

Survey 5. Your views on nusinersen treatment, having experienced it in the UK with your child (including any parents/main carers who have since been bereaved)

The Scottish Medicines Consortium (SMC) will shortly be assessing nusinersen (Spinraza™) to decide whether it can be used by NHS Scotland for the treatment of SMA Types 1, 2 and 3.

We have been invited to make a Patient Group Submission (PGS) to the SMC. The charities Muscular Dystrophy UK (MD UK) and the SMA Trust have also been invited to make submissions. We are working with them and also TreatSMA - the campaign group. The SMC will not accept submissions from individuals, therefore people's views have to be expressed via one of the groups invited to make a submission.

As so few children have been treated with nusinersen, the SMC has advised that the views of families whose child received / is receiving treatment within and outside Scotland may be helpful - including any who are bereaved.

This survey is designed to offer you a simple framework for telling us about your experience of nusinersen treatment. It does not seek to gather any clinical measures or evidence. It asks you about the physical, practical and emotional impact of the treatment.

For ease, questions refer to 'your child' throughout.

We will collate all the surveys and summarise what we have been told. The anonymised statistical results of this survey will be used to inform our Patient Submission to the SMC. We would also like to share the information with MDUK, the SMA Trust, TreatSMA and other people in the future who want to understand more about the impact of SMA and family experiences of nusinersen treatment. We will not tell anyone your name or contact details, but we would also like to be able to quote what people have told us. We will not identify you if we do this, but may add non-identifying information that may help readers to understand some of the reasons for what is being said e.g. age of child / grandparent's view. You will be asked at the end of the survey if you consent to this.

The survey should take up to 30 minutes to complete. The closing date for your comments via this survey is January 12th 2018

Thank you.

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* 1. Please tell us who is completing this survey (please tick as many as apply)

- I am the parent / main carer of a child who **is currently being treated with nusinersen** in the UK
- I am the parent / main carer of a child who **was treated with nusinersen** in the UK
- Other - please describe below

Other (please specify)

* 2. Please tell us where you live

- Scotland
- England
- Wales
- Northern Ireland
- Other

* 3. Please give the month and year of birth of your child with SMA (we will not share this specific information)

* 4. Which ethnicity best describes /described your child? (please tick as many as apply)

- | | | |
|---|---|--|
| <input type="checkbox"/> English / Scottish / Welsh / Northern Irish / UK | <input type="checkbox"/> Bangladeshi | <input type="checkbox"/> Arab |
| <input type="checkbox"/> Irish | <input type="checkbox"/> Chinese | <input type="checkbox"/> Any other ethnic group |
| <input type="checkbox"/> Gypsy or Irish Traveller | <input type="checkbox"/> Any other Asian Background | <input type="checkbox"/> Mixed ethnic background |
| <input type="checkbox"/> Any other white background | <input type="checkbox"/> African | <input type="checkbox"/> Prefer not to say |
| <input type="checkbox"/> Indian | <input type="checkbox"/> Caribbean | |
| <input type="checkbox"/> Pakistani | <input type="checkbox"/> Any other Black / African / Caribbean background | |

* 5. Which Type of 5q SMA affects / affected your child?

Type 1

Type 2

Type 1 / 2

* 6. Prior to the diagnosis of SMA, how old was your child when symptoms were noticed? Your answer may be in weeks of age, in months of age.

* 7. How old was your child when SMA was confirmed? Your answer may be in weeks of age, in months of age, in years and months of age.

* 8. How old was your child when they had their first nusinersen injection? Your answer may be in weeks of age, in months of age, in years and months of age.

* 9. What was the date when your child received their first nusinersen injection? Date or Month and Year

* 10. Are you bereaved by the loss of your child?

