

The impact of not being eligible to access nusinersen treatment on those who have SMA Type 3 and their families

P = Person who has SMA Type 3.

R = Relative of the person who has SMA Type 3.

1. Respondents

	%	Nos.
Young people / adults who have Type 3 SMA themselves	60.5	32
Parents re: children / young people / adults who have SMA Type 3 and have not responded themselves (proxies)	9.5	5
Relatives of children / young people / adults who have SMA Type 3 and have responded themselves	30.0	16
		53

Two surveys were made available to the SMA community via survey monkey: 10th January - 16th February 2020. One was for young people and adults who have SMA Type 3 and are ineligible for access to nusinersen under the current terms of the NHS England Managed Access Agreement. The other was for relatives. Notices and links appeared in SMA UK, MDUK and TreatSMA social media feeds & in SMA UK's January 31st 2020 enews. This report collates the results of both surveys.

2. Which country the person who has SMA Type 3 lives in

	%	Nos.
England	97	36
Northern Ireland	0	0
Scotland	0	0
Wales	3	1
		37

3. Age of the person who has SMA Type 3

	%	Nos.
7 - 9 years	3	1
10 - 12 years	0	0
13 - 15 years	8	3
16 - 18 years	5	2
19 - 28 years	27	10
29 - 38 years	19	7
39 - 48 years	8	3
49 - 58 years	22	8
59 years +	8	3
		37

4. When the clinical diagnosis of SMA Type 3 was given.

	%	Nos.
Diagnosed before 18 months of age	16	6
Diagnosed between 18 months and 3 years of age	32	12
Diagnosed when older than 3 years of age	49	18
Don't know / not sure	3	1
		37

5. Has a clinician assessed the person for nusinersen treatment?

	%	Nos.
Yes	11	4
No	86	32
Don't know	3	1
		37

6. Does the person who has SMA Type 3 have any curving of their spine (scoliosis)? (Tick as many as apply)

	%	Nos.
Yes - had spinal surgery	22	8
Yes - waiting for spinal surgery	3	1
Yes - have a spinal brace	3	1
Yes - but no intervention yet (i.e. none of the above)	30	11
Not sure	0	0
No	43	16
		37

7. What statement best describes why the person who has SMA Type 3 is not eligible for treatment

	%	Nos.
18 years or under and lost ability to walk 5 steps unaided before 24 th July 2018	18	7
18 years or under and never able to walk more than 5 steps without assistance	0	0
19 years or over and unable to walk	58	22
Doctors consider the spinal surgery makes it clinically unsafe to deliver treatment	0	0
Other	24	9
Explain more if you wish	29	11
		37

8. What impact has not being eligible for nusinersen treatment had on the person who has SMA?

	Strongly agree		Agree		Neither agree nor disagree		Disagree		Strongly disagree		Total replies
	%	Nos.	%	Nos.	%	Nos.	%	Nos.	%	Nos.	
It has made them stressed	54	20	22	8	16	6	8	3	0	0	37
It has affected them emotionally	57	21	22	8	14	5	8	3	0	0	37
It has made them anxious	46	17	22	8	19	7	11	4	3	1	37
It has made them angry	57	21	22	8	16	6	5	2	0	0	37
It has affected their day-to-day well-being	42	15	14	5	28	10	14	5	3	1	36

Comments:

P29 (age 29 – 38 years): The thought that I might be able to have the treatment was a big boost for me. I was looking forward to being able to have a life.

P1 (age 19 – 28 years): It has made me feel unworthy, unimportant & neglected.

P23 (age 16 – 18 years): It makes me really angry that those who are stronger than me are eligible for the treatment, those who are weaker than me are eligible for the treatment and yet I am not. Spinraza has been proven to halt progression in my type, so why am I not eligible? This would be enough for me - I'm not looking for major improvements, just a sense of stability so I can carry out my future how I want to live it. My arms are already getting weaker and weaker and so is my breathing and my swallow. It's said there isn't enough benefit to me having the treatment as I wouldn't regain or maintain the ability to walk. But that's not what's important to me! I just want to be able to not choke on my packet of crisps and to be able to lift my cup of tea to my mouth!!! **R18 parent:** devastated.....**R15 Grandparent:** She gets on with things with determination to succeed and do the best in life. I don't think this gives her time for being stressed. **R5 Parent:** She feels like a ticking time bomb that is causing more muscle weakness that maybe permanent the longer she is denied treatment. She feels it is unfair that she cannot walk and neither can type 2s and they get treated. She feels if NICE delays she will then be 18 and declined because she's an adult. She had a gap left specifically, which meant 2 operations and now Bristol says they still cannot use her spine. She is not interested in walking and is annoyed that benefit is measured with this as she just wants to be able to use her arms, swallow and breathe! All more important than walking and should have been measured in trials.

