BIA consultation submission: Labour Health Policy Commission

July 2020

UK BioIndustry Association

Summary

- A vibrant UK life sciences sector can bring many economic and social benefits through rewarding, well-paid jobs and high-value exports, but our ultimate shared aim is for patients to have access to lifesaving and life-enhancing new medicines.
- Treatments produced by the life sciences sector save and improve millions of lives in the UK and around the world every year. Despite advances that have given hope to children and adults with terrible life-limiting conditions, too few NHS patients are able to access the gold-standard of care
- A much broader understanding of value is needed when assessing and appraising medicines that goes beyond pure cost-effectiveness and encompasses wider 'quality of life' measures. NICE and the NHS must be much more ambitious when reviewing their processes.
- Traditional cost-effectiveness measures – such as Quality-Adjusted Life Years (QALYs) used by NICE – are inappropriate for rare and ultra-rare medicines owing to small patient populations. A separate process for rare diseases medicines that can take into account these challenges would be welcome.
- We are encouraged by the development of an Innovative Medicines Fund but are keen to ensure that it is not dominated by oncology medicines and that it proves a sustainable vehicle for rapid access to orphan medicines, in particular.

Overview of the life sciences sector's contribution to health in the UK and Labour's priorities

Helping people live full and active lives is at the heart of what the life sciences industry does. A vibrant UK life sciences sector can bring many economic and social benefits through rewarding, well-paid jobs and high-value exports, but the ultimate aim is for patients to have access to lifesaving or life-enhancing new medicines.

However, the UK has lagged behind its international neighbours with regard to ensuring rapid access to innovative medicines for patients. For orphan medicines – those for rare diseases with no other treatments – less than half of orphan medicines are reimbursed in England, compared to over 80 per cent in Germany and France. For those that are reimbursed, the process is far slower on average, at roughly 28 months in England, compared to 20 months in France and immediate access in Germany[1].

[1] Office of Health Economics, Comparing Access to Orphan Medicinal Products (OMPs) in the United Kingdom and other European countries, 2017. Available online via: https://www.ohe.org/publications/comparing-access-orphan-medicinal-products-omps-united-kingdom-and-other-european

Indeed, the innovativeness of a medicine appears to align with delays in access; the Institute for Cancer Research found that in oncology medicines, the more innovative a medicine was the longer it took to go through regulatory and HTA approvals[2].

As a sector, we understand the pressures that the NHS faces and we are keen to help the NHS meet that challenge. With limited healthcare budgets, ensuring patient access to these latest medical advances will require new flexible routes for licensing, evaluation, uptake and reimbursement. We have welcomed progress in building flexibility into the system, but there is still much that can be done to help ensure that patients are able to access new medicines.

In addition, spending on new medicines will benefit the NHS and patients in the long run by serving as an investment for the next generation of low cost generics and biosimilars, that will become the standard of care in years to come.

National Institute for Health and Care Excellence (NICE) and value assessment

NICE needs to become better at understanding the broader value of medicines and should review health technology appraisals to ensure they capture the real value of medicines to patients, carers and society, not just cost-effectiveness for the NHS. NICE – and NHS England in partnership – should build the value of medicines to patients, carers and society into the Health Technology Assessment (HTA) process in a robust way, ensuring processes capture the real value of medicines not, just cost-effectiveness for the NHS.

While cost-effectiveness and clinical effectiveness are important measures of value, they do not take into account the whole picture of what value means for patients. Factors such as productivity, the impact of poly-pharmacy, the impact on social care use, the impact on other people – such as family and friends – and patient choice are all examples of issues that are not currently within the scope of NICE's processes.

Value must also be seen in the context of long-term benefits, not simply in short term financial savings. For example, by reducing side effects newer medicines may help to support better medicines adherence and thus reduce costs in the longer term. Some new medicines that are becoming available are potentially curative for conditions that have long been chronic or debilitating, but access is being delayed owing to a failure to account for these long-term benefits – for example, discounting processes place the highest value on short-term gains, whole long-term gains are less valuable.

Reform of NICE has been iterative since its creation. Despite changes over time, the fact that the UK lags behind comparable countries in terms of access to innovative medicines is well-documented. NICE – and NHS England in partnership – should build the value of medicines to patients, carers and society into the HTA process in a robust way, ensuring processes capture the real value of medicines not, just cost-effectiveness for the NHS.

