

Appendix 7 Those currently receiving nusinersen treatment in England

18 of those replying who are currently receiving nusinersen treatment live in England. Two replies are from parents who can be identified as referring to the same child, so best estimate is that 17 of the children / adults with SMA in England who replied to the survey are currently receiving nusinersen. All of them responded that they would want risdiplam treatment.

Clinical classification	
Type 1	4
Type 2	10
Type 3	3

Age in years	
0 - 4	5
5 - 11	9
12 -17	2
35 - 44	2

Comments

A port will be left for lumbar puncture but no guarantee how effective this will be.

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.

For my daughter, having Spiranza 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. The last two lumbar punctures have been very difficult.....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 Spiranza. From her 3rd injection, 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.

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

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.

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