NICE National Institute for Health and Care Excellence

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Sent by email only

3 September 2018

Our ref EH95974

Dear Professor Muntoni and Dr Quinlivan

Re: nusinersen NICE technology appraisal for Spinal Muscular Atrophy

Thank you for your letter dated 20 August 2018, raising concerns about the appraisal of nusinersen for spinal muscular atrophy (ID1069).

I acknowledge your frustrations and concerns about the time it is taking to complete this appraisal.

The technology appraisal process is designed to provide recommendations, in the form of NICE guidance, on the use of new and existing medicines, products and treatments in the NHS.

NICE seeks relevant evidence from several sources. The company submits the principal evidence. The evidence review group (ERG), an external academic organisation independent of NICE, produces a review of the evidence submission. Consultees provide information and selected clinical experts, NHS commissioning experts and patient experts also give evidence.

We base our recommendations on a review of clinical and economic evidence:

- Clinical evidence shows how well the medicine or treatment works.
- Economic evidence shows how well the medicine or treatment works in relation to how much it costs the NHS - does it represent value for money?

Nusinersen did not meet all of the topic selection criteria (which can be found listed on our website) to allow access to the highly specialised technologies process. The topic did not meet 4/7 required criteria. Therefore, the NICE technology appraisal process was deemed the most appropriate route to appraise this product.

The appraisal committee have now met and considered the evidence presented to them and an appraisal consultation document has been produced. The company, NHS England and other stakeholders (including the public) now have an opportunity to comment on this. In addition, stakeholders can provide any further evidence that may go some way to address the concerns the committee highlighted in the released documentation.

Alongside this, NICE have met with Biogen colleagues and are undertaking further negotiations on how this technology can proceed through to the next stage. These

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discussions have focussed on how Biogen can respond to the concerns outlined by the committee that can then be translated into a dialogue with NHS England.

As a result of these conversations, NICE have asked stakeholders to comment on whether there is a clinically distinct subgroup of people in whom nusinersen is expected to be more clinically effective, and how this group could be identified in clinical practice. This consultation launched on 14 August 2018. We would appreciate your input into this question by the consultation deadline of **5 September 2018**.

You could choose to collaborate with one of the national organisations that are involved in the appraisal (listed on our website) to provide a response, or you can respond via the consultation facility on the website. I appreciate this leaves a very short period of time in which to formally submit your comments in this way (if you have not already done so), and am sorry that we are not able to extend the consultation deadline.

The committee will be meeting again on 23 October 2018 to consider the consultation comments, and progress made by Biogen and NHS England.

The process is designed to provide consistent decisions for all technologies and populations.

Yours sincerely,

Sir David Haslam
Chair, National Institute for Health and Care Excellence