P21 (age 29 – 38 years): I don't tend to get stressed, emotional or anxious at all, so the above responses may need to be given less importance than other people's responses. My slight anger has come from the announcement which stated all patents Type I to III will be assessed for treatment and then the exclusion of Type III patients who haven't walked in over 12 months, with little clarity as to why. I am currently looking into this though, so once further information has been obtained, my feelings could change.

P20 (age 29 – 38 years): If a treatment is available to help me stay at or improve my ability slightly then it is worth it, my condition is only going to get worse so surely trying to stop it, delay it or improve it by any means is better than nothing, I would rather stay the way I am now being able to do some things for myself rather than not be able to do anything at all which is the way it will end up going.

P19 (age 39 – 48 years): Not being eligible for treatment has had a severe impact on my mental health I have been suffering from anxiety and panic attacks (something I've never experienced before) had trouble sleeping and have lost a considerable amount of weight.

P18 (age 16 – 18 years): Been struggling a lot more in recent years with upper body strength and would really like to improve this or at least maintain it.

P16 (age 49 – 58 years): I have fought this condition all my life since diagnosed at age 9. I am on the edge of losing the little function I have left to stand and walk and expect to have to give up work in the next few years if nothing is done to stop the decline in my condition. I have no one to help me with my daily living needs other than a neighbour who cleans for me and helps from time to time with hospital trips when I fall over. Since May 2013 when I fell and broke my knee my employer asked me to use a wheelchair which I had been avoiding for many years. I accepted that and then shortly after that my 6-year-old son was diagnosed with leukaemia and died 3 years later. My wife who knew I had sma when she married me decided she could not cope and has deserted me and divorced me because she can't cope with living with someone like me who needs a wheelchair. I have lost a lot, but I still have my daughter and remain as positive as I can and am still employed and working as hard as I can and managing to shower once or twice a week and ensure we are fed etc. I am now trying to fund the adaption of My bungalow to meet my needs (bathroom etc) but get no help financially. This will help me a lot to manage better, but I really want to also try and get some form of treatment if available please. I never expected a cure in my lifetime but now several are available I am of course wanting to try them to save what function I have and carry on supporting my daughter. I've worked all my life, never been unemployed, always done what I could to help others and now find myself in a position where the IOM health Service does not think it's worth spending money on me. After having lost so much I got so excited about a potential cure to maybe get my life back on track. To be told there is a cure, but you can't have it is unbelievable after I've worked so hard all my life and always paid by NII an Taxes. I've never been unemployed, but I fear if this gets worse, I will be, and I'll have to start claiming incapacity benefit and the state will have to look at care needs for me. That will cost a lot I'm sure so would it not be better to stop my condition so I can continue to work? I get no help from anyone and am appalled at the NHS lack of support on age grounds just because I'm 50 and they don't want to spend money on me.

P15 (age 19 – 28 years): I'd just like any change even if so small and the fact it's been denied is, in my not so expert opinion, a joke.

P11 (age 49 – 58 years): feel worthless, frustrated, a burden

P9 (age 59+ years): As I have progressed my anxiety/panic attacks have become more, in the time since nusinersen has been approved by NICE, I have lost the ability to transfer by myself from my wheelchair/toilet/bed and car! I am fast losing the ability to do any weight bearing at all. This has caused me extreme anxiety; I'm losing any independence that could have been saved.

P8 (age 19 – 28 years): Since i have been denied access to Spinraza my condition has continued to get worse and is taking away my independence

P7 (age 39 – 48 years): This decision has caused severe depression. All my life I have had hope for a drug or therapy, as I deteriorate in function. Even just to stop the deterioration or have a small improvement would have been hugely important. I work full time and thinking about resigning as a direct result of the depression. Since, I lost the ability to walk (within the last 6 months) - I have been referred to 5 other NHS services and along with the specialised equipment I need, it will become very costly to the NHS. I'm sure could have all been avoided if I had been given a drug that would have kept me mobile to some degree. This decision basically caused me to lose the hope I once had, and to see everyone else from different countries thriving (including adults' similar function to me) whilst I continue to deteriorate is difficult. Now I have given up emotionally, put on weight, lost confidence and can't motivate myself anymore, very unlike who I used to be. This has also affected my husband's mental health too, with the extra help and care he is having to undertake and watching me deteriorate.

