Meeting Notes

1. EJ opened the meeting and set out the rules of confidentiality in relation to the purdah period. The attendees agreed that the
content of the meeting would not be discussed until the end of the purdah period. After the purdah period (expected to be on Friday 9 June) draft minutes will be circulated and these will then be in the public domain.

2. EJ summarised the current position:
   - The ENDEAR unpublished phase 3 study showed that nusinersen is dramatically effective, but long term effects are not known.
   - Pre-market authorisation:-, Biogen have confirmed that the drug is free when linked to the expanded access scheme for patients with SMA with symptom onset before 6 months (SMA Type 1) and that there are no production or supply issues, but there are additional costs associated with its administration that are not included in the expanded access scheme.
   - NHS England’s position is that it does not commission drugs at the pre-market authorisation stage.
   - NHS England’s Clinical Panel considered a proposal for supporting the expanded access scheme additional costs but was not persuaded by the published evidence presented.
   - James Palmer (Specialised Services Medical Director) has indicated that when the European Public Assessment Report is available a further submission to the Clinical Panel will be made.
   - There are conditions associated with administration but it is expected that any NorthStar centre could meet those conditions for administration.
   - Up to Friday, some hospitals have administered nusinersen with free product and have absorbed the administration costs. Other trusts have not accessed free product through the Extended Access Programme. As a consequence, there is an inequity of access across England.
   - EJ believes that there are approximately 50 children in England with SMA type 1. If these patients were distributed across the 15 NorthStar centres this would be about 3 patients per centre. If all centres could absorb the additional costs for these patients that could be a way forward.
   - NICE have not confirmed whether they will be assessing the drug as part of the HST process or through their regular assessment process. Whilst NICE have not confirmed the above or outlined a date for making a decision regarding this, NHS England would be unable to develop a separate policy whilst this is under NICE consideration.
   - EJ explained that on Thursday 1st June the European Medicines Agency granted a broad license for 5qSMA which includes types 1, 2 and 3. EJ outlined that this could
impact on Biogen’s position with regard to free access and that this requires clarification from them.

3. EJ confirmed his summary with the group and opened the floor for discussion.
   - LR updated the group that Biogen had contacted SMA Support on Friday to state that the list price would be £270,000 pa for non-loading years and that there are 350 patients in Europe currently on the Extended Access Programme. LR outlined her concerns that NHS England has missed the opportunity for children to be part of the EAP in England.
   - EJ confirmed that Biogen have put in writing that existing patients accessing the drug free, will have this for life and that his working assumption is that there will be provision of free product to existing patients on the EAP with type 1 SMA. There are precedents for this in other companies. LR clarified that her understanding was that the EAP would not yet be closed and there was a window of opportunity until the NICE appraisal route was confirmed. PB agreed with that and said that it was his understanding that the EAP would be open for a period after market authorisation.
   - EJ confirmed that NHS England’s commissioning position at this point in time is to ‘not routinely commission’. AP confirmed that a ‘not routinely commissioning policy’ has been drafted following the decision of the Clinical Panel and this will be issued when the purdah period is over.
   - FM and AP confirmed that, though patients could potentially apply for access to the drug through the existing Individual Funding Request process, it was unlikely that patients would be able to demonstrate the necessary ‘clinical exceptionality’ of an individual child seeking treatment given the nature of the disease and, sadly, its usual course within the cohort of patients with SMA type 1.
   - EJ clarified that, given the current NHS England ‘not routinely commission’ position for access to the drug, NHS England will not make payments to support the administration and treatment costs. He also confirmed that it is also not the responsibility of CCGs to pay for any part of the treatment or ancillary costs as they are not the responsible commissioner. He also confirmed that Trusts should not be billing CCGs for any aspects of the treatment that patients are receiving now.
   - AP explained that Biogen have not yet confirmed if they will or will not support additional administration costs. Biogen has approached Centres to help them work through what the infrastructure costs are to enable additional costs to be calculated. Biogen have not made clear what the mechanism would be if they did decide to pay towards the additional costs
Given the above, EJ outlined that the best way forward was for Trusts to pay the additional costs. He acknowledged that this was a financial pressure but as outlined above, if the cost was spread across the North Star Centres, that this could help to share the pressure.

CMB confirmed that Newcastle is potentially willing to provide the treatment and to open this up to out of area patients if the transferring trust will fund the additional costs.

EJ thanked CMB for this offer, acknowledging that the ideal position was that patients should be able to go to the hospital within their catchment area so as to minimise unnecessary travel for families.

