Labour National Policy Forum 2023

Submission on access to medicines

March 2023

UK BioIndustry Association

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.

Innovative new medicines, such as cell and gene therapies, have the potential to transform patient care in the UK by treating the root cause of previously incurable diseases. These treatments have unique potential to treat diseases where there is a high level of unmet need, including many rare diseases. They also have potential to generate significant cost savings for the NHS, as fewer patients would require long-term or lifelong treatment and care for their condition.

As more innovative medicines become available in the next few years, the future Labour government should work alongside the life sciences industry to ensure that NHS patients can access the best available treatments on an equitable basis.

Summary

- Cell and gene therapies are already delivering life-changing and life-extending outcomes for patients in the UK, including those living with leukaemia and Spinal Muscular Atrophy. We are on the brink of a new era in medicine as more cell and gene therapies become available for a wider range of diseases, and it is important that the NHS is able to make them available to patients in a sustainable way.
- Approximately 3.5 million people in the UK will be affected by a rare disease at some point in their lifetime. However, approved medicines are available for only 5% of rare diseases and even where licensed treatments exist, patients can face an uphill battle accessing these treatments on the NHS. This means that many patients with rare diseases face high levels of unmet need.
- One key barrier to broad and timely patient access to rare disease treatments is the evaluation of their cost-effectiveness by the National Institute for Health and Care Excellence (NICE). Due to a range of factors, including high levels of uncertainty from clinical trial data, treatments for rare diseases are often unable to demonstrate their cost-effectiveness in the same way as other treatments. Changes to NICE's methods and processes are required to improve patient access to rare disease treatments.
- We were encouraged by the introduction of the new Innovative Medicines Fund in June 2022 but are concerned that it is not an effective means for improving patient access to the UK's growing pipeline of innovative medicines. The future Labour government should work with NHS England and NICE to review the principles of the IMF to ensure that it is delivering on its stated ambitions.

Cell and gene therapies

One of the most promising new areas of medicine is cell and gene therapies, which have potential to address complex diseases, including many rare diseases for which there are no effective treatments. Cell and gene therapies are designed to halt a disease in its tracks rather than simply manage symptoms, as is usually the case for conventional therapies. These treatments therefore have the potential to transform patient outcomes in areas where there is a high level of unmet need.

Cell and gene therapies' potentially transformative effects on the health outcomes and treatment requirements of many serious diseases could generate significant cost savings for health systems. Fewer patients would require multiple rounds of expensive, intrusive and often risky procedures (such as enzyme replacement therapy or blood transfusions) throughout their lives. This could reduce therapy and hospital equipment costs and cut the costs of trained medical and nursing support staff required to carry out or oversee these procedures. Patients benefiting from cell and gene therapies would also be less likely to suffer the serious and costly complications associated with their conditions, meaning fewer emergency hospitalizations and reducing the burden on families and carers.

However, these treatments face challenges in securing patient access due to high up-front costs and uncertainty about long-term outcomes. In addition, as most cell and gene therapies are single dose or short course treatments, the cost is one off. This poses a major challenge to healthcare payment systems which are largely geared to dealing with chronic and long-running diseases and whose budgets are limited to a single accounting year.

In the past few years, a number of cell and gene therapies have been made available to patients after NHS England struck a confidential deal with the drug company to secure a significant discount. While this approach has been effective in the short-term, we believe that a more sustainable approach is required to secure access to cell and gene therapies and to balance affordability, sustainability and risk between NHS and industry.

Other European countries, including Spain and Italy, are exploring the use of innovative mechanisms for reimbursement, such as outcome-based payment and annuity models, for treatments where signficiant uncertainty exists. These approaches help to ensure timely patient access to treatments while minimising the financial risk to healthcare providers. [1]

[1] BIA, *Ensuring patient access to cell and gene therapies: the case for an innovative payment model*, 2021. Available online via: https://www.bioindustry.org/news-listing/ensuring-patient-access-to-cell-and-gene-therapies-the-case-for-an-innovative-payment-model.html

In order to ensure that NHS patients are able to benefit from transformative treatments, the future Labour government should:

- Support HM Treasury to amend its accounting rules to allow multi-year payments for cell and gene therapies to secure patient access to these potentially curative treatments.
- Work with NHS England, industry, patient groups and other partners to develop a pilot for an innovative payment model that balances affordability and risk to ensure that patients are able to benefit from innovative cell and gene therapies now and in the future.
- Work with NHS England, industry, patient groups and other partners to ensure that the data infrastructure is in place to collect outcome measures to support any new payment model.

Treatments for rare diseases

In the UK it is estimated that 1 in 17 people – approximately 3.5 million people – will be affected by a rare disease at some point in their lifetime. There are over 7,000 known rare diseases, however, only 5% of rare diseases have a single licensed treatment. Even where these treatments exist, patients can face an uphill battle accessing them through the NHS.

In 2021, the Department of Health and Social Care (DHSC) published the UK Rare Diseases Framework, which sets out four priority areas for improving the lives of people with rare diseases over a five-year period. The Framework is implemented via the development of nation-specific Action Plans developed by each UK nation.

- The 2022 and 2023 England Action Plans reference several initiatives which are designed to support greater access to treatments for rare diseases, including the Innovative Medicines Fund and the NICE Methods and Processes Review. It is important that these initiatives are aligned to create a more robust access pathway, and the Framework should be used to ensure this alignment.
- It is important that the progress reports on previous actions provided in each annual Action Plan provides sufficient detail about the progress that has been made and the impact they have had in improving the lives of people living with rare diseases. These need to be supported by metrics that are appropriately related to the objective of the action in question.