P6 (age 59+ years): I do not wish to receive this treatment as I am aged 66 and would prefer that younger people can take advantage of it. In addition, I would not choose to receive treatment delivered by the method used.

P2 (age 13 – 15 years): I have been waiting for this treatment for 3 years. My parents told me about it and parents campaigned for it to available. I am getting weaker and want to have treatment to maintain what strength I have left and for an independent future. And do things myself rather than asking for help all the time. I know it Spinraza will make me stronger so I can so continue to finish education and get a job one day
R6 parent: The decision is so Cruel and unfair I believe the criteria is unethical and against human rights. We waited and campaigned for 3 years for Spinraza to be approved and access from all. When it was approved, we were so happy but when the eligibility criteria were introduced, we felt we were dropped disgusted and let Down it was devastating. I told my son it's been approved and then now I can't believe I have to tell him sorry son you can have it it's heart-breaking.

R22 parent (Proxy / age 29 – 38 years): It should be available to everyone who needs it. He is upset he is not eligible for this.

R21 grandparent (Proxy / age 19 – 28 years): It has encouraged him to take risks in an effort to live life to the full. If he falls when alone he telephones for help.

R12 parent (P13 / 13 – 15 years): My son feels discriminated against and cannot understand how other children who are both stronger and weaker are eligible but he is not. This form of discrimination from a national health body is particularly upsetting for someone who deals with low level discrimination daily. He looks to the government and NHS for support.

9. What impact has the decision that the person who has SMA Type 3 is not eligible for nusinersen treatment had on relatives?

	%	Nos
Parent / guardian	67	14
Partner	5	1
Sibling	0	0
Grandparent	19	4
Other relative	10	2
		21

	Strongly Agree		Agree		Neither agree /disagree		Disagree		Strongly Disagree		Total responses
	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	
It has made me stressed	62	13	19	4	14	3	0	0	5	1	21
It has affected me emotionally	67	14	19	4	10	2	0	0	5	1	21
It has made me angry	76	16	10	2	10	2	0	0	5	1	21
It has affected my day-to day well-being	48	10	19	3	19	4	9	2	10	2	21
I am relieved	0	0	0	0	5	1	19	4	76	16	21

Comments:

R22 parent (Proxy / age 29 – 38 years): Very sad he can't have it.
R19 Grandparent (Proxy / age 19 – 28): I am concerned for the welfare of my grandson but not under stress.
R18 parent (P23 / 16 – 18 years): When the authorities made this decision, I think that they forgot about the massive impact that this condition has on the families and friends of the patient. Just a small change in her condition would make a massive difference to our day to day lives.

R15 grandparent: As a family we have longed for treatment for this progressive condition since diagnosis in 2006. Spinal surgery to straighten her spine with rods, following scoliosis left a space for the injection of nusinersen which was eagerly anticipated. Our grandchild is losing upper body strength weekly and her only hope of continuing using her arms to write, draw, cake decorate, hold a drink, feed herself, dress, wash etc. is to have treatment, she is well aware of this. Hope has been taken away from her and a small group of people with SMA, who have been denied treatment, because walking appears to be the ultimate aim. She does not have an expectation to walk again but a hope of some gain in upper body strength and a halt in muscle weakness progression, which ultimately will lead to breathing and swallowing problems as well as arm weakness. This is a young person looking to a future where she will contribute to society, go to university and have a fulfilling successful career. The decision to deny treatment to her takes hope of this future away. She has a very acute awareness of how things will progress and talking to her this week about delaying an education aim for a year her response was " I have to do it now because this is the strongest and most well I will ever be" words that should not be said or thought by a sixteen year old if there is possibility of an alternative. **R14 grandparent** Increasing upper body weakness has made the patient and all the family feel that therapy should not be delayed. We feel a window of opportunity may be missed.

R12 parent (P13 age 13 – 15 years): I find it very upsetting. We have followed the Spinraza drug for over 6 years through the lads and tests and we have helped in workshops and inputs to data collection over the years. for our two children with SMA to be excluded in this way is very stressful and upsetting.

