NICE WIDE TOPIC PRIORITISATION MANUAL CONSULTATION

Submission from The Brain Tumour Charity

The Brain Tumour Charity is the world's leading brain tumour charity and the largest dedicated funder of research into brain tumours globally.

As a charity we provide support to the brain tumour community through the funding of new, innovative research, as well as through events, a dedicated support team, and advocating for necessary change to improve the lives of those affected by this disease.

We welcome the opportunity to submit to this consultation inquiry and share some our answers on the NICE wide topic prioritisation manual and considerations for how it could be improved.

1) Do you agree NICE should focus on guidance production and maintenance on priority areas (section 5) and these should be reviewed periodically to ensure NICE is supporting what the health and care system needs? If you disagree, please give reason and rationale.

The Brain Tumour Charity agrees that NICE should focus on guidance production and maintenance on priority areas and that these priorities should be reviewed periodically to ensure NICE is supporting what the health and care system needs.

However, it's vital that the priorities NICE opts to focus on align with other health and care system bodies including NHS England's Cancer Programme, the Office for Life Sciences, the MHRA, commissioning bodies and the National Institute for Health and Research (NIHR).

At present, misalignment between NICE priorities and other health system bodies can create confusion amongst innovators for what the health system in England is prioritising – in particular, alignment must be achieved with NHS commissioners.

The Brain Tumour Charity would advocate for the need for more formal joint-horizon scanning and shared prioritisation processes across these bodies, whilst recognising that NICE's independence in evidence appraisal is fundamental to the credibility of its guidance.

We would also encourage NICE to consider the rate of change within a disease area as an explicit criterion when setting and reviewing priorities. Though priority setting should take account more established measures such as disease burden, unmet need inequity of access and whether a condition has been historically under-prioritised.

2) Do you agree that NICE should be transparent about its remit (section 3.12, section 6.1.1 and 6.2)? If you disagree, please give reason and rationale.

The Brain Tumour Charity broadly agrees that NICE should be transparent about its remit and thus enabling stakeholders to know areas of work that NICE is responsible for.

Uncertainty about whether a topic falls within NICE's remit, or that of another body, could lead to avoidable delays in guidance development.

However, transparency about remit boundaries is not sufficient on its own if these boundaries create gaps in guidance or patients. In the context of brain tumours where treatment pathways often involve novel medicines, devices and diagnostics, there are real risks that topics fall into grey areas between NICE and the MHRA.

The Brain Tumour Charity notes that the manual states that topic areas within the remit of the MHRA are outside NICE's scope but does not address what happens when a topic crosses both bodies' responsibilities – an increasingly common scenario given the pace of change surrounding targeted and combination therapies as well as medical devices, and hybrid tech that may straddle the line between med-tech and treatment.

We know there are ongoing efforts to improve alignment between NICE and MHRA, and we believe this manual provides another opportunity to reflect that work. The Brain Tumour Charity recommends that the manual explicitly acknowledges this collaboration and provides a clear process for topic referral between the two bodies, so that when a topic is deemed outside of NICE's remit, there is a transparent pathway for it to be picked up by the appropriate organisation rather than simply declined. In this regard, there is a need for clearer referral routes and better coordination between the two regulatory bodies when topics sit across NICE, MHRA and commissioning responsibilities.

To summarise, we support the principle of transparency about NICE's remit but urge NICE to go further, making inter-agency processes visible within the manual itself.

3) Do you think the new process and consideration factors for NICE to propose topics to the Secretary of State for NHAP are robust and transparent (section 4 and Annex A)? If you disagree, please give reason and rationale. Are there any other factors NICE should include to consider?

The Brain Tumour Charity predominantly agrees that the new process and consideration factors for NICE to propose topics to the Secretary of State for NHAP are robust and transparent, but they can also be strengthened.

In particular, we support the following aspects of the proposed approach:

- The commitment to publish all proposals and rationales on the NICE website supports genuine accountability.

- Requiring the HTFG to reach consensus and document its reasoning on each of the three factors provides a structured and auditable decision-making trail – beneficial for understanding rationale on any decisions that are reached.
- The inclusion of patient and user-engagement within the Commercial Intelligence Briefing is a welcome recognition of the importance of lived experience and the patient voice.

However, we believe that there are additional factors that should be considered. These include:

Factor 2: Evidence

The requirement for 'sufficient evidence' to achieve a routine recommendation may inadvertently disadvantage brain tumour related technologies. Individual brain tumour types are classed as rare or even ultra-rare diseases and we know that small patient populations can make large-scale trial evidence difficult to generate. Therefore, it would be beneficial if NICE could clarify how Factor 2 is applied in rare disease contexts – including what alternative forms of evidence may be considered acceptable and information related to evidence generation that may be helpful for innovators who are developing technologies for rare and less common conditions.

Factor 3: transformation of clinical pathways and services

The current framing within this factor emphasises system-wide pathway transformation. However, The Brain Tumour Charity would welcome acknowledgement in this area that technologies can satisfy Factor 3 by fundamentally resharing the care pathway for specific conditions – even where the patient population may be small. For instance, for brain tumours patients' possible improvements and technological developments related to diagnosis or treatment could be profound.

Patient engagement

The Brain Tumour Charity welcomes patient and carer engagement as being listed alongside industry and system engagement as a source of 'additional intelligence' to the Commercial Intelligence Briefing. The Charity would encourage NICE to make this a mandatory component of the process rather than a discretionary one – particularly for conditions where quality of life and care burden must be central to evaluating a technology's value when evidence generation may be more challenging. Patient and carer input should inform judgements about unmet need, acceptable risk, quality of life, care burden, travel burden as well as wider family and social impact.

Health Technology Funding Decision Group (HTFG) membership transparency

The Brain Tumour Charity would welcome greater transparency about how HTFG membership is determined and how relevant clinical or patient experience will be ensured when specialist condition areas, such as neuro-oncology, are under consideration.

It is The Brain Tumour Charity's hope that the above adjustments will be considered which will ensure the framework works fairly and comprehensively for people living with brain tumours and other rare and complex conditions.

4) Do you think the new merged single-stage prioritisation framework is more streamlined and efficient for topic selection? If you disagree, please give reason and rationale.

The Brain Tumour Charity supports the new merged single-stage prioritisation framework. The implementation of an organisation-wide approach overseen by a single prioritisation board is a welcome step towards greater coherence and coordination in guidance development.

Equally welcome is the explicit recognition that population impact will not merely focus on prevalence but will also consider whether the target population is experiencing severely life-limiting or debilitating diseases with a lack of, or no, treatment options. Brain tumours fit into this description. Streamlining of process must not unintentionally favour conditions with larger populations or easier-to-measure system impact. A small patient population can still represent a very high priority where the condition is severe, life-limiting, under-served and has few or no treatment options.

The Brain Tumour Charity welcomes the commitment that rationales for trade-offs and deliberations will be documented transparently and published on the NICE website, enabling patient organisations to engage meaningfully. However, we would encourage NICE to ensure that patient perspectives are sought consistently and proactively to the consideration process – particularly in conditions, such as brain tumours, where evidence generation can be challenging.