DK reported that she was unable to reach agreement at Addenbrooke’s for access to the EAP. DK will raise this issue again with the medical director. EJ offered to discuss/visit Addenbrooke’s to try and support a resolution.

4. The group identified a number of issues which trusts are experiencing:

- Some trusts have queried whether they would face potential legal action if the drug was subsequently not funded by Biogen and the trust withdrew treatment. EJ confirmed that Trusts accessing the EAP have received written assurance from Biogen about continued funding for existing patients. NorthStar members agreed to share this agreement/assurance with Trusts who have not yet agreed to access the EAP.

- LR raised concerns that patients who can’t access the EAP through their regional centre are finding it difficult to find a place and that it could be useful to put in place a clear system or registry. As a first step, it was agreed that hospitals will supply information to EJ about the patients that they will accept and whether they have to be living within their catchment area. EJ emphasised that the ideal position would be for all patients to have access within their own regional centre. (LR is aware, for example, of a patient in Ipswich who is unable to find a place. Their regional centre would be Addenbrooke’s.)

- The need to clinically prioritise patients was raised. There is a draft document prepared by North Star clinicians on this which is based on clinical outcomes to date. EJ queried why this was an issue given that Biogen have confirmed that there is no issue with product availability and that if patients can access the treatment within their regional centre then this should not be a problem. In the short term, as Centres such as GOSH enrol their 19 patients, prioritisation is required. Another potential issue is with some parents with older children seeking a review of their child’s diagnosis.
The EAP criteria is onset of symptoms before 6 months (consistent with SMA Type 1)

- The administration of nusinersen via intrathecal injection for some Trusts is challenging in terms of clinical logistics (staff and space/equipment). Some trusts have experienced strict internal criteria regarding accessing intrathecal injections whilst some have had no issues at all.
- EW explained that the cumulative effect of new patients will impact on capacity. CMB agreed and felt that 50 patients per year was an underestimate. It was acknowledged that if the drug is extended to type 2 and 3 patients that this will be further compounded. EJ agreed but felt that this was not an issue that could be addressed at this meeting.
- Some Trusts have linked up to support additional capacity – e.g. Evelina and GOSH – acting together as a regional centre. However this can result in other clinics being cancelled to accommodate patients requiring nusinersen.
- FM queried if 15 centres was the right ultimate model for delivery of the treatment. The group agreed that all NorthStar centres should have the ability to administer nusinersen and there was no reason to restrict the number of centres. The NICE Highly Specialised Technology appraisal is predicated on a smaller number of centres.
- The meeting raised a concern about 13 English children who have been travelling to Paris for treatment and that this arrangement will, now EMA has given marketing authorisation, cease – potentially imminently. GOSH confirmed that they have agreed to take on 5 of these patients but it was confirmed that it is not known if decisions had been made regarding the remaining 8 patients. Individual Trusts will need to consider how these patients who live in their areas will be prioritised for treatment within their existing caseloads.

5. EJ thanked participants for travelling and closed the meeting.

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<tr>
<th>Actions</th>
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<tr>
<td>Confirm with Biogen whether the EAP commitment of drugs for life, is extended to new patients post the market authorisation and the timescale with regard to how long the EAP will remain open.</td>
<td>AP/PG</td>
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<td>To clarify further with the Medical Director at Addenbrooke’s their position about accessing the EAP (and in particular the position relating to children from the Ipswich part of the Region). If that position is not positive, to liaise with EJ and a meeting with the MD will be organised to work through the issues.</td>
<td>DK/EJ</td>
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To share the NorthStar Network letter from Biogen confirming their position that outlines free access to the drug for existing patients.

NorthStar members to confirm in writing their position to EJ in relation to:
- Number of children they currently see with SMA type 1 who may be eligible for the EAP
- If the trust is accessing free product through the EAP
- If so how many children are being treated now and what is their expected future capacity
- Are they accepting patients from their catchment area or and from further afield or not
- If the trust is not accessing the EAP do they have plans to do this

Trusts to list the names of patients from within their areas who have been accessing treatment in Paris and who will now need a place within England. (Patient information can be shared over NHS Net)

The NorthStar draft clinical prioritisation criteria will be circulated with the minutes

NorthStar network to share any policies/protocols they are developing on administration of nusinersen with others in the network

Confirm with Biogen their position on funding administration costs and their options for payment (e.g. the possibility of offering a payment per head tariff)