No - please go to question 11

Yes - please go to question 21

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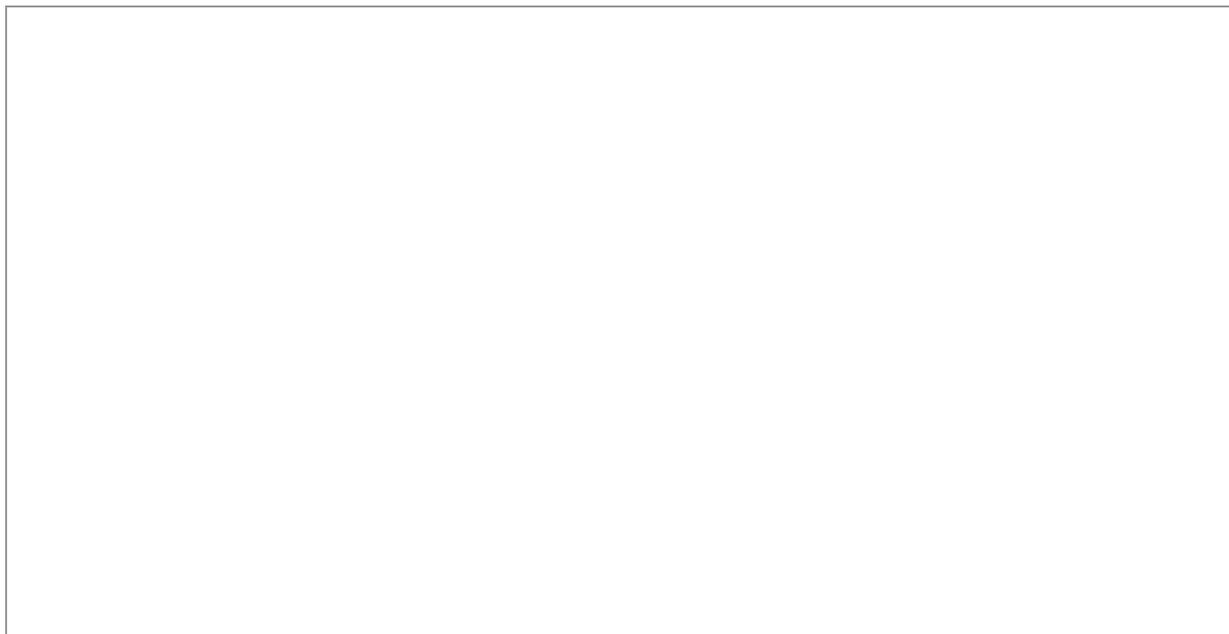
11. Is your child still being treated with nusinersen?

- Yes - please go to Q.12
- No - please go to Q.17

12. How many nusinersen injections in total has your child had as of 31st December 2017?

13. In your view, what impact is the nusinersen treatment having **on your child** physically, emotionally and practically?

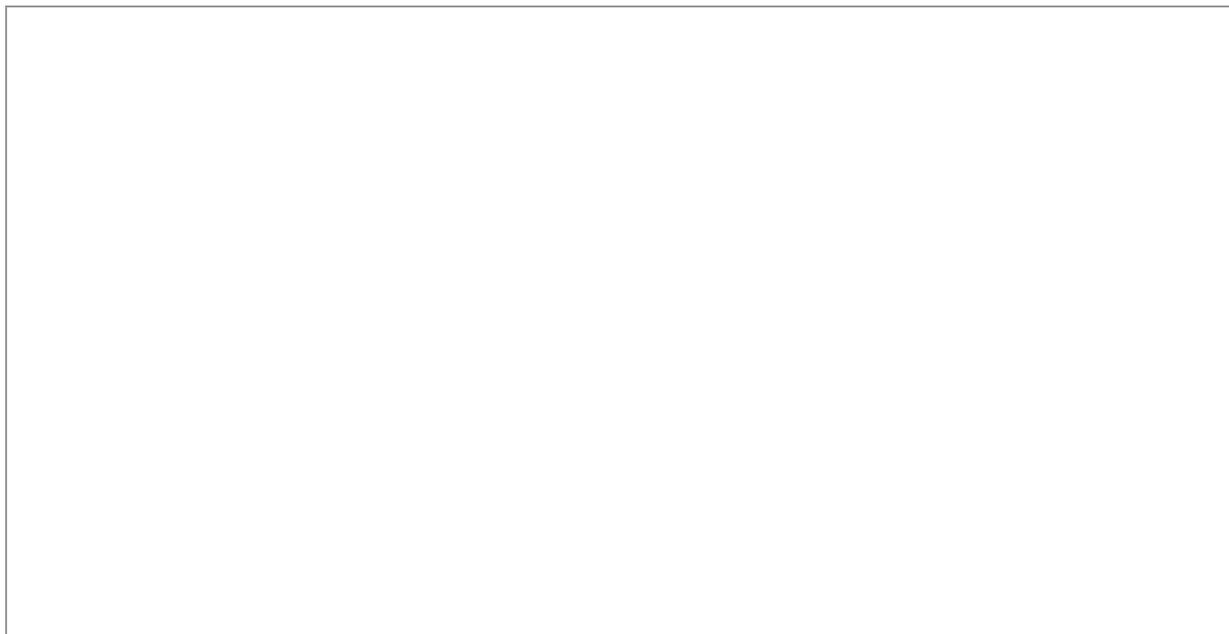
14. What impact is the nusinersen treatment having **on you and other people who help care for your child** - physically, emotionally and practically?



