BIA response to NICE consultation on integrated topic prioritisation and strategic principles

The BIA has responded to the National Institute for Health and Care Excellence (NICE)'s consultation on integrated topic prioritisation and strategic principles. For further details about the consultation, please visit the consultation page on the NICE website.

Consultation response

The BIA welcomes the opportunity to provide feedback on these proposals. Please see below our comments on specific aspects of the proposals.

Integrated topic prioritisation

The BIA welcomes NICE's intention to establish a new overarching integrated approach to prioritisation and topic selection that aims to improve NICE's prioritisation work and coordination of relevant NICE guidance. However, the BIA recommends that NICE provides substantial additional clarity on the consultations proposals as detailed in the points below. Also, to avoid confusion or misalignment with NICE's other live consultation on the inclusion of technology appraisal recommendations into guidelines, NICE should clarify its use of integration terminology in this consultation document.

- Section 6 sets out NICE's prioritisation framework eligibility criteria. The BIA believes that further clarification is required on how routing decisions for products to HST will be considered by NICE's new prioritisation board and how it will appropriately align with VPAG commitments to automatically route new medicines and significant indications directly into a NICE technology appraisal. Additional information is also needed in section 6.3.3 to make clear the circumstances under which new medicines need 'further ratification' by the prioritisation board before being routed to HST.
- Section 7.3.1 describes the prioritisation framework stage 2 criteria. The proposals suggest that medicines which are being considered for the HST programme may also be subject to prioritisation framework stage 2 criteria, meaning treatments for rare diseases would have to satisfy these criteria in addition to HST qualification criteria. We are concerned that the requirement to fulfil two sets of criteria could potentially restrict the number of rare disease medicines from receiving positive outcomes, therefore limiting patient access to rare disease treatments. In particular, the criterion relating to the size of the relevant target population in stage 2 could risk significantly

disadvantaging rare disease medicines. NICE should consider the potential negative impact that applying population impact criterion to determine medicines eligibility for technology appraisal could have on rare diseases, since these populations are inherently smaller compared to populations with more common conditions.

- Section 9.1 outlines the clarification process if a prioritisation decision is queried by stakeholders. This section states that while the request for clarification will not usually offer an opportunity to revisit or overturn the prioritisation board's decision, in the case of a very rare disease, a decision could be overturned if there is evidence that the highly specialised technologies (HST) routing criteria have not been appropriately applied. Further information is required on how routing decisions will be addressed where clarification is required and the types of evidence that would be considered in these cases.
- Section 11 sets out NICE's approach to engagement with stakeholders. It is positive that NICE intends to enhance topic prioritisation decision making by utilising opportunities for engagement with stakeholders and relevant organisations throughout the topic prioritisation process. The BIA supports NICE's commitment to enhancing engagement with stakeholders, especially early engagement, to enable effective information sharing and support optimal decision making. However further clarity is required on exactly how NICE will support routinely engaging with stakeholders, including industry and patient advocacy groups, and how this engagement will be monitored. It would also be useful to understand how stakeholders will have opportunities to engage with the prioritisation board during routing decisions.

Rare disease principles

The BIA welcomes NICE's commitment to clearly define its prioritisation approach to rare disease topics to help guide its prioritisation under its new integrated prioritisation process. It is well known that people living with rare diseases face inequities within the healthcare system, from receiving a diagnosis to accessing specialist care and treatment. It is positive that NICE recognises that the impact of lack of evidence and limited effective rare disease treatments on patients and families is profound and that guidance on rare diseases may be overlooked.

The BIA supports these strategic principles as a means to support the new prioritisation board adopt a clear and consistent approach to decision making on guidance relating to rare diseases, ensuring rare disease topics are proportionately considered by NICE. However, it is currently unclear in the proposals how these rare disease principles will support the prioritisation board's decision making in practice and there appears to be a

strong risk of worsening the inequity of access for patients with rare diseases. NICE should provide greater clarity in some areas as described below.

Principle 1

We welcome NICE's first rare disease principle on creating an attractive environment for pharmaceutical companies to develop innovative treatments for rare diseases. This is important and timely as companies are finding it increasingly difficult to make the case for the UK, in comparison to global competitors, as a launch market for new medicines for rare diseases, and there is a risk of the UK becoming deprioritised as a launch market for rare disease treatments. It is therefore important that the UK has an attractive commercial environment and remains a leader in the development and manufacture of innovate medicines to ensure these innovative products are brought to UK patients. Moreover, this commercial environment impacts wider investment decisions by such companies, such as R&D and manufacturing, which can impact the UK life sciences ecosystem more broadly. In order to create an attractive environment, this principle should be embedded across NICE methods and processes.

