NICE National Institute for Health and Care Excellence

Onasemnogene abeparvovec for treating spinal muscular atrophy [ID1473]

Consultation on the evaluation consultation document – deadline for comments 5pm on Tuesday 6 April 2021.email: NICE DOCS

	Please read the checklist for submitting comments at the end of this form. We cannot accept forms that are not filled in correctly. The Appraisal Committee is interested in receiving comments on the following: • has all of the relevant evidence been taken into account? • are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence? • are the provisional recommendations sound and a suitable basis for guidance to the NHS? NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the preliminary recommendations may need changing in order to meet these aims. In particular, please tell us if the preliminary recommendations: • could have a different impact on people protected by the equality legislation than on the wider population, for example by making it more difficult in practice for a specific group to access the technology; • could have any adverse impact on people with a particular disability or disabilities. Please provide any relevant information or data you have regarding such impacts and how they could be avoided or reduced**.**
Organisation name – Stakeholder or respondent (if you are responding as an individual rather than a registered stakeholder please leave blank):	[**Spinal Muscular Atrophy UK**]
Disclosure Please disclose any past or current, direct or indirect links to, or funding from, the tobacco industry.	[**None**]
Name of commentator person completing form:	[**Liz Ryburn**]

Comment number	**Comments** Insert each comment in a new row.

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	Do not paste other tables into this table, because your comments could get lost – type directly into this table.
Example 1	We are concerned that this recommendation may imply that ……..
1	We thank NICE for the time and careful consideration that has clearly gone into decision making about this important new treatment that offers such potential for the future.
2	We welcome the recommendations as ones that follow NICE's processes and consider clinical trial evidence submitted by the Company. We note that this is currently limited to infants with a clinical diagnosis of SMA Type 1 who were six months and under, and to a study of pre-symptomatic infants that is still at an early stage. We also note the Company's economic modelling focuses on these same groups.
3	Given this evidence, we consider the recommendations outlined in 1.1 to be a sound and suitable basis for the option of using onasemnogene abeparvovec for those who are clinically diagnosed with SMA Type 1 and described as eligible.
4	We welcome the committee's recognition of the clinical skills and experience of our clinicians who, it is recommended in 1.2, will form a national multidisciplinary team (MDT) to develop auditable criteria for babies age 7 – 12 months.
5	We note that it was acknowledged in 4.14 that diagnosis may be delayed in some disadvantaged groups, and that this was one of the factors that led the committee to recommend treatment for infants who have SMA Type 1 and are between 7 and 12 months of age. The ongoing work to raise awareness of the symptoms of SMA and the possibility of treatment, will hopefully result in earlier diagnosis. Along with this, patient groups, clinicians and pharmaceutical companies are focusing on the need to ensure the earliest possible introduction of newborn screening for SMA. We are therefore comfortable that the upper age limit of 12 months will ensure the possibility of treatment for the current and future incident SMA Type 1 population. Given that NHS England's parallel agreement will enable some young children with SMA Type 1 who are older than 12 months to also be considered for treatment via the MDT, we are comfortable that the best safe treatment options for children in the prevalent Type 1 population will be considered individually with their families
6	We acknowledge the additional work and responsibility that will fall on members of the MDT and hope that they will be well supported and resourced. We are keen, in view of the earliest possible treatment being so vital, to see clinical criteria and processes developed that will, as far as possible, enable quick decisions that are sensitively and transparently relayed to individual families.
7	We also welcome the recommendation in 1.3 of a managed access agreement for presymptomatic babies who meet the criteria which have been set. We are pleased that the further results due from the Company's clinical trial will be a key source of clinical effectiveness evidence.
8	We continue to raise the points made in 4.17, that clinical experts expect that this treatment could also provide health benefits for a proportion of children with SMA Type 2 and 3 SMA and that these health gains would be highly valued by those children and their carers. We acknowledge that no evidence or modelling was presented to NICE to support this and urge the Company to continue to work to address this gap in access to onasemnogene abeparvovec.

Insert extra rows as needed

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Checklist for submitting comments

- Use this comment form and submit it as a Word document (not a PDF).
- Complete the disclosure about links with, or funding from, the tobacco industry.
- Combine all comments from your organisation into 1 response. We cannot accept more than 1 set of comments from each organisation.
- Do not paste other tables into this table – type directly into the table.
- Please underline all confidential information, and separately highlight information that is submitted under 'commercial in confidence' in turquoise and all information submitted under 'academic in confidence' in yellow. If confidential information is submitted, please also send a 2nd version of your comment with that information replaced with the following text: 'academic / commercial in confidence information removed'. See the Guide to the processes of technology appraisal (section 3.1.23 to 3.1.29) for more information.
- Do not include medical information about yourself or another person from which you or the person could be identified.
- Do not use abbreviations
- Do not include attachments such as research articles, letters or leaflets. For copyright reasons, we will have to return comments forms that have attachments without reading them. You can resubmit your comments form without attachments, it must send it by the deadline.
- If you have received agreement from NICE to submit additional evidence with your comments on the appraisal consultation document, please submit these separately.

Note: We reserve the right to summarise and edit comments received during consultations, or not to publish them at all, if we consider the comments are too long, or publication would be unlawful or otherwise inappropriate.

Comments received during our consultations are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the comments we received, and are not endorsed by NICE, its officers or advisory committees.

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