15. Going forward, please tell us about your expectations of further nusinersen treatment for your child.



16. Is there anything you have heard or read about nusinersen that concerns you in any way? (please then go to question 24)

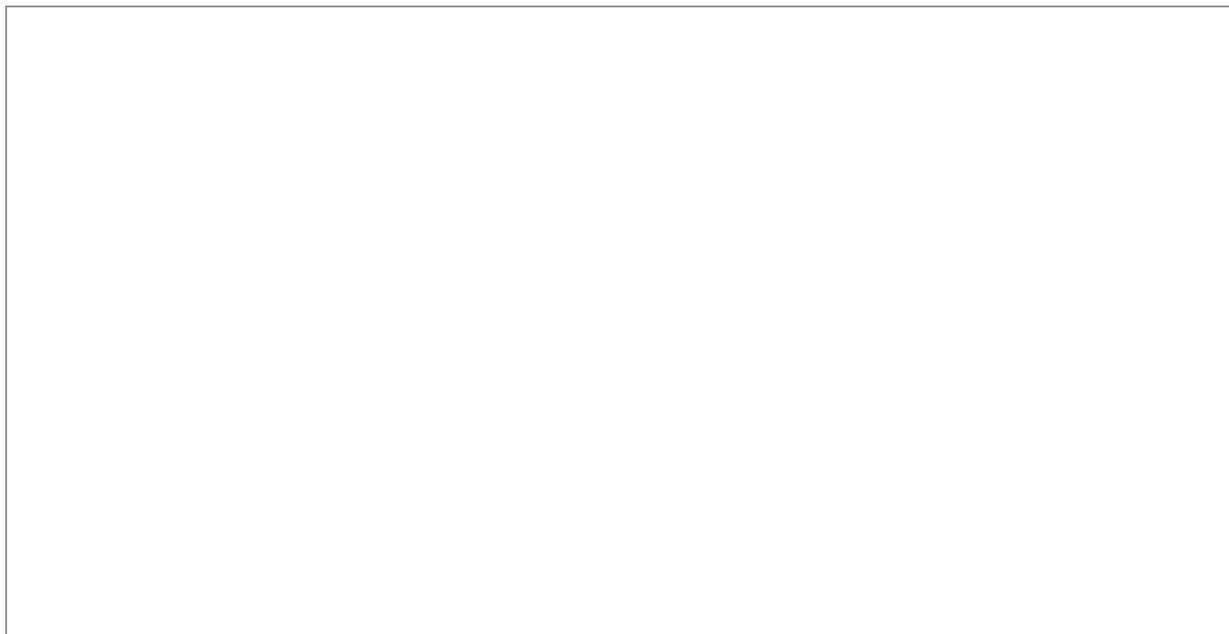
A large, empty rectangular box with a thin black border, intended for the respondent to write their answer to question 16. The box is positioned below the question text and occupies a significant portion of the page's vertical space.

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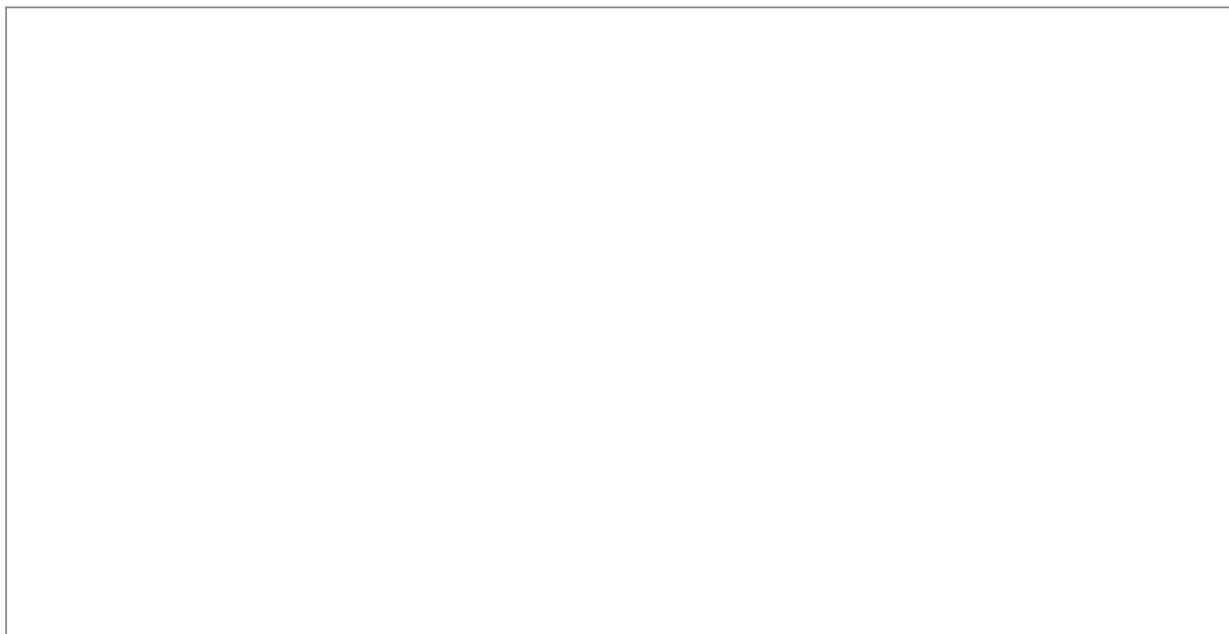
17. How many nusinersen injections did your child have before treatment stopped?

