

Sheela Upadhyaya,  
Associate Director Technology Appraisals and Highly Specialised Technologies,  
NICE

29<sup>th</sup> November 2018

Dear Ms Upadhyaya,

### **Access to nusinersen discussions with Biogen and NHS England**

We are contacting you following your announcement on 12<sup>th</sup> November that NICE is continuing to progress discussions with Biogen and your assurance that you are committed to swiftly progressing these discussions with both Biogen and NHS England.

We are encouraged that Biogen and NICE are working on potential routes around the cost-effectiveness calculations which have appeared to be a significant barrier in the process so far. We hope that these discussions can be conducted in parallel with negotiations with NHS England on reimbursement and understand that the negotiations are at a delicate stage.

However, you will no doubt be aware that there are already at least two babies with a new diagnosis of SMA Type 1 who have not been able to access nusinersen treatment due to Biogen's decision to close the EAP on 1<sup>st</sup> November. The health of these infants will decline because of this and the delay in the process for evaluating this drug. Tragically, even if the drug is made available in the future, the fact that there has been an avoidable delay means that there will be a worse outcome.

As patient groups we cannot stand by while families endure the distress that this brings and watch clinicians having to deliver this devastating news without contacting you, Biogen and NHS England. We therefore ask that, as an act of 'good faith' in the commitment of all parties to progress discussions to a satisfactory conclusion, you all agree with immediate effect to implement a 'bridging solution' for these and any other infants with SMA Type 1.

This request is also being made to Terry O'Regan - Vice President and Managing Director, Biogen UK and Ireland - and John Stewart - National Director of Specialised Commissioning, NHS England - with copies to our clinical colleagues, Professor Francesco Muntoni and Dr Ros Quinlivan.

We look forward to your earliest possible reply to our request and thank you for your continued efforts in this difficult process.

Yours sincerely,



**Catherine Woodhead**  
**Chief Executive**  
**Muscular Dystrophy UK**



**Doug Henderson**  
**Managing Director**  
**Spinal Muscular Atrophy UK**



**Gennadiy Ilyashenko**  
**Co-founder TreatSMA**