficulties with walking.

Parents of young people age < 18 years with SMA responses

I hope that we will be able to take this medicine and that it will improve our condition

To stabilise and reverse the effects of a degenerative disease like SMA is amazing. Criteria should be based on facts and clinical guidance. The 5 independent steps in 12 months criteria placed in the Spinraza approval should be removed. There are many more abilities and skills far more important to everyday life, cutting up food, back, neck strength, ability to transfer. It's been written that SMA is a spectrum and the Typing system was implemented by a team of clinicians and geneticists to allow centers to compare finding when trying to identify the genes. This is now being used to discriminate.

My 11-year-old daughter has type 2 and is getting significantly weaker especially since her spinal surgery followed by lockdown and then to have her current anti fatigue medication discontinued (salbutamol) has had a devastating heartbreak effect of her. She is weaker all over especially in her arms now. She is of an age where image and independence is key as much as stamina to keep up with peers at secondary school where workload has quadrupled! My daughter needs treatment urgently before she declines further. My daughter is vulnerable enough and no parent should have to watch their child fade away in the way she will if she does not receive risdiplam and SOON! she's already been through so much in her 11 years of life on this earth. She deserves treatment and fast DD

My son has started access to Spinraza however the treatment is difficult to access due to spinal fusion surgery. He wants to maintain his strength, health and independence so he can lead a full and happy life. He hopes to access a treatment that is effective, safe and easier to access.

5q SMA is surely one of the most devastating conditions for anyone to endure. It governs every aspect of their life as well as their family. Any potential treatment is good, but as risdiplam has already been proven to be effective, and can be easily administered, it is essential that it is made available for all types as soon as possible. This should be funded immediately by the NHS. Considering the amount of money that seems to have magically grown on trees to fight Covid19, then there is no excuse at all for funds not being made available to ensure that everyone with 5q SMA gets risdiplam straight away. Every day without treatment is a day that someone's health declines just that little bit further and living gets just that bit harder.

Risdiplam has made such a big difference so far in so many ways - more energy, more strength, better appetite, better sitting ability, better digestion - generally much healthier in every way - so so grateful he's accessing it.

It is a must asap

Even a small change made to improve the condition would make a massive improvement to the life and well-being of the sufferer.

I believe that anything that can help my daughter have greater strength in her muscles so that she can do things like raise her hand, pick heavier objects up, greater head control, can only be a good thing. It would give her greater independence both at home and school.

I have heard great things about risdiplam and as my son has no option for Spinraza this would be perfect being able to take orally and still show improvements.

Surely this treatment if rolled out on the NHS would be cheaper option than Spinraza for all types of SMA if oral and can be administer Ed at home

It is heartbreaking to watch your loved one failing to thrive and deteriorating before your eyes with this irreversible condition. This is compounded by knowing there are drug trials she cannot access and drugs funded in other countries that if she had had access to 3 years ago would have kept her walking and prevented scoliosis and back surgery. As a carer, I used to be a nurse working in the NHS, I had to give this up to care for her. The impact on the family is also great. Overwhelmingly, the psychological impact of being unable to access a drug (you could never fund yourself), that could stop your child fading away in front of your eyes, is the most tormenting part of this situation. I am actually in disbelief that this could happen in a country with state funded health care. Risdiplam could change the course of her life forever.

As we all know, to have SMA, or to have someone you love with SMA, is hard, very hard. I had times when I've cried seeing my daughter in distress, maybe in pain, and not being able to do nothing to help her. My daughter has so far 13 Spinraza. From her 3rd injection, I have been allowed to be with her in the room while she was having it. But I never find the courage to actually look at the needle going in her spine. So, a drug as Risdiplam, that can be taken orally in the comfort of our own home, is a dream come true.

It would be a lot less intrusive & reduce hospital trips. For 1 dose of Nusinersen for my son it involves: Transport to & from gosh, A room on a ward, Anaesthetist, Radiologist, Medical nurse, After care on ward, IV fluids & painkillers, Time off school, Time off work for myself & husband, All this could be avoided giving risdiplam at home

Others' responses

This could make a huge difference to my nephew's life. If it could reduce or slow the progress of his disease it would enable him to live as independent a life as possible.

