

**Appendix 4.
Surveys January – February 2018
Type 2 / 3 and 3 ages 0 – 17 years
Biggest Challenges**

Type 2 /3 age 0 – 2 years mother

Watching your child lose abilities, falling over suddenly, hearing them scream in the middle of the night that they can't turn over - all the time knowing that there is nothing that can be done as spinraza is not available. Not being able to stop their distress at what is happening to them

Type 3 age 3-4 father

Knowing your lovely little girl's condition is going to get worse until there's nothing left, even though she is a type 3 she will slide into greater disablement. Physically there is a lot of manual handling that is hard now and in the future, will only get worse as she gets bigger and we get older. My wife has to have physio because of all the holding and picking up of our little girl for transfers or otherwise. I usually pick up and or move around / transfer my little girl around 100 times a day. As parents we have become an extension of her body, so she will ask us to dress a doll, pick up a toy off the floor. Because she can walk a little she gets herself in all sorts of trouble, like getting stuck in twisted positions as she has tried to get something off a shelf, floor or table. Practically it is a challenge to do anything, the extra transport and setting up of a wheelchair as well as having another child to look after makes even simple things like going to the super market extremely difficult.

Emotionally.. where to start. myself and my wife have had counselling, our other little girl does not get the attention she deserves because were always running after her little sister. I personally feel a crushing guilt, I have suicidal thoughts, I cry every day, sometimes I hide at work so no one see me. We have so little time for our marriage that it feels I just live with another carer not my wife. I can't remember the last time we laughed, I feel there is not hope, I future plan in my mind over and over so that I can feel secure in so far as we can maintain security for our little girl, because she will be so weak by the time we get older and there will be no one to care for her; it stops me from sleeping. It feels like my soul has been ripped in two and at the same time I have this beautiful little girl smiling at me that does not realise the condition that I have given her.

Type 3 age 3-4 years mother

For my daughter it is physically not being able to do anything for herself and not having her own independence. For myself it's the emotional effects everyday seeing my daughter slowly getting worse. And the struggle of day to day duties whilst caring and looking after her.

Type 3 age 3-4 years grandparent

As grandparents and carers on a part time basis of our granddaughter with SMA type 3 (this is to allow our daughter to work part time as the family as a whole require the extra finance to enable them to live) we are struggling physically. We have to be with her constantly as once her legs give way she is unable to either crawl or get herself up off the floor. Also, we are finding it increasingly difficult to manage to lift and carry her into the car seat /toilet etc. This situation can only get worse as she grows and we age. The only solution we can see for the future is for our daughter to give up working but this will only put more pressure onto the family financially and emotionally causing more stress to them as a whole. It just seems that if she could be treated with the newly developed drugs on offer not only would she benefit but the family too. The emotion well-being would be felt not just by us but for everyone concerned with her care. I know that at present the drugs are expensive but surely this would be cost effective in the long run against health care bills not just for her but all concerned with her care as for instance bad backs with grandparents and mother are already a fact.

Type 3 age 5-12 years mother

Type 3 is a tricky one because they are much more capable than type 1 and 2 and isn't life-threatening. Some of them are near-normal but never normal. And if that's the case the chance of living a normal life is more of a reality. At the moment, all I can project for my son is that he won't be able to take the tube. He has to only take a bus or private vehicles. That really limits his ability to go places. Also, stairs are another important element to type 3. He can definitely take a tube or a bus independently. He can go places his friends go. Because type 3 is well enough to go to school, to be in a normal classroom and participate in PE classes. However, my son always asks when will he be able to jump and run like his friends. The gap is widening the older he gets. Boys are very physical and I am concerned that he won't have anyone to play with if he can't play football or run around, let alone deteriorated and be confined in a wheelchair.

Type 3 age 5-12 years father

The lack of treatment, and every month for illness to progress and take some physical abilities. Would be much better with treatment and rehabilitation. This is unmoral that about the life of so many kids are deciding money, and prolonged procrastination.

Type 3 age 5-12 years mother

Being on a wheelchair referral waiting list for so long. Waiting for possible adaptations to house, ground floor bedroom for son as stairs a hazard. As a parent the emotional stress of watching my son's strength quickly deteriorating is unbearable.

Type 3 age 13-17 years young person

Getting around. - I am gradually getting weaker - I am facing mental issues (depression) due to SMA I feel like I am being mentally tortured knowing there is a possible treatment available sitting on a shelf somewhere but I am being excluded from access to it ... WHY?? How can we allow pharmaceutical companies to hold us to ransom, to imply that they are in the business of making better lives for sufferers of countless diseases whilst making a fortune out of our conditions? It's immoral!!

Type 3 age 13 – 17 years mother

Every day is a challenge for both the person with SMA and the unpaid carer nothing is easy as the world is designed for people with no disability. Emotionally, why us? And no help. Booking trips, everything takes so much research before booking.

Type 3 age 13 – 17 years grandparent

The emotional impact on the family is enormous only surpassed by the physical effort managing the physical needs every single day with little or no respite. Essential surgery has brought a further layer of needs to be met and in the case of my 2 grandchildren with SMA it feels like the constant commitment has not just been doubled but has been 'squared'! To see my daughter's (mother of the 2 children) physical and emotional health deteriorate - with a limit on how to support the whole family - is only what I can describe as heart-breaking....

Type 3 age 13 – 17 years grandparent

My grandson is now unable to walk unaided and uses a wheelchair all the time. He is also slowly losing the strength in his arms. Until the age of 15 he was at least able to walk albeit slowly so you can imagine how frightening it is for the whole family to see how quickly he is deteriorating. It affects us all emotionally, and my grandson physically and practically. He has days when he just can't come to terms with what is happening to him.