[2] Institute for Cancer Research, *From patent to patient: analysing access to innovative cancer drugs*, 2018. Available online via: https://d1ijoxngr27nfi.cloudfront.net/docs/default-source/default-document-library/icr-cancer-drug-manifesto.pdf?sfvrsn=86d95a69_4

NICE is currently undertaking a review of its methods, which has gone some way to identify and tackle some of the issues with regard to medicines appraisal. However, we believe the review lacked the ambition needed to ensure a medicine appraisal process that is ready for the therapies of the future, that are coming down the pipeline quickly – for example, potentially curative cell and gene therapies.

Rare diseases and orphan medicines

There is strong public support for treating patients with ultra-rare diseases as part of the comprehensive foundational offer of the NHS. This includes ensuring they have access to new and effective medicines. We believe that the evaluation of orphan and ultra-orphan medicines (those that treat the rarest diseases) should put patients' needs front and centre and take the widest possible view of value.

Traditional cost-effectiveness measures – such as Quality-Adjusted Life Years (QALYs) used by NICE – are particularly inappropriate for rare and ultra-rare medicines owing to small patient populations, relatively poor understanding of the epidemiology of these conditions, the absence of a 'standard of care' to act as a benchmark (which is required in most cost-effectiveness analyses) and a methodology ill-suited to take into account long-term benefits.

The challenge facing people trying to access rare disease medicines in the UK is all the more frustrating owing to the UK's rich heritage of research and discovery of treatments for these conditions; and the great investment in the search for the next generation of treatments, for example, the 100,000 Genomes Project.

NICE and the NHS should introduce a separate process to evaluate ultra-orphan medicines and orphan medicines which sit just outside the HST evaluation criteria, that is not reliant on cost-per-QALY thresholds. The process should be flexible and recognise the data limitations and methodological challenges associated with orphan and ultra-orphan medicines in particular. We would be keen to see a system that:

- Evaluates orphan medicines and ultra-orphan medicines through a single rare disease pathway: Adopt a single process to ensure that all orphan medicines are assessed by a process that accounts for their unique challenges.
- Assesses empirically based ICER thresholds on a sliding scale: Create a sliding scale of thresholds for assessing orphan medicines supported by clear criteria on where an orphan medicine falls on the scale, to remove the need for arbitrary thresholds.
- Increases sustainability of funding for rare diseases: Increase sustainability in funding arrangements for orphan and ultra-orphan medicines by reinvesting money from VPAS rebates towards funding for innovative medicines.

Innovative Medicines Fund

The development of the Innovative Medicines Fund presents a unique opportunity to address many of the challenges faced in the area of rare diseases.

The Cancer Drugs Fund (CDF) has worked well by funding and facilitating temporary access to promising cancer medicines with some degree of clinical uncertainty that demands further investigation, through data collection in the NHS or clinical studies. The CDF has also proven successful in subsequently transferring medicines from the fund and into routine commissioning.

An equivalent approach to rare disease medicines within the IMF will be useful in addressing some of the issues around data limitations that drug developers face. This will allow patients to access treatments rapidly while developers generate substantial real-world data with the aim of achieving a positive NICE recommendation.

However, we are concerned that the Innovative Medicines Fund may be dominated by oncology medicines, particularly if it, as is planned, grows out of the CDF. We would like to see specific measures to ensure that non-oncology medicines are able to use the fund and that it does not become the CDF by another name. This will be particularly important for medicines for rare diseases, where uncertainty is a real challenge for appraisal in the current system.

This top-level submission is the start of a conversation with the Labour Policy Commission. We would welcome further discussions to provide more detail.

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About the BIA

The BIA is the trade association for innovative life sciences in the UK. Our goal is to secure the UK's position as a global hub and as the best location for innovative research and commercialisation, enabling our world-leading research base to deliver healthcare solutions that can truly make a difference to people's lives.

Our members include:

- Start-ups, biotechnology and innovative life science companies
- Pharmaceutical and technological companies
- Universities, research centres, tech transfer offices, incubators and accelerators
- A wide range of life science service providers: investors; lawyers; IP consultants; and investor relations agencies

We promote an ecosystem that enables innovative life science companies to start and grow successfully and sustainably.