my sister has SMA, she is 7 years older than me. When I was young all I remember was her struggle with everyday activities, the most important thing in my childhood her possibility of any possible treatments. My parents tried their best. For the last few years as my parents are getting older I am helping caring for my sister - she is very nice person, and is dreaming for "even to use the toilet alone without any help". We live all together and I married and have a 4 yr old boy. She loves him to bit, and is always saying that she will not have her own kid, so he is like a son she would never has! this is really heart-breaking. she barely cry and she is very positive person, however one day my son wanted to play hide and seek so, she said she wants to play as well - he then replied very innocent, but you CAN'T walk (he knows and usually don't comment or say anything or act weird etc) - this was the saddest moment in my life - she then cried - I think was the first time for some years she cried! I think everyone deserve the hope and the chance for a better-quality life! I think NHS should invest as this will minimise other costly treatments e.g. mental or physiotherapies etc

I am the grandmother of the 7-year-old patient in question who was able to start treatment with Spinraza in December 2019. There has been a remarkable change for the better in his strength and stamina. There was a worrying problem at his 4th treatment when the

neurologist's inability to find a space due to curvature of the spine resulted in abandonment on that occasion. Another attempt with the assistance of a radiologist went very smoothly and this is now the routine practice. A tube fed drug would be beneficial in eliminating future problems; would avoiding invasive procedures greatly reducing clinical expense.

She is my granddaughter, 20 years old with a good quality of life despite her disability. However, she has been warned that her supply of motor neurons will inevitable decline as she matures.....

Family friend 9/27 my wife's sister is with SMA – I've been married for 5 years and know my sister-in-law for 6 -she is very clever lady but not able to perform her day to day tasks such as using toilet, bath, etc my wife or her parents are taking care of her - she really need some independence since the parents are getting older. I am trying to help as well, but if she can have a drug to help her situation will be a life saver!

I can honestly say that my sister is very strong person, very well educated and Knowledgeable. She was till few years ago be able to eat herself, but not anymore, she can't use the toilet, can't get dress, brush her teeth, comp her hair - she need help with everything. She has been on a wheelchair and fully depended since toddlerhood - she needs some home; she deserves some independence and she is really hoping that she can get the risdiplam! Please, Please, Please NHS approve the funding and please let the Risdiplam be available to everyone not a person with specific type or age, give a chance to everyone!

The fact we have to go through this procedure is ridiculous. The drug should be made available to all those who need it now..... no exceptions... everyone.

Risdiplam is the only option left for continuing improvement to my grandson's improvement.

Every SMA patient fears each small deterioration in their remaining function whether it be walking or upper limb movement, swallowing or fine motor movement. The gradual progression to complete dependence is heart breaking..

I have read both the Information Summary and the Trial Outcomes documents and I understand the pros and cons of Risdiplam treatments. I played a big part in my granddaughter's in life from birth, the realisation of her problems and the eventual diagnosis of SMA type 3, up to the present day. Although Risdiplam will not be a miracle cure it will give her much more independence and freedom from worry that her present store of motor neurons will gradually deplete and her present quality of life will disappear.

As a family friend of a young adult (that I've known since birth) I've seen the struggles, frustration and impact that 5q SMA causes, on the individual and family. It is my understanding, that over time this condition could remain stable but there is a possibility that mobility in the upper limbs could dramatically deteriorate. This would have a huge impact on the individual's independence and the life they currently live.

Q. Are there any groups of people who have 5q SMA you think should have priority access to risdiplam?

Adults with SMA responses

I think the less strong types should get priority

I feel that people with types 1 and 2 should have priority as their condition is life threatening, and the quicker they receive treatment, the better.

everyone should get it depending upon their needs and not age or type of SMA

Type 1 and Type 2 as these are the sub groups with greater risk of life expectancy implications with breathing, swallowing etc. That being said, Type 3 can also face these challenges as SMA progresses, so it needs to be universal eligibility.

I think newborns should be given asap

Type 1

Infants with the most acute and life-threatening conditions should get priority..

Any group that includes me because I am biased. Trying to move away from my bias but still considering my situation. I was able to work with very little, if any, specific assistance, with not much more physical ability than I have now (I currently can't work but do actually live independently, cleaner every other week to do things I can't do and have to employ people to do all DIY and gardening etc.).

Anyone who cannot access Spinraza

Perhaps people who didn't qualify for other treatments. Anyone single with no family ties

Many people have access to SPINRAZA, there is approximately 50% of the SMA population that cannot get access to it. Surely, it's about time that those left with nothing should get the option now to be included.

Type II who cannot access Spinraza

children

1&2

Those who have lived with it the longest...

Those with more frequent hospital stays, those with rapid deterioration and those without access to Nusinersen.