R9 parent (P29 age 29 – 38 years): I find it unacceptable that my son struggles in everyday situations, but they are not willing to help and give him a better standard of life. WHICH EVERYONE DESERVES!!!

R9 parent (P2 age 13 – 15 years): It has been a devastating blow to us as parents the whole family feel for him and us all. Watching your child deteriorate over time is heart-breaking and we feel so desperately helpless. Knowing now the treatment is available makes me feel ill and desperately depressed. We as parents have researched Spinraza and followed and campaigned for access UK. We have waited and finally when the news of it being approved, we were jumping for joy and I told him the exciting news finally a treatment for him, but now I can't face telling him he's not eligible. I believe he is because he needed aids to help him, he may have stepped more than 5 steps but never alone or unaided. He may have walked a few minutes back when he was diagnosed unaided not never with a straight back or one foot in front of the other! His back swayed and walked side to side steps. It's so unfair.

R5 parent (P23 age 16 – 18 years): I feel totally powerless to help or sort this problem. I am watching my bright and independent daughter fade away despite a treatment being available that could stop progression. I cannot get straight answers from clinicians. I have just taken another 4 months unpaid leave from work so she could have bottom screws from fusion replaced. These were loose as the gap left by fusion (for Spinraza) 2.5 years earlier caused instability in lower spine. 2 expensive operations for no reason! A second bout of unpaid leave to look after her. 4 months missed of A levels. A levels must now be done over 3 years with a delay to Uni. More pain and anxiety for our family. More cost to NHS and education. Her ability to give back to society at end of education in question as her mobility increases. This could be stopped.

R4 parent (Proxy / age 48 – 59 years): As a parent I would love for my son to be eligible for treatment.

R13 aunt (P12 age 13 – 15 years & P13 age 13 – 15 years): My family have been hoping for a miracle for 15 years, and we all thought this could be it. Finally, there was some effective treatment. To have hopes raised and then dashed has had a devastating effect on my sister in law especially in terms of her mental and physical health. It has left the parents feeling even more helpless than before. This could make such a difference in so many ways to my niece and nephew.

10. If treatment is made available, would the person want it?

Answer Choices	%	Nos.
Yes	82	31
No	3	1
Maybe	11	4
Don't know	5	2
		38

Comments:

P29 (age 29 – 38 years): My outlook on life would be enhanced. I've read reports that the difference it can make to people with SMA has been quite remarkable. Just to be given the chance for this treatment would be something special.

P21 (age 29 – 38 years): Even a small increase in my muscle mass will provide a significant improvement in my lifestyle.

P17 (age 29 – 38 years): If it helps just keep my strength in my arms so I can keep feeding my self

P16 (age 49 – 58 years): Happy to trial other drugs still in trials phases if Spinraza not seen as best approach for me (would have to travel to /from UK regularly etc for the injections) Perhaps another oral drug or one-off injection style treatment that's are in testing at the moment would be better?

P15 (age 19 – 28 years): 100%!!

P11 (age 49 – 58 years): A treatment is available in my lifetime which will prevent further deterioration and progression of this severe condition and, may even improve my muscle power, physical and emotional wellbeing

P9 (age 59+ years): After years of fighting for treatment, and seeing others benefit, even at older age groups, I would definitely take it.

<p>P8 (age 19 – 28 years): I am completely sure the treatment would Significantly help me. Considering i am on the edge of SMA completely destroying my body i am i need of Spinraza the most. If i am unable to get it soon my condition will continue to get worse and all my abilities will be taken from me. This drug is my only hope</p>
<p>P7 (39 – 48 years): I would travel any distance to receive this treatment. I would consider relocating to Scotland but would need to be certain that they are treating adults before I go.</p>
<p>P6 (age 59+ years): If an alternative method of delivery is offered, I might consider it.</p>
<p>P5 (age 49 – 58 years): Currently everything I do independently is on the limits of my strength. Even a 5% increase of strength would have a huge effect on my quality of life.</p>
<p>P3 (age 49 – 58 years): I'm not sure about injections into my spine, sounds painful. Also, I'm unsure as to how effective it will be as a treatment for me at my age.</p>
<p>P2 (age 13 – 15 years): I want Spinraza now before I get weaker. R6 parent: Most definitely yes and are prepared for the commitment on the treatment.</p>
<p>P1 (age 19 – 28 years): Are the risks too high?</p>
<p>R18 parent (P23 age 16 – 18 years): Yes definitely, she had a gap left when she had spinal fusion to allow future treatment. R14 grandparent: I would like them to have it or failing that an unequivocal statement that it will not benefit their case. The general feeling in the family is that a useful form of treatment is being denied. R5 parent I would travel for it</p>
<p>R4 parent (Proxy / age 48 – 59 years): To stabilise his condition would be marvellous but to improve it would be more than I could ever wish for</p>

