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Professor Francesco Muntoni
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By email

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Dear Francesco

Nusinersen for spinal muscular atrophy

I have discussed your letter of 23rd August with James Palmer, Medical Director Specialised Services, and he has asked me to respond. I apologise for the delay.

You have asked NHS England to amend its urgent clinical commissioning policy statement on nusinersen to broaden the clinical indications in respect of SMN2 copy number and age. This urgent clinical commissioning policy statement was issued because 'Nusinersen to treat children with SMA which is genetically confirmed and clinically Type 1 is of such significant clinical importance that an immediate clinical commissioning policy statement has been adopted. The time taken to develop a full clinical commissioning policy proposition for relative prioritisation and implementation would not meet the immediate need for patients, clinicians and the NHS to have clarity about whether an intervention is or is not routinely commissioned.' NHS England aims to make considered prioritisation decisions through its prioritisation rounds that take place every 6 months in order to make the very best choices it can about resources for patients using the NHS. 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. Please understand that further potential clinical indications for nusinersen can be considered for commissioning through the development of a further clinical commissioning policy for nusinersen. This may then be considered through relative prioritisation or may result in an urgent clinical commissioning policy statement. The factors taken into account to determine whether an urgent clinical commissioning policy is agreed include; clinical urgency, strength of the evidence, degree of benefit and cost benefit and whether the policy represents a priority as high or higher priority than other agreed service developments.

NHS England is always willing to consider developing new clinical commissioning policy and does so where there is relevant, appropriate published research evidence. The first step is to submit a Preliminary Policy Proposition for consideration by the NHS England specialised commissioning clinical panel (I will advise you separately how to do this) and this also requires the submission of the

supporting published evidence.

I note that the NICE is working on nusinersen with a suggested remit 'To appraise the clinical and cost effectiveness of nusinersen within its marketing authorisation for treating spinal muscular atrophy'. The time scale for this technology appraisal is not yet clear, although NHS England tries to avoid duplication of work with NICE where the timings are suitable.

Best wishes

edmund

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Public health adviser

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