18. In your view, what impact did the nusinersen treatment have **on your child** physically, emotionally and practically?

19. What impact did the nusinersen treatment have **on you and other people who helped care for your child** - physically, emotionally and practically?



20. Is there anything you have heard or read about nusinersen that concerns you in any way? (Please then go to question 24)

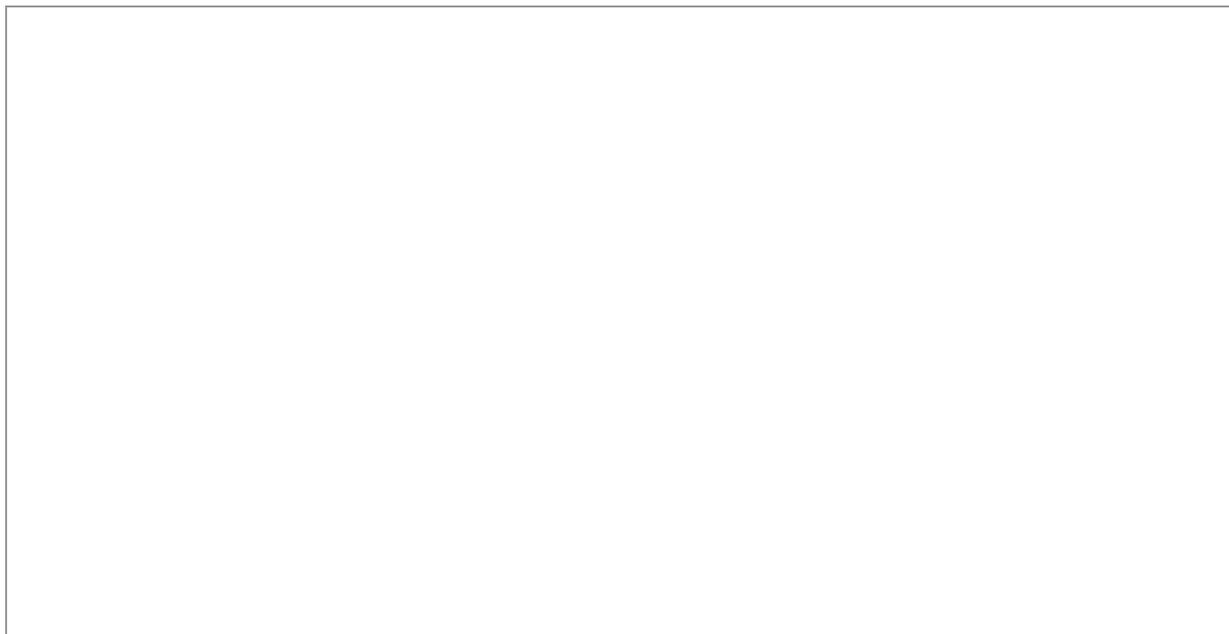


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21. How many nusinersen injections did your child have?

22. In your view, what impact did the nusinersen treatment have **on your child** physically, emotionally and practically?

23. What impact did the nusinersen treatment have **on you and other people who helped care for your child** - physically, emotionally and practically?



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24. Please add anything else here that you want the SMC to know about SMA and your views on whether NHS Scotland should fund nusinersen for all with 5q SMA Types 1, 2 and 3.

* 25. The anonymised statistical results of this survey will be used to inform our Patient Submission to the SMC. We would also like to share this information with MDUK, the SMA Trust, TreatSMA and other people in the future who want to understand more about the impact of SMA. We will not tell anyone your name or contact details, but we would also like to be able to quote what people have told us. We will not identify you if we do this but may add what Type of SMA / age or other non-identifying information is helpful for readers to understand some of the reasons for what is being said. Please tell us if you are willing for us to include what you have written in this survey.

- Yes, I am willing for you to use the comments I have written
- No, please don't use the comments I have written

* 26. We may wish to contact you to ask further questions about your responses. Do you consent for us to do this?

- Yes - please give us your contact details when you get to Q 28
- No

27. If you are willing to say who you are, please give your name and contact details here. We will not share this information.

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Thank you very much for completing the survey.

A copy of our submission will be made available to the SMC. We also plan to publish it on our website. We will notify people of this posting via our monthly e-news. If you are not already on the mailing list, you can sign up at: www.smasupportuk.org.uk/sign-up-for-mailings

If you want to talk further about any issues this has raised for you, please contact supportservices@smasupportuk.org.uk or phone us on 01789 267520. Our office is closed from 3.30pm on Thursday 21st December and re-opens at 9.00am on Tuesday 2nd January.