11. If access to treatment was possible, what outcome(s) would be expected?

	Yes		Maybe		No		Total replies
	%	Nos.	%	Nos.	%	Nos.	Nos.
Stabilisation of condition	79	30	21	8	0	0	38
Improved upper body strength	50	19	47	18		1	38
Improved lower body strength	30	11	51	19	19	7	37
Other	53	10	37	7	11	2	19

Comments:

P29 (age 29 – 38 years): This would be a chance in a lifetime to experience and be able to do things differently and hopefully enhance my life. Everybody deserves the chance to be able to make a change.

P23 (age 16 – 18 years): Just because Spinraza may not have a big impact on my lower body strength doesn't mean it's not worth me having it. Improved lower body strength wouldn't affect my life half as much as upper body strength improvements. My arm strength is the thing that affects me every minute of the day - I'm already in a wheelchair so it doesn't make much difference if Spinraza helps me stand up for a few seconds, I still couldn't go to the toilet independently. The creative activities that I'm most passionate about require arm strength, not leg strength (such as painting, drawing, cake decorating etc.) I can't imagine not being able to do these things anymore and yet soon I won't have to imagine it because it will be real. Spinraza's benefits have been measured through tests of leg strength which just isn't what is important in daily practical life. I would still use a wheelchair even if I could walk a few paces. **R5 parent** Improved swallowing. Improved breathing, less chest infections, improved constipation, less swelling to legs, less pain as she would move about more in chair, less time off school, less time off work for me, less stress in family. More time for other children.

P22 (age 19 – 28 years): With the improved strength of my body I would hope to gain the ability to stand and possibly walk a few steps unaided which would allow me to gain more independence.

P21 (age 29 – 38 years): Improved efficiency of breathing is an outcome I'd expect. From what I've recently read, I'm not convinced about the outcomes of this drug. It confuses me even more as to the ineligibility of myself and others. Again, I am currently seeking clarification about this.

P19 (age 39 – 48 years): Hopefully improve lung function
P16 (age 49 – 58 years): Stop condition getting worse. Improve strength, mobility and mental situation. Stop worrying about not being able to get out of bed in the morning or get on and of the toilet or take a shower. Feel able to do more and re-engage with life more positively to support my daughter and carry on working. Not lose my house and move into care. Remain independent.
P14 (age 59+ years): It would relieve my 80-year-old wife of tasks such as assisting in my DRESSING, getting in and out of bed and washing.
P13 (age 13 – 15 years): Independence. R1 aunt: Any stabilisation of their condition would be a miracle. We are realistic and any sort of stabilisation or slowing of the effects would make such a difference in their lives
P11 (age 49 – 58 years): Emotional wellbeing
P9 (59+ years): To stop progression would be a miracle! Any other improvements would be very gratefully received!
P8 (age 19 – 28 years): Big improvements would be in swallowing and coughing. R10 parent: All evidence that we have seen across all types of SMA has at the minimum stabilised the condition and, in most cases, improved physical function. This is true across the age spectrum.
P7 (age 39 – 48 years): I would hope for stabilisation as a minimum, and any strength improvement is a bonus. I just wish I had the chance before I lost walking function.
P5 (age 49 – 58 years): As per previous comment. Currently everything I do independently is on the limits of my strength. Even a 5% increase of strength would have a huge effect on my quality of life. R7 partner: My husband is fiercely independent but a lot of daily chores he requires help. A small improvement or stability of condition would make a huge difference to his quality of life and well-being.
P4 (age 19 – 28 years): I would like to stop progress sma
P3 (age 49 – 58 years): hopefully it will help with breathing and apnoea issues and I am experiencing as I'm getting older.
P2 (age 13 – 15 years): I want to get stronger so I can use my hands and arms for day to day life activities like brushing my teeth washing writing using cutlery holding my computer controller. R6 parent: I believe Spinraza will stop the progression of the condition and gain back some strength and This would be miracle/life changing. He had been able to use his arms well but now he can't even hold his arms out and struggling to do simple everyday tasks like brushing his teeth with his arms he moves his head and showering independently. It's not about walking again but worth everything if he could stand in his frame without getting too tired and to turn himself in bed like he used to would be amazing and independent for him as I turn him now it's exhausting. He is an independent kid and hates all the restrictions in his life and he wants to able to go to school and get a job but he's just so tired lately and I can't see he l'll be able to without Spinraza.

