



To:

Terry O'Regan (Vice President & Managing Director, Biogen UK and Ireland)
Alessandro Marcuzzi (Senior Director & Head of SMA Europe and Canada)
Caren Deardorf (Senior Director & Global Brand Lead)

28th August 2018

Dear Directors,

Thank you for your community statement following the publication of NICE's Appraisal Consultation Document (ACD) which has not recommended Spinraza for use on the NHS in England.

This is heart-breaking news for families and those living with this devastating condition. We welcome Biogen's early engagement with NHS England and NICE to discuss a proposed Managed Access Agreement (MAA) and are encouraged to hear that further meetings are planned over the forthcoming weeks to find a solution.

Whilst NICE has acknowledged the evidence that Spinraza is clinically effective and provides important health benefits for patients with early and later-onset SMA, the price of Spinraza is a significant barrier at the present time, as NICE clearly stated, "*the cost of nusinersen is too high for it to be considered a cost-effective use of NHS resources*", also outlining "*There are also important limitations and uncertainties in the economic evidence. The most plausible cost-effectiveness estimates, based on the list price of nusinersen, are likely to be between £400,000 and £600,000 per quality-adjusted life year gained but may be higher*".

There needs to be movement from both Biogen's and NHS England's current position in order to reach a successful outcome to the MAA discussions and NICE has provided a platform for that to happen, "*Although the committee recognised that a managed access arrangement could reduce the risk to the NHS, the ICER for nusinersen would need to plausibly be within a range that could be considered cost effective, and it would require NHS England, patients, carers and clinicians to sign up to it*".

We acknowledge there will be further confidential information which you will share with NICE in order to address points which NICE has made in the ACD, and we thank you for your commitment to and engagement with the SMA community.

Biogen's announcement that the Expanded Access Programme will close to new patients after 1st November places even more importance on successfully concluding the Managed Access Agreement negotiations as soon as possible and no later than October 2018.

We are urging you and NHS England to be as flexible as possible in your discussions on price so that, as a matter of urgency, a Managed Access Agreement that merges seamlessly with the current EAP is set up and ensures that patients are able to access this life-changing treatment at the earliest possible opportunity.

Yours sincerely,



Robert Meadowcroft
Chief Executive
Muscular Dystrophy UK



Doug Henderson
Managing Director
SMA Support UK



Joanna Mitchell
CEO
The SMA Trust