The future Labour government should ensure the delivery of the commitments set out in the Rare Disease Framework action plans in order to ensure that rare disease patients in the UK receive the best possible care.

NICE Methods and Processes

One key barrier to broad and timely patient access to rare disease treatments is the evaluation of their cost-effectiveness by NICE. Due to a range of factors, including high levels of uncertainty from clinical trial data, treatments for rare diseases are often unable to demonstrate their cost-effectiveness in the same way as other treatments. As a result, these treatments often take significantly longer to reach patients and where access is secured, the eligible population is often restricted. This means that many rare disease patients are not provided with equitable access to treatments.

The UK lags behind other major EU countries in providing access to medicines for rare diseases.[2] Not only is this frustrating for patients but it risks damaging the attractiveness of the UK as a priority market for developing and launching new drugs.

Between 2019 and 2022, NICE undertook a large-scale review of the methods and processes it uses to carry out health technology assessments. While some positive changes were made, such as allowing greater flexibility in the face of uncertainty and a modifier for severity, the BIA believes that the review failed to sufficiently address the challenges associated with providing access to rare disease treatments. We are also concerned that the changes to the criteria for entry to the Highly Specialised Technologies (HST) programme could restrict the number of treatments for very rare diseases that are eligible, further limiting patient access to these treatments.

We were particularly disappointed that the discount rate did not remain within the scope of the review given that NICE continues to recognise the evidence-based case for changing the reference-case discount rate to 1.5%. While we recognise that NICE was limited by wider policy and fiscal constraints, we are concerned that failure to bring the discount rate in line with the HM Treasury Green Book will undermine the UK's ability to position itself as a first-launch country for innovative new treatments. The future Labour government should work with NICE to ensure that changes are made to its discount rates for health outcomes to bring it in line with HM Treasury's Green Book.

Following the Review, NICE adopted a more modular approach to updating its methods and processes, allowing for specific updates in priority areas.

In 2022, the BIA commissioned research into public opinion on how treatments for rare diseases should be funded and evaluated, which is due to be published in April 2023. In a series of focus groups, we found that 93% of the public agreed that "people with rare diseases should have equitable access to treatments, even if this means additional costs for the NHS". We also found that 89% of the public disagreed with NICE's current approach of evaluating the cost effectiveness of medicines for rare diseases in the same way as more common diseases.

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[2] EFPIA, *EFPIA Patients W.A.I.T. Indicator 2021 Survey,* 2022. Available online via: https://www.efpia.eu/media/676539/efpia-patient-wait-indicator_update-july-2022_final.pdf

In light of this research, we believe NICE should conduct its own research into public opinion on treatments for rare diseases and assess whether a modular update is required. As future Health Secretary, Wes Streeting should work with NICE to support the implementation of any changes to its methods and processes that are deemed necessary to reflect contemporary social value judgements on rare diseases.

Innovative Medicines Fund

The Innovative Medicines Fund (IMF) is a ringfenced managed access fund of £340 million designed to fast-track patient access to treatments with substantial clinical promise but significant uncertainty around their clinical and cost-effectiveness. The IMF works by providing interim funding for treatments whilst further data can be collected to resolve the uncertainty.

NHS England previously said that the IMF would support patients 'with any condition, including those with rare and genetic diseases',[3] however, since its launch in June 2022, no company has entered into an IMF commercial agreement. The BIA is concerned that as it stands, the principles around which the IMF operates fail to address the challenges faced by many of the most innovative medicines, including cell and gene therapies and treatments for rare diseases. In particular, we are concerned that:

- The requirement for industry to agree to continue funding treatment in perpetuity in the case of a negative NICE recommendation, makes entering the IMF unviable for many SMEs, particularly for manufacturers of treatments for chronic conditions that require life-long treatment.
- The IMF limits the timeframe for data collection during a managed access agreement to five years, which may not allow for robust data to be collected to address uncertainties, especially for many cell and gene therapies with long-term benefit realisation.
- The requirement for a full NICE assessment on both entry and exit of the IMF and will add more time to the overall process and risk delaying patient access to safe treatments. We recommend that drug companies are able to request a 'light touch' appraisal (focused on clinical effectiveness) and direct entry into the IMF, rather than via a full NICE assessment.

The future Labour government should work with NHS England and NICE to review the principles of the IMF to ensure that it is effective in improving patient access to innovative new treatments in the way that was originally intended.

[3] NHS England, 'NHS England announces new Innovative Medicines Fund to fast-track promising new drugs', 2021. Available online via: https://www.england.nhs.uk/2021/07/nhs-england-announces-new-innovative-medicines-fund-to-fast-track-promising-new-drugs/

About the BIA

The BioIndustry Association (BIA) is the voice of the innovative life sciences and biotech industry, enabling and connecting the UK ecosystem so that businesses can start, grow and deliver world-changing innovation.

Our members include start-ups, biotechnology and innovative life science companies, large pharmaceutical companies, universities, research centres, tech transfer offices, incubators and accelerators, and a wide range of life science service providers: investors, lawyers, IP consultants, and IR agencies. We promote an ecosystem that enables innovative life science companies to start and grow successfully and sustainably.

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