R 21 parent (Proxy / age 19 – 28 years): Improved core
R19 grandparent (Proxy / age 19 – 28 years): His ambition is to rise from a chair unaided. To rise from the ground would be a bonus.
R11 parent (P12 age 13 – 15 years): Independence. Brush own hair. Brush own teeth. More stamina to socialise with their friends. Academic to be able to get my books and pens out. Widen career opportunities. Being to start doing exercise. Less reliance on NHS. R1 aunt Hopefully it would go some way to halting the progression and allow my beautiful niece to do basic things like brush her own hair. What we hope for, for her and my nephew is some quality of life, and some hope.

12. Additional Comments

<p>R2 parent (age 7 – 9 years): It was 15th May when we were told that it was access for all and it had been approved, which was just fantastic. We'd just got home from holiday and were putting the boys to bed and to see SMA has treatment was just incredible. We were so happy, such a high. We then decided to tell him because he still knew nothing about treatment. So, we did and told him there was a treatment which would hopefully give him some strength back and he was happy, he cried to think that he might be able to stand with his KAFOs and not have his frame around him. All the hard work he's done to achieve as much as he had, our whole "move it or lose it" attitude we'd had for the last 6 or 7 years we'd been told would pay off because he's got treatment. And then 2 weeks later, just torn apart because of the criteria and that he had lost walking when he was age 3 and he's Type 3 which we always thought was a really positive thing because he walked is now tied around him and he doesn't tick their box and can't walk and he's weak. And he's weak because it's taken so long to get treatment. He's deteriorated because they've taken so long.</p> <p>It was so hard to tell him and dash all his dreams and maybe stop him trying. It hurts a lot but we keep going, we keep saying keep doing your exercises, keep stepping, it's really important to keep going because one day we might get it. It's heart-breaking. They don't see him and the fight he does every day just to do a couple of steps.</p> <p>Obviously, he's 9, his mental health is so important to us. We need to keep him positive and strong, not just physically but mentally as well. It's so important that his mindset is in a good place. He's had struggles in the past with the fact that he can't walk, and he can't stand, and he can't bounce on the bouncy castle the same as his friends and we don't want him to have those worries. But we will stay hopeful that one day there will be a treatment or a trial he can be part of. We will continue to do his exercises and stretching and keep him happy. We just have to remain positive.</p> <p>Meeting other families has always been important to us and there's a core group of five of us who've got to know each other really well, both us parents and our children. Two of the children have Type 2 and are weaker than our son and two have Type 3 and are stronger than him. All</p>

four have now started Spinraza and it doesn't make sense that because he's in the middle he's the only one who can't have treatment. Of course, we're really glad for them, but it makes it even harder for us.

To see everybody else doing so well on their treatment and know that he can't have that is just torturous for a parent. We're keeping his mental health safe by not telling him, but what about our mental health? He can't have treatment because he doesn't tick a box. That's just cruel.

P24 (age 19 – 28 years): It's unfair that we were all given hope at first, and it was stripped away by adding criteria which made a lot of individuals such as myself ineligible for the treatment it has made me feel depressed and it has stripped away the hope I had, I feel it was inhumane in the way it was done, first giving someone hope then shattering that hope in an instant.

P16 (age 49 – 58 years): Thank you for the chance to share my views. I am a very unusual case with very mild SMA type 3b. I am just about to lose the ability to walk in doors which will be devastating. It's amazing I have not done so already given the challenges of the last 6 years but I'm still standing all be it only within my own home. I believe it would be clinically interesting and beneficial to try a drug on me to see if it keeps me walking and keeps me independent. We would know fairly soon if it worked because I'm on the very edge of losing ability to walk now. The benefits are clear - whilst I'm 50 I still have a lot to give to my daughter and society as a whole and treatment would help keep me out of the care system.

