BIA response to NICE's consultation on adopting the EQ-5D-5L value set

Summary

The BIA has responded to NICE's consultation on adopting the EQ-5D-5L value set. Summary of BIA response:

- BIA understands the rationale for moving to a new UK value set that reflects more up-to-date methods for capturing societal preferences.
- However, we are concerned that adoption of the new value set may further exacerbate existing barriers to patient access to medicines targeting rare, disabling and life-long conditions, where treatment objectives often focus on symptom relief and functional improvement rather than extended survival.
- We urge NICE to adopt a phased approach to implementing the EQ-5D-5L value set, enabling both EQ-5D-3L and EQ-5D-5L value sets to be permitted as reference case options to mitigate any unintended impacts on patient access to innovative medicines.
- BIA also recommends NICE provide clearer guidance on the evidentiary requirements for demonstrating when EQ-5D-5L is unsuitable, including greater flexibility on the acceptability of alternative measures and international data to support the use of condition-specific or other quality of life tools.
- **Have we considered all the relevant evidence in preparation for adopting the EQ-5D-5L value set? Is our interpretation of the evidence appropriate?**

BIA recognises the significant work NICE has undertaken to support the adoption of the EQ-5D-5L value set and understands the rationale for moving to a new UK value set that reflects more up-to-date methods for capturing societal preferences that were previously outdated and based on preferences from over 30 years ago.

However, BIA does not believe that all relevant evidence has been considered in full, including the potential impacts of the new value set on certain groups and populations

affected by NICE decisions. For example, the impact of the new value set on people living with rare and ultra-rare conditions, and their carers, has not been considered in NICE's impact assessment.

This omission is significant given NICE's own analysis shows that use of the EQ-5D-5L value set significantly changes how health-related quality of life gains are valued compared with the EQ-5D-3L value set. These key changes include:

- higher, on average, utility values for more severe health states
- a greater weighting on life extension relative to quality-of-life improvements
- a lower relative weighting on mobility and self-care health dimensions

For people living with chronic rare or severe diseases and their carers, in many cases health gains are achieved through small but meaningful improvements in symptoms, functioning or activities of daily living, rather than extended survival. These groups will therefore be particularly affected by these shifts in how quality-of-life gains are now valued.

This issue is reflected in the scope of the NICE commissioned impact assessment, which excluded evaluating the impact of the change of the EQ-5D-5L value set on carers' health-related quality of life, and did not look at Highly Specialised Technologies (HST). Both areas form an important part of NICE's remit and could be among those most likely to be negatively impacted by changes in the valuation of quality-of-life gains. Indeed, NICE's own analysis indicates that the EQ-5D-5L value set may, on average, favour life-extending medicines whilst disadvantaging medicines that primarily deliver improvements in quality of life.

Treatments for rare diseases already face significant difficulty demonstrating cost-effectiveness under conventional QALY-based frameworks due to structural evidence constraints associated with small patient populations. BIA is therefore concerned that adoption of the new value set may further exacerbate existing barriers to access and reimbursement for medicines targeting rare, disabling and life-long conditions, where treatment objectives often focus on symptom relief and functional improvement rather than extended survival.

- **Are the changes to the NICE technology appraisal and highly specialised technologies guidance manual (PMG36) appropriate?**

BIA considers the proposed changes to the NICE technology appraisal and highly specialised technologies guidance manual (PMG36) to be broadly appropriate in principle. However, we are concerned that the proposed changes may not provide sufficient mitigation for medicines for rare and ultra rare diseases and paediatric conditions, where the impact of the new value set is likely to be significant.

The flexibilities set out in PMG36, including the option to use alternative health related quality of life measures where EQ-5D-5L is unsuitable, are welcome. However, demonstrating that an alternative measure is sufficiently robust and acceptable for use in economic evaluation can be highly complex, particularly in rare diseases where data is inherently limited and alternative measures are often not available, therefore making it difficult for alternative measures to be adopted in practice.

