SMA UK's Submission to NICE Methods Review

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The National Institute of Health and Care Excellence (NICE)'s 6-week public consultation on the case for changing how it evaluates new medicines for use by the NHS started in November 2020. We attended a number of consultations and discussions run both by and for Patient Groups.

Some of the key points we made in our December response to the consultation were:

Uncertainty

We welcomed that NICE acknowledges that uncertainty is always a problem with rare conditions like SMA where there are small populations and limited clinical trial evidence, and that the current evaluation rules impact negatively on the availability of treatments for rare conditions. We highlighted that currently discussions around uncertainty often result in lengthy appraisals, as with nusinersen, resulting not only in emotional stress for families and adults waiting for the outcomes but also in hugely concerning travel for treatment to countries that have set up earliest possible access. We asked for this acknowledgment to be translated into action that will see early access programmes set up that will then capture the evidence of clinical efficacy and cost effectiveness.

A wider review and inclusion of the costs and benefits

We asked for a wider review and inclusion of the costs and benefits that NICE considers in its evaluations e.g.

- health and social care costs borne by families: equipment and housing adaptation costs.
- Emotional and psychological costs to patient and family – sleep deprivation, impact on social life and well-being
- education costs: requiring Teaching Assistants, school adaptations, University PAs.
- unpaid carers (parents and grandparents) and patient costs – loss of potential productivity and contribution to the economy through work / taxes.

Outcome tools that will reflect patient values and include impact on carers

We welcomed proposals for outcome tools that will reflect patient values and include impact on carers. We agreed that more work needs to be done to establish appropriate tools to measure outcomes for children. There should be a high level of patient group involvement to assist with the development of suitable tools.

Real-World Evidence (RWE)

We welcomed the proposals to include reviews of Real-World Evidence (RWE) in the process.

Greater focus and support for patient involvement in NICE appraisal processes

We called for greater focus and support for patient involvement in NICE appraisal processes. We pointed out that the innovations resulting in the development of more treatment possibilities mean that for the first time many small charities engage with the regulatory systems to make the patient voice heard and that for many with very limited resources, this is a formidable task on top of their information and support roles. Many groups are concerned to ensure they present their community's voice in ways that will be heard and will 'fit' NICE processes in a way that will have impact, and that will leave their communities satisfied that they have indeed fulfilled their role as advocates. The option of recruiting or paying skilled specialists is rarely a realistic budget option. Without support, groups with resources will have an unfair advantage over smaller less well-resourced groups – which all too often are groups for rarer conditions.

We confirmed our view that an independent Evidence Review Group is a necessary part of the process but commented that we have been struck by the absence of patient expert input into this stage of the NICE method / process and the message this sends about the value of this view.