**April 2021**

**NICE Process Review Case for Change – SMA UK's on-line submission – key points.**

**Theme 1: Alignment of the current guidance development processes**

We have reviewed and agree with the Charity Medicines Access Coalition's (CMAC's) general and detailed submission on this theme. Additionally:

Many patient groups (PGs) for rare conditions are small organisations with very limited resources most often focused on providing information and support. Many rely on volunteers. Our experience when faced with the NICE regulatory system for the first time in December 2016 was probably not untypical. The scoping response request initially didn't arrive as NICE hadn't got the correct contact details. When it did, we were faced with a completely new language and set of expectations with no time to delve into what could or could not change at this stage of the appraisal and no support from NICE. Scoping consultations limited to a maximum of 20 days could well prevent small PGs, already with stretched or limited capacity, from inputting in an informed way into the consultation stage.

We have been part of three appraisals. The first one (which took a year from first scoping for an appraisal route to be selected and then a further 18 months to reach a final determination) in particular suffered greatly from a failure to keep PGs and the patient community 'up-to-date'. We would welcome proposals that will address this. Even being told there is 'no news yet, but work is in progress' is better than silence. The tantalising possibility of access to treatment creates great stress and anxiety. During the first appraisal we often heard people say it was almost better when there was no possibility of treatment and they got on with life than to live in this limbo of not knowing.

Technical engagement was not a step open to us in the nusinersen STA but has been in the risdiplam STA. It offered a safe and calm opportunity to bring stakeholders together and identify gaps and consider data. We have yet to see if this makes a difference to outcomes, but it felt a much better process and that the patient voice really was being considered from the outset. We would not want to see this step as optional.

**Theme 2: Opportunities for new process improvements and ways of working**

We have reviewed and agree with Charity Medicines Access Coalition's CMAC's general and detailed submission on this theme.

Additionally:
Having taken part in three appraisals to date we agree that honest feedback as to which parts of our evidence were helpful and informing and which may not have been helpful could save much time and energy. We agree with CMAC that 'the Final Appraisal Documents should provide an opportunity to reflect how patient input has shaped NICE's decision, and that feedback on written submissions should be an expected minimum requirement……it is also essential that clear feedback is given to those patients and patient representative organisations on how their contributions have made an impact on the decision-making process'.

**Theme 3: Commercial and Managed Access processes**

We have reviewed and agree with Charity Medicines Access Coalition's CMAC's general and detailed submission on this theme. Additionally:

We look forward to viewing the Innovative Medicines Fund consultation paper to understand how this will work. Until we see this, we cannot know how the newly structured STA processes with their moderators, the new HST processes and this Fund will work together and what the impact will be on access to technologies for rare conditions. It is essential that all the new approaches and changes fit together well to make a system that improves the experience and outcomes for PGs like ours.

**Theme 4: Highly Specialised Technologies programme**
We have reviewed Genetic Alliance UK's general and detailed submission addressing all the NICE HST proposals. We agree with the concerns they have raised and their alternative proposals.

Additionally:
Without knowing how the modifiers for the STA pathway proposed in the methods review will work, what the proposed Innovative Medicines Fund will look like, and how these two systems will interface with a re-imagined HST process, it is impossible to say if the current gaps and issues created by the binary system of STA versus a reworked HST system will have been addressed.

What we are keen to avoid is a repeat of our experience with nusinersen and its STA path. Following scoping, it took NICE a year to decide that nusinersen, the first treatment for rare condition SMA, would be subject to an STA. As a patient group, we had no feedback as to why this route was chosen, even though it was clear to all from the beginning that an STA's cost / QALY threshold would disadvantage the treatment. This was particularly the case for the most severely affected infants, for whom there was the most evidence of clinical effectiveness and an Early Access Scheme. Even at zero pricing, the usual STA's cost / QALY threshold would be breached for these infants. It took a further 18 months and four committee meetings, when we watched hours of work, time and money going into yet more economic modelling, arguing and negotiations, to reach a final determination that ensured the inclusion of these severely affected infants and other groups. The resulting rushed Managed Access Agreement then created its own unexpected barriers to access for some and continued to create immense frustration and distress in the community.

Subsequently, we experienced the HST pathway, and, despite both processes having excellent Chairs, there is no doubt the latter provided a much better opportunity to really explore uncertainties in a more timely way with both patient and clinical experts.
We are left speculating that an HST pathway for nusinersen would have provided both more realistic price thresholds and a more efficient process. This in turn would have ensured a more timely, in depth exploration of the inherent uncertainties that come with an innovative medicine for a rare condition, and probably an earlier delivery of this treatment in the NHS.

The consultation document acknowledges that *'there has been an increase in the number of treatments in development across the spectrum of rare disease'*, we are therefore concerned to read that NICE anticipates that the number of HST evaluations will not change, as this increase is likely to mean that there will be more treatments that are disadvantaged by the standard pathway, and that there will be more experiences like ours with nusinersen. HST should be available for all rare disease treatments that would be disadvantaged by the standard pathway.

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