

What is your view of the possibility of the NHS funding access to onasemnogene abeparvovec (also known as Zolgensma) for the treatment of infants diagnosed with SMA Type 1?

The National Institute for Health and Care Excellence (NICE) is considering whether to recommend that NHS England funds the new drug treatment onasemnogene abeparvovec (also known as Zolgensma™) for infants who have SMA Type 1

Patient Groups would like to find out what the views are of the SMA community and are putting out this survey for anyone who has SMA or is related to someone who has, or had, SMA.

SMA UK will collate all the surveys, summarise what we have been told and share the results with MDUK and TreatSMA. The anonymised statistical results of the survey may then be used to inform our Patient Group Submissions to NICE.

Some questions about nusinersen (Spinraza™) are included as the submission form also asks what people think about current treatments and care.

For ease of reading, throughout this survey we refer to onasemnogene abeparvovec by its trade name Zolgensma and to nusinersen by its trade name Spinraza.

We would also like to be able to quote what people have told us. We may include non-identifying information to show the age / type of SMA that affects the person. You will be asked at the end of the survey whether you consent to this. Whatever your decision, SMA UK will not tell anyone your name or contact details.

If you haven't already done so, please open the accompanying [Drug Treatments Summary Table](#) as you'll need to refer to this as you respond.

The survey should take around 20 minutes to complete, depending on how much detail you wish to include in your comments. You will need to complete it in one go as the survey doesn't allow for breaks.

More than one person from any family is welcome to submit their views - please could each person complete a separate survey.

The closing date for this survey is 11.00pm Sunday 14th July 2019.

Thank you.

What is your view of the possibility of the NHS funding access to onasemnogene abeparovvec (also known as Zolgensma) for the treatment of infants diagnosed with SMA Type 1?

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

- I am an adult (age 18 years +) who has SMA
- I am a young person (age under 18 years) who has SMA
- I am the parent of someone who has SMA
- I am the grandparent of someone who has SMA
- I am the partner of someone who has SMA
- I am related in another way to someone who has SMA
- I am bereaved by SMA
- Other - please say more in the comments box

Other (please tell us more)

*** 2. Which ethnicity best describes the person who has / had SMA? (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 | |

*** 3. Where does / did the person with SMA live?**

- England
- Scotland
- Wales
- Northern Ireland
- Other - please say more in the comments box

Other (please tell us more)

*** 4. Which Type of SMA affects or affected you or the person with SMA?**

Type 1

Type 3

Type 1 / 2

Type 4

Type 2

Other - please describe below

Type 2 / 3

Other (please describe)

5. If you are bereaved by SMA, when were you bereaved? Please then go to Q 6.

2015 - 2019

2010 - 2014

2005 - 2009

2000 - 2004

Before 2000

Any further comment

6. What age is the person who has SMA?

0-4

5 - 11

12 - 17

18-24

25-34

35-44

45-54

55-64

65+

7. Is the person with SMA receiving / have they received / did they receive any drug treatments?

- Continuing to receive **Spinraza**
- Previously received **Spinraza** (if you wish, please give reason for stopping in comment box)
- Received **Zolgensma** via a clinical trial
- Continuing to receive **risdiplam** via a clinical trial
- Previously received **risdiplam** via a clinical trial (if you wish, please give reason for stopping in comment box)
- Continuing to receive **other drug treatment** via clinical trial (please tell us more in comment box)
- Previously received **other drug treatment** via clinical trial (please tell us more in comment box)
- Never** received any drug treatment

Other information you wish to give

8. Please read the information we have provided in the Drugs Treatments Summary Table about **Spinraza™ and **Zolgensma™** and confirm you have done this (by ticking the box below) before answering the next questions.**

- I confirm that I have read the information provided

9. Referring to the information in the Drug Treatments Summary Table, consider the following aspects of **Zolgensma™** treatment for an infant newly diagnosed with SMA Type 1. Please rate whether you consider each aspect to be an **advantage / disadvantage** of this treatment.