In addition, identifying and justifying the use of alternative measures may increase appraisal complexity and timelines. This could result in delays to decision making, with potential consequences for patient access to new treatments, which can be critical for people living with life-limiting rare conditions with no other treatment options. Delays to access would also run counter to wider Government ambitions to accelerate patient access to innovative medicines.

BIA is also concerned that NICE's stated intention to mitigate against any negative impact by promoting existing flexibilities in its methods, does not represent a new or additional safeguard for those most likely to be impacted by these changes. For example, in many appraisals for rare disease medicines, flexibilities are already applied where possible to manage inherent evidence limitations associated with small populations. As such, they provide limited additional mitigation of the impact of adopting the EQ-5D-5L value set.

Crucially, these flexibilities are unlikely to address situations where a medicine appears less cost effective because the EQ-5D-5L value set places a greater relative weight on life extension than on improvements in quality of life. This shift disproportionately affects treatments for chronic, rare and disabling conditions, where benefits are often realised

through gains in symptoms, functioning or activities of daily living rather than extended survival.

This issue is also pertinent for treatments that are already close to the upper end of NICE's cost-effectiveness thresholds. In these cases, even small changes in how health gains are valued can affect appraisal outcomes and, ultimately, patient access to potentially life-changing treatments.

Access to rare disease medicines in England already lags behind other countries and, given the disproportionate impact of the proposed changes on many rare disease treatments, they risk making this situation worse. International data demonstrates that between 2020-2023 only 50% of EMA approved non-oncology rare disease medicines were reimbursed and made available to patients in England, compared to 85% in Germany, 67% in France and 61% in Spain. At the same time, the socioeconomic impact and burden of rare diseases in the UK is significant. Research conducted by Costello Medical, commissioned by BIA, shows the estimated cost associated with a rare condition is £70,000 per patient per year, which is approximately eight times higher compared to common conditions like diabetes, cardiovascular disease, cancer and Alzheimer's.

We believe that a cost-effectiveness-based approach is not appropriate for many rare disease medicines. BIA is advocating for an alternative evaluation pathway for rare disease therapies with structural evidence constraints that make conventional cost-effectiveness assessment unsuitable. It would complement existing NICE processes and, adopt a budget-impact approach which focuses on clinical and societal value.

We also urge NICE to adopt a time-limited interim approach to implementing the EQ-5D-5L value set as an additional risk-mitigation measure. For a set period of time, both EQ-5D-3L and EQ-5D-5L should be permitted as reference case options where the value set is likely to affect appraisal outcome and patient access. This would enable NICE to assess the practical impact of the new value set across appraisals, while reducing the risk of unintended consequences before the 5L is fully embedded.

- **Are the changes to the developing NICE guidelines manual (PMG20) appropriate?**

The proposed changes to the NICE guidelines manual (PMG20) are consistent with the approach set out in PMG36 and are appropriate in the context of adopting the EQ-5D-5L value set.

However, under PMG20, guideline committees will be expected to place greater emphasis on using EQ-5D-5L or agree when other quality-of-life measures are acceptable. There is a risk that this could increase guideline development timelines, particularly in areas where the evidence base is limited or complex, which could have implications for clinical decision-making and patient access to care, especially for rare or specialised conditions.

- **Is it clear when alternative methods for capturing health-related quality of life may be accepted, and what the preferred hierarchy is for selecting from the available alternatives?**

BIA recognises that NICE has set out a hierarchy of preferred approaches for capturing health related quality of life, however, there remains uncertainty about how this hierarchy will be applied in practice. In particular, the criteria for determining when EQ-5D-5L is not suitable are not always clear, creating a risk that decisions may vary across appraisals, potentially reducing predictability in decision-making.

Some condition-specific measures may better capture meaningful changes in patients and carers quality of life, however these alternative measures may be considered less robust than EQ-5D-5L and may not be available. Demonstrating that the EQ-5D-5L is unsuitable can therefore be difficult in practice, especially where patient numbers are small and data is limited.