This principle states that NICE aims to create an attractive environment for the development of innovative treatments for rare diseases with few or no treatment options. NICE should also ensure that there is an attractive environment for innovative treatments that offer substantially greater clinical benefit than existing treatment options.

It would also be useful to better understand how principle one will be applied in prioritisation decision making and exactly how NICE will foster an attractive environment that stimulates innovation for rare disease medicines through this new approach.

Principle 2

The BIA believes that additional detail and clarification on the reasons and potential implications of the points set out in principle two is required.

It is important to understand how the principle of not routinely producing guidelines that relate to single rare diseases would work in the context of NICE's plans to incorporate and integrate technology appraisals into guidelines, as set out in NICE's live consultation. Also, we are concerned that this principle could disadvantage rare disease patients as there may be circumstances in which developing guidelines for single rare diseases would be an appropriate use of NICE resources.

Principle 3

The BIA strongly supports NICE's commitment in this principle to proactively collaborate with rare disease stakeholders in both clinical guidelines and technology appraisals. We believe collaboration, early engagement, and shared value and risk between industry and

NICE is crucial in optimising positive outcomes and ensuring transparency throughout the technology appraisal process. Additional details should be provided on how NICE plans to implement and ensure proactive collaboration with industry going forward and on how NICE will "work with stakeholders to increase the evidence base relating to rare diseases".

The BIA recently published a report on evaluating the UK environment for patient access to medicines for rare diseases. Informed by the evidence presented in this report, the BIA has since called for the UK to lead greater collaboration between system stakeholders to facilitate broader and faster access to new treatments. This comes as NICE is seeking to increase its collaboration with other international regulatory bodies via the MHRA's new International Recognition Procedure (IRP).

The UK Rare Diseases Framework provides an important five-year national vision that sets out a multifaceted approach for how the UK will improve the lives of those living with rare diseases. It has helped to drive accountability and facilitate greater coordination across the four nations and between stakeholders. The BIA therefore strongly supports NICE's commitment to ensure alignment with the Framework as addressing its four priorities will be key in driving improvements for people living with rare diseases. We would welcome further information on how NICE plans to ensure alignment with the Framework and would encourage NICE to provide regular updates on the progress it has made in this area.

Highly Specialised Technologies (HST)

The consultation paper notes that NICE's HST programme provides guidance for ultra-rare diseases with the aim of securing fairer and more equitable access to treatments in this area of high unmet need. However, the BIA is highly concerned that in its present form, the HST's eligibility criteria is exacerbating inequitable access to medicines for patients with rare and ultra-rare diseases.

Following NICE's Methods Review, the number of eligibility criteria for a drug to be routed through HST was reduced from seven to four. Whilst the intention to make the criteria simpler and clearer was welcome, we are concerned that the routing criteria is highly selective and is making it more challenging for products to be routed to HST. This results in many treatments for ultra-rare diseases being routed to the Single Technology Appraisal (STA) process, where the cost-effectiveness thresholds are much lower and therefore are less likely to receive a positive reimbursement decision. This will significantly impact patient's access to life changing medicines and calls into question the UK's ability to deliver genetically targeted medicines as part of its ambition to create the most advanced genomic healthcare system in the world.

The BIA welcomes NICE's commitment to review the application of the HST routing criteria later this year and recommends that industry experience and feedback should be

considered as part of the review. Due to the clear negative impact that inappropriate routing via STA can have on patient's access to life changing medicines, BIA recommends that NICE urgently brings forward the consultation on the HST routing criteria to take place as soon as possible.

About the BIA

The BIA is the trade association for innovative life sciences and biotech industry in the UK, counting over 500 companies including start-ups, biotechnology, universities, research centres, investors and lawyers among its members. Our mission is to be the voice of the industry, enabling and connecting the UK ecosystem so that businesses can start, grow and deliver world-changing innovation.

BIA represents the interests of its members to a broad section of stakeholders, from Government and regulators to patient groups and the media. We also work with organisations at an international level to ensure that UK biotech is represented on the global stage including EuropaBio, the European Federation of Pharmaceutical Industries and Associations (EFPIA) and the International Council of Biotechnology Associations (ICBA).

BIA is the key thought leader for the sector, operating across a wide range of areas such as policy, finance, science, regulatory, legal, skills and talent as well as genomics, engineering biology and techbio.