This depends on the availability of risdiplam. If shortages to begin with, then I could understand priority being granted to type 1 patients. However, I still believe everyone has the right to access treatment.

type 1 SMA should have priority. However, everyone should have access to it, there should not be discrimination because of age.

The forgotten adult group, 50+

For one, it will be a drama that he cannot walk. For the other, the drama will be that he will never raise his hand again. Still others will cry because they can't breathe on their own.

Living with SMA is hard for everyone... For me, for my friends with SMA, for their parents. I'm unable to travel, I have no one who can travel with me to hospital. Risk management at home will be the best solution

everyone should get it, no one should be left out.

Type 4 should be given a chance. Up till now they have been forgotten about.

Although people the drug will help most significantly should have access, I do think that all people affected should, in time, have the opportunity.

I am sure everyone feels they are a priority. I feel it's important not to forget about middle and older age groups who have had to manage this condition for many years and who would still be greatly from treatment to reduce further deterioration.

Type 1 and weak Type 2 [those struggling most with health and breathing]. But there's no need to delay others getting treatment, it's not really a hospital dependent treatment.

Those who aren't eligible for or don't have access to Spinraza

As a young adult who is clinging onto any ounce of independence I may have, it's so important to maintain this before losing it

Those that can't access Spinraza due to medical or practical reasons, those currently not receiving treatment.

Type 2 and 3

Everyone should have access to the drug if it can be given to them.

Anyone who cannot access other treatments (e.g. lumbar puncture issues)

The more severe types should have priority access

Type 1 and 2 (who are more severely affected by SMA) who have not already been able to access treatment

Type 3 who are unable to walk are unable to get treatment at the moment. Also, people who can't be injected with Spinraza.

Type 1

People who are not eligible for Spinraza or Zolgensma (with spinal fusion, scoliosis, etc)

Parents of young people age < 18 years with SMA responses

Anyone who is suffering an impact on their quality of life should receive treatment. Definitely initial focus should be given to those not accessing Spinraza on the MAA or is has lost Spinraza as a treatment on the MAA. Including Type 3a who have lost Spinraza due to not meeting the 5 steps and those who could not access treatment due to when they lost ambulation.

Should be available to all

I think there are a finite amount of type 3s left now as all new SMA diagnosis should be in a category that will get treatment. I am unsure how type 3s can be refused treatment as this seems against united nations human rights. To leave children / adults to fade away while there's something that could at the least arrest progression seems unbelievable in a developed country. Type 3s are not lucky to have been born stronger than other types, they are unlucky to have been born weaker than the general population. They should not be side lined because their lives are not immediately under threat. They deserve equal treatment after inheriting this debilitating condition.

Those not currently receiving any other treatment

I think everyone should get it if they want it, it isn't like there are many people with SMA out there anyway! But if you have to do it in groups, then always type 0 and 1 first, then type 2 and 3, based on severity, I think!

Those who lose Spinraza after 1 year as they did not reach the 5 independent steps milestone within 12 months as per MAA Those who did not qualify for Spinraza.

Babies. Infants. Toddlers. Because of the ease of dosage.

Every living person with spinal muscular atrophy should have priority to improve their quality of life! You can't put a price on that!!

Those who cannot gain or maintain access to Spinraza

Everybody

those that have more severe forms of SMA

People with SMA who have been excluded from Spinraza

SMA 3 AS MISSED OUT ON OTHER DRUGS AVAILABLE

Children/adults who have not been offered any other therapy or trial

I think that everyone with SMA, if they want, they should be allowed to have access to risdiplam.

Others' responses

Type 1 to 4

I think everyone should have the same option - it should not be age determined - if it is a young person - has a whole life ahead to be more independent. If it is an older person – they had struggle long time, surely, they deserve to see and enjoy a future more independently

Those that are not currently receiving any other treatment

Yes. Types 1, 2 and 3 without restriction to be closely followed by Type 4.

Those in whom trial data shows any benefit. Every SMA patient fears each small deterioration in their remaining function whether it be walking or upper limb movement, swallowing or fine motor movement. The gradual progression to complete dependence is upsetting.

The group who have been refused Spinraza after being told they could have it in August 19

Groups of teenagers/ young adults with type 3 or 4 should have priority access as they are approaching an age where their mobility is potentially going to decline. If it's possible to maintain the mobility they currently have in their lower limbs in particular that should be taken advantage of.

Each case should be treated individually