More broadly, this highlights the need to consider the wider aspects of value associated with innovative treatments and how these are captured within NICE's appraisal framework, alongside EQ-5D-5L. This is crucial for rare disease therapies, where benefits often extend beyond direct clinical outcomes to include impacts on caregiver burden, and ability to participate in work or education. BIA recommends that the existing framework be broadened to better capture these aspects of value, which are not fully reflected under the current QALY-based approach.

BIA also recommends NICE provide clearer guidance on the evidentiary requirements for demonstrating when EQ-5D-5L is unsuitable, including greater flexibility on the acceptability of alternative measures and international data to support the use of condition-specific or other generic quality of life tools. As set out previously, establishing a defined interim period that allows flexibility in the use of both 3L and 5L value sets, would also provide an important safeguard while the implications of the new value set are better understood.

- **Beyond what is described in the equality and health inequality impact assessment, are there any aspects of the proposed changes that need particular consideration to ensure they do not result in unlawful discrimination against any group of people on the grounds of age, disability, gender reassignment, pregnancy and maternity, race, religion or belief, sex or sexual orientation?**

BIA recognises the work NICE has undertaken to assess equality and health inequality impacts. However, we remain concerned that the proposed changes may have disproportionately impacts on certain groups of patients, particularly those with rare diseases.

As NICE has recognised, people living with certain chronic and disabling conditions may be negatively impacted by the new value set because treatments for their conditions may appear less cost-effective. For these patients, even small improvements in symptoms or daily living can be transformative and changes in how these benefits are valued risk negatively affecting appraisal outcomes and equitable access to treatment.

These risks are compounded by the existing barriers to access people living with rare diseases face, with only 5% of rare conditions having a licenced treatment option. Without appropriate mitigation, adoption of the new value set may therefore disproportionately impact patients with the greatest unmet clinical need and the innovative companies developing these cutting-edge therapies.

The 2026 England Rare Diseases Action Plan commits to including rare conditions as part of the NHS England Core20PLUS5 programme, the national initiative designed to reduce health inequalities across England. This decision was based on an evidence base which found that people with rare diseases experience inequalities in access to healthcare services.

This reinforces the importance of ensuring that additional mitigations can be adopted if certain populations with high unmet clinical need are negatively impacted as a direct result of adopting the new value set.

- **If you have any further comments in relation to the proposed changes set out in this consultation, please include them here.**

BIA supports the adoption of the EQ-5D-5L value set in principle, however, we strongly encourage NICE to adopt a phased approach to implementation, with appropriate flexibilities to mitigate any unintended impacts on patient access to innovative medicines.

It is also crucial that NICE carefully assesses the implications of adopting the new value set on appraisal timelines and speed of access. Potential increases in appraisal timelines could have significant consequences on timely access to new treatments and may make it more challenging to deliver against the Government's ambitions for the UK to be one of the top three fastest places in Europe for patient access to medicines.

While the announcement of the new value set was framed in the context of supporting life science innovation and increased investment in the UK, there is a risk that many innovative therapies, particularly for rare diseases, where benefits are primarily reflected in quality-of-life improvements, may no longer be deemed cost-effective. This could exacerbate existing systematic barriers for these medicines and reinforces the longstanding need to adapt conventional appraisal approaches to ensure they adequately capture their full clinical and societal value.

Further, if a number of therapies are significantly disadvantaged and are therefore no longer commercially viable, this may in turn impact company's investment decisions, including about whether to launch innovative products in the UK, which risks running counter to wider government objectives to strengthen the UK life sciences ecosystem and support UK competitiveness.

As set out above, we believe that a cost-effectiveness-based approach is not appropriate for many rare disease medicines. Therefore, the BIA is advocating for an alternative evaluation pathway for rare disease therapies with structural evidence constraints that make conventional cost-effectiveness assessment unsuitable. It would complement NICE

processes and adopt a budget-impact approach which focuses on clinical and societal value.

About the BIA

The BIA is the trade association for innovative life sciences and biotech industry in the UK, counting over 600 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. Please contact Senior Policy and Public Affairs Manager Rosie Lindup at rlindup@bioindustry.org for any further information regarding this consultation response.