1 means you consider this to be a **strong disadvantage** of **Zolgensma™** treatment

5 means you consider this to be a **strong advantage** of **Zolgensma™** treatment

	1 Strong Disadvantage	2	3	4	5 Strong Advantage
How it is delivered	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
How often it is delivered	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The range of cells and tissues it reaches in the body	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The long term effects on the patient's genetic make-up	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The potential risks and how these are managed	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

	1 Strong Disadvantage	2	3	4	5 Strong Advantage
Based on clinical trial results, possible effect on survival / life expectancy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Based on clinical trial results, possible effect on breathing	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Based on clinical trial results, possible effect on motor milestones	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
What is known about this technology	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Additional comments and / or any other aspects of Zolgensma treatment which you consider to be advantages or disadvantages

10. Consider the information about **Zolgensma™** treatment as a whole. Please rate **how acceptable** it is in your view as a treatment for an infant newly diagnosed with SMA Type 1.

1 means **Zolgensma™** treatment is **totally unacceptable to you**

5 means **Zolgensma™** treatment is **totally acceptable to you**

1	2	3	4	5
Totally unacceptable				Totally acceptable
<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Additional comments and / or any other aspects of Zolgensma treatment that are acceptable or not acceptable to you

11. Referring to the information in the **summary**, consider the following aspects of **Spinraza™** treatment for an infant newly diagnosed with SMA Type 1. Please rate whether you consider each aspect to be an **advantage / disadvantage** of this treatment.

1 means you consider this to be a **strong disadvantage of Spinraza™** treatment

5 means you consider this to be a **strong advantage of Spinraza™** treatment

	1	2	3	4	5
	Strong Disadvantage				Strong Advantage
How it is delivered	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
How often it is delivered	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The range of cells and tissues it reaches in the body	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The long term effects on the patient's genetic make-up	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
The potential risks and how these are managed	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

	1 Strong Disadvantage	2	3	4	5 Strong Advantage
Based on clinical trial results, possible effect on survival / life expectancy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Based on clinical trial results, possible effect on breathing	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Based on clinical trial results, possible effect on motor milestones	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
What is known about this technology	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Additional comments and / or any other aspects of Spinraza treatment which you consider to be advantages or disadvantages

12. Consider the information about **Spinraza™ treatment** as a whole. Please rate **how acceptable** it is in your view as a treatment for an infant newly diagnosed with SMA Type 1

1 means **Spinraza™ treatment** is **totally unacceptable** to you

5 means **Spinraza™ treatment** is **totally acceptable** to you

1				5
Totally Unacceptable	2	3	4	Totally Acceptable
<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Additional comments and / or any other aspects of Spinraza treatment that are acceptable or not acceptable to you

13. Thinking about the information you have read in the summary and your answers to Questions 9, 10, 11 and 12, please consider an infant newly diagnosed with SMA Type 1

- **who is well enough to receive Spinraza™**

and

- **who is well enough to receive, and has been screened as suitable for, Zolgensma™ treatment**

Faced with a decision of choosing **one treatment only**, which of the following would you select? (tick one only)

Spinraza

Zolgensma

Neither - I would choose management as outlined in the international Standards of Care for SMA and palliative care

Other (please tell us more / explain your choice if you wish)

14. If there is any more you would like to share about your view of **Zolgensma™** as a potential treatment for infants who have SMA Type 1, please tell us here

15. The anonymised statistical results of this survey will be collated by SMA UK and shared with MDUK and TreatSMA and may be included in our Patient Submissions to NICE. We will not tell anyone your name or contact details, but we would like to be able to quote and share comments people have made. We will not identify you if we do this but may include non-identifying information that may help readers to understand some of the reasons for what is being said e.g. age / Type of SMA. Please tell us if you are willing for us to include what you have written in this survey.

Yes, you may use my comments in the way you describe

No, you may not use my comments in the way you describe

16. If you are willing to say who you are, please give your name and contact details here. This is so that we can contact you to discuss your responses further if we need to. Your contact information will not appear in the summary report or be passed on. The views you have expressed will not affect any services you receive from SMA UK.

First Name

Surname

Address

Address 2

City/Town

County

Postcode

Country

Email Address

Phone Number

What is your view of the possibility of the NHS funding access to onasemnogene abeparovvec (also known as Zolgensma) for the treatment of infants diagnosed with SMA Type 1?

Thank you very much for sharing your views.

The overall results of the consultation will be published on SMA UK's 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.smauk.org.uk/sign-up-for-mailings

If you want to talk further about any issues this has raised for you, please contact supportservices@smauk.org.uk or phone us on 01789 267520.