Dear Mr Revel

Duchenne Muscular Dystrophy

Thank you for your letter of 13 February concerning the timing of NHS England’s funding decision for Ataluren in the treatment of Duchenne Muscular Dystrophy.

I do appreciate that any delay in reaching a decision on whether funding might be routinely provided by NHS England in 2015/16 will be of real concern to families hoping to benefit from the drug and we note the public vigil held outside Skipton House on 11 February 2015. Four of my colleagues also had the opportunity to speak to parents in person at a recent meeting on 17 February 2015. At this meeting, they explained that NHS England has committed to completing its current public consultation on its funding prioritisation process (please see: https://www.engage.england.nhs.uk/consultation/investing-in-specialised-commissioning) prior to considering a range of specialised services proposals that would require additional investment in 2015/16. This is because Ataluren is only one of a number of proposals under consideration and it is important that fair and due consideration is given to each proposal. The process adopted needs to be sufficiently robust to minimise the potential for further stakeholder challenge that might further delay implementation and the consultation process is an important part of this.

The consultation will close on 27 April and NHS England will need to fully consider the responses made and finalise its prioritisation process accordingly before the Clinical
Priorities Advisory Group (CPAG) can consider its recommendations in respect of these potential investments. This is why the Ataluren proposal will unfortunately not be able to be considered until a few weeks into the new financial year rather than in, or ahead of, April 2015.

In respect of the specific queries in your letter: CPAG is likely to want to consider the timing of the likely guidance that will result from the NICE HST review of Ataluren. NHS England has an obligation to meet the requirements of HST guidance and as such would not normally look to agree or develop policy in parallel where definitive HST guidance is either available or pending. However, CPAG will consider the likely timing of the review being completed in reaching its recommendations.

I can confirm that NHS England has not set aside an indicative budget for Ataluren in 2015/16 (nor any of the other potential 2015/16 specialised services investments under consideration) in advance of the completion of the funding prioritisation decision making process. It is therefore regrettably not possible to provide early or differential access to NHS England funding for Ataluren ahead of the prioritisation process being completed. We have however agreed the arrangements with PTC Therapeutics for the continuation of funded treatment for all individuals who have been part of the drugs trial and for whom there is agreement of ongoing clinical benefit.

As you have indicated, NHS England has made reference in its public consultation proposals for putting in place a process linked to a first to fourth order sequence. As the consultation process has not yet been completed, NHS England has not considered Ataluren against any of the principles set out in the consultation document.

With regard to your statement about the ‘score card’, as part of the consultation exercise on prioritisation, NHS England is seeking to ensure that the principles and characteristics for making decisions on investing in specialised services are well informed, evidence led and in line with the expectations of patients and the public. We would welcome your comments and observations as part of that process.

I note that the IFR option was discussed at the vigil on 11 February 2015 and it might therefore be helpful to provide some more information about this mechanism. NHS England’s Individual Funding Request (IFR) process is designed to respond to cases where a clinician believes that there are genuinely clinically exceptional circumstances in

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an individual case such that there would be likely to be significant benefit in that individual receiving treatment compared to the majority of patients for whom that treatment might be considered, but would not currently be routinely funded by the NHS. In these circumstances an IFR request can be considered for treatment with Ataluren.

However, if requests are received for an individual who is more clinically typical of the wider group of patients for whom Ataluren would be considered, regrettably these would be rejected as unsuitable for the IFR mechanism as they would be considered applications relating to a wider group or cohort of patients. For these cohorts of patients, a single consistent funding (clinical commissioning) policy needs to be considered to ensure fair and equal treatment for everyone and this is the route, as you know, that is being followed for Ataluren. The IFR mechanism should therefore not be considered as a mechanism to try to achieve access to funding for a wider group of patients; I am conscious that this can cause additional distress to families whose expectations can be unfairly raised through a misunderstanding of the limitations of the IFR process.

Yours sincerely,

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National Medical Director
NHS England

cc: Mr Richard Jeavons, Director of Commissioning Specialised Services
Mr James Palmer, Clinical Director of Specialised Services