P13 (age 13 – 15 years): I feel upset that I cannot get access to treatment others with SMA are having. It would provide me with strength, independence, stop deterioration and preserve my dignity. **R8 grandparent** I have 2 grandchildren with SMA3 aged 13 and 15. The impact on the family after offering hope for improvement and then taking it away has been devastating. Their family life is difficult enough on a day-to-day basis from a practical and emotional basis and there is an impact on the wider family from this and the fall-out of denying some hope for improvement as we all feel the pain! Some hope is better than none at all!

P11 (age 49 – 58 years): I will not be such a financial burden on the health services. Why is my choice/quality of life not considered the same as type 1 and 2 SMAers?

P9 (age 59+ years): I feel it's grossly unfair that part of the SMA community have been 'chosen' to not have access to treatment, especially when the majority of us can see the benefits that so many are getting. No matter what type or how old you are, surely our lives are worth more, if good treatment is there, we should be able to have it. I feel like I'm being punished for having this disease.

P7 (age 39 – 48 years): I am very scared for my future. I am losing power in my arms now and I don't have any support other than my husband. Being denied access to the only drug available, after many years of hope, and knowing that I'm in such a small minority has broken me. I know there will be other better drugs in the future (big assumption they will even be made available to me), but I really needed it 6 months ago, or as soon as possible. I cannot wait another 2-3 years, as I dread how weak I will be by then.

P4 (age 19 – 28): I don't get enough care from my specialist in hospital. When i was asked for a rehabilitation, they said that nothing cannot help me, everything will be without benefits for me. I got proposition that I should takes painkillers if i feel pain in my knee, hip, arms but I have this pain because I don't have any movement. When i have chest infection I am unable to cough and no one care about this. I'm worry that they can also refuse treatment for me.

P3 (age 49 – 58 years): I've never really been in touch with doctors because doctors have never been able to do anything for me as regards my SMA. I am unsure whether anyone will EVER contact me to offer me treatment because I don't really engage with the medical community and I don't know if anybody knows I'm here?

P2 (age 13 – 15): I feel let down by the NHS and nice who have put in cruel eligibility criteria after telling me theirs was access for all types. I am so close to being able to be eligible as I needed aids all my life to help me walk and now more like a type 2 rather than type 3, I can't walk since 8 years. I am feeling depressed and worthless and my life isn't worth the money of the drug. Spinraza would be life changing for me and my future life **R6 parent** I hope this information will be considered as a positive campaign and proof that the eligibility criteria for type 3 is unfair and cruel and unethical and against human rights. For access for treatment for all.

R19 grandparent (age 19 – 28 years): He is in full-time employment. A recent fall resulted in a badly broken arm which needed specialist treatment and a journey to hospital of some 400miles each way. He was unable to work for several weeks. Because of the distance to travel he loses two days' work to attend each hospital appointment. Treatment for SMA could have prevented this fall.

P23 (age 16 – 18 years) parent: I would have liked my daughter to have been eligible for a trial, but none were offered. I feel it is against her human rights that NICE have decided that walking is more precious than swallowing or breathing, washing your own hair or brushing your own teeth. I would happily speak to anyone that could help with them gaining access. **R14 grandparent** Before the latest guidelines several paediatricians expressed their optimism that this patient would benefit from nusinersen. This caused hope and optimism in the family.

R11 parent (P12 age 13 – 15 years): Feel discriminated against and marginalised as I have two children with SMA 3 who did not hit the criteria to receive Spinraza as lost the ability to walk more than a year again. This treatment should be available for all SMA patients without prejudice **R8 grandparent:** I have 2 grandchildren with SMA3 aged 13 and 15. The impact on the family after offering hope for improvement and then taking it away has been devastating. Their family life is difficult enough on a day-to-day basis from a practical and emotional basis and there is an impact on the wider family from this and the fall-out of denying some hope for improvement as we all feel the pain! Some hope is better than none at all!

R10 parent (P8 age 19 – 28 years): It is quite clear that the rationale for denying treatment to some SMA patients is based on arbitrary criteria and can only logically be seen as a cost saving exercise. In our son's case, denying treatment is reducing his independence and will end up not only destroying his and our lives but also result in him becoming a costly burden to the state instead of allowing him to reach his full potential and allow him to give something back to society. This decision is inhumane, cruel and a blight on our health service.