

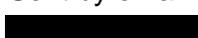


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01 November 2017

Dear Gennadiy,

Re: Nusinersen expanded access programme (EAP).

Thank you for your letter of 19th October asking us to look again at our policy statement supporting the Biogen Extended Access Programme for nusinersen in spinal muscular atrophy (SMA) Type 1. I am aware that you have seen the letter sent by Dr Jessop on my behalf to Professor Muntoni but it may be helpful if I reiterate some of its content.

You have asked NHS England to amend its urgent clinical commissioning policy statement on nusinersen use in SMA1 to broaden the clinical indications in respect of SMN2 copy number and age. The nusinersen urgent clinical commissioning policy statement was consistent with the evidence from patients who were entered into the CS3B 'ENDEAR' trial. The outcomes were considered to be of a magnitude, the research sufficiently robust and nature of the condition such that an urgent decision to commission and invest in this treatment outside the prioritisation process could be made.

The clinical trial was designed to evaluate the efficacy of treatment for infants with 2 copies of the SMN2 and an onset of symptoms 6 months or younger. We now have the published paper and I have included below the wording from that publication.

series.¹² We report the final results of the ENDEAR trial, a 13-month, international, randomized, multicenter, sham-controlled, phase 3 trial that assessed the clinical efficacy and safety of nusinersen in infants who had received a genetic diagnosis of spinal muscular atrophy, had two copies of *SMN2* (which is subject to copy-number variation), and had had onset of symptoms at 6 months of age or younger.⁷

Expanding the criteria for the use of nusinersen in SMA1 (for example in respect of *SMN2* copy number and age) can be considered for commissioning through the development of a further clinical commissioning policy for nusinersen. As you know, nusinersen is being provided by Biogen for NHS patients in England under its expanded access programme. NHS England's policy is conditional on the continuation of that programme.

NHS England is always willing to consider new evidence, but of course evidence must be carefully assessed. Our Clinical Panel is constituted to carry out that task and advise NHS England on whether the evidence is rigorous enough to form or amend our commissioning policies. We know that in very rare conditions the evidence may be sparse and are familiar with the evaluation of such evidence. We do however require that any evidence is fully presented and so do not accept, for example, poster presentations or abstracts: these formats simply do not have enough detail for proper evaluation.

In practical terms, the first step in considering new evidence is for the clinicians who have care of patients to submit it to us. I have asked Dr Jessop to make contact urgently with Professor Muntoni to take this forward.

Yours sincerely,

A handwritten signature in black ink, appearing to read 'J Palmer', written in a cursive style.

James Palmer

Medical Director Specialised Services, NHS England