How to make sure patients get faster, more equitable access to innovative treatments

A report from the Patient Advisory Council and the ABPI

February 2024

About the report

This report forms part of a programme of work between the ABPI's Patient Advisory Council and the ABPI Board to deliver a programme of shared priorities to improve patient outcomes.

The shared priorities are built around the following core challenges:

- improving the equity and speed of uptake of innovative treatments
- building a culture of research and innovation within the NHS
- tackling equality and diversity in clinical trials
- improving system capacity to support new models of care, pathway optimisation, and adoption of innovation

Foreword by the ABPI Patient Advisory Council

Addressing the human cost of inequity and uptake of innovative treatments

As a collective of charity CEOs working collaboratively with the ABPI, we have long been aware of the human cost of NHS decisions and processes that fail to ensure equitable access and timely uptake of proven innovative treatments. This is a problem that compounds already deep-seated health inequalities across the UK.

This human cost can be significant, and our report illustrates the impact that the inequitable uptake of innovative treatments can have on patients, their families and the health professionals who care for them. More positively, our report also sets out practical examples of how to minimise this inequity and ameliorate patient outcomes and care.

This includes case studies where inequity has been tackled within a therapy or geographical area through political and administrative choices and with the commitment and support of health professionals alongside their patients.

Our aim is to help accelerate and enhance the implementation of government and NHS initiatives already in progress that tackle the challenge of inequity in access to innovative treatment and care.

Common themes are emerging from our case studies and we offer these as lessons for others to reflect on. At local level:

- start with a holistic view of patient need and lived experience to understand and determine what is needed to make any given treatment more likely to succeed
- plan the treatment pathway from the patient perspective to minimise the shuttling and delays between primary and secondary care
- plan the treatment approach using all health professionals – specialist nurses, pharmacists, paramedics, as well as doctors – to make best use of workforce expertise and availability
- use digital, remote, home-based and self-management treatment options alongside in-person care
- beyond the therapies themselves:
  - prioritise clear and open communications with patients
  - consider the physical aspects of access – practicalities and cost for the patient
  - plan the timing, frequency and locations of clinics and diagnostic centres to meet patient needs

At national level:

- explore how to make funding available for the running costs of essential medical equipment that patients can use at home and that is integral to their care and keeps them out of hospital
- review how local decision-making fits with national evaluation and guidance from NICE and its devolved nation equivalents
- align the levers of legislation, incentives, funding and accountability to improve equity of access

Taken together, we believe these steps can show how equitable and timely access to innovative treatment can improve outcomes and the standard of care for patients and save vital resources. We offer these insights to both national and local NHS and government leaders as a contribution to help them address the challenges of equity, uptake, and health inequalities more widely.

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Tackling the challenge of equity and speed of uptake of innovative treatments

The challenge

Uptake of innovative medicines remains stubbornly variable across the UK. 'Transforming lives, improving health outcomes: tackling the true cost of variation in uptake of innovative medicines',[1] a joint report by the ABPI and the NHS Confederation in January 2023, set out the significant variation in uptake at primary and secondary care level, demonstrating the disparity in care that patients receive across England and Wales. Whilst the analysis focused on England and Wales, the Patient Advisory Council and the ABPI recognise the report's themes and findings across all four nations of the UK.

There are three key areas where variation exists along the innovative medicines research and care pathway:

1. during clinical trial stages
2. when the NHS adopts innovative treatments onto formulary/protocol after approval is given by NICE or its devolved nation equivalent
3. at the point of patient access to treatment

At all three stages, variation leads to inequity, and impacts on health outcomes for patients. Tackling inequity is possible at all three stages and helps reduce the postcode lottery still present in our health and care system.

Through case studies, this report sets out the real-life impact of inequitable access to innovative treatments on individuals and communities and exposes the missed opportunities to improve health outcomes for entire cohorts of people living with disease and under-treated conditions. It also presents some examples of best practice in tackling unequal uptake of innovative treatments, illustrating that the solutions to the challenge of inequity lie within our collective control as partners in the health and care ecosystem.

Context

Many major causes of morbidity and mortality disproportionately impact people from lower socio-economic backgrounds. For example, 86 per cent of northern local authorities in England have a lower population life expectancy than the England-wide average. Indeed, access to effective treatment among these populations remains disproportionally variable, despite numerous initiatives to address the issue. The resulting costs from variation in uptake of innovative medicines are significant.[2]

A joint ABPI and PwC report published in May 2022 demonstrated that more equitable use of just 13 medicines in line with NICE recommendations across four treatment areas – stroke prevention, kidney disease, asthma and type 2 diabetes – could bring significant economic benefits to the UK, in addition to wider individual and community benefits for patients, carers and families. For these four medicine classes alone, 1.2 million patients are missing out on innovative treatments.[3]

We know that there are operational pressures on the health and care systems across the UK – continuing or rising demand in most areas, shifting demographic need, workforce capacity and skills, constrained funding, external targets and political expectations. It is difficult for those working every day under these ever-present pressures to look beyond the current challenges.

However, innovative medicines provide a key response to health inequalities and improved public health outcomes. There are ways in which appropriate uptake can be encouraged and spread so that all eligible patients who could benefit have an equitable chance to do so, regardless of their geographical location and socio-economic or cultural background.

The impact of inequitable access on patients at three key stages along the pathway

Clinical trials

Variation in access to clinical trials is common – by their nature, clinical trials are not available in every health system and setting. However, how and where they are designed and set up can add to the built-in inequity of access, as this case study shows.

Case study 1

For a patient with kidney cancer, there was only one treatment option that could potentially work for him. He could only access this innovative treatment through participation in a privately funded clinical trial, which was being run in one hospital in the UK hundreds of miles away from his home. For this trial, patients were hospitalised for the duration of each treatment cycle (four weeks).

This posed serious problems for the patient, who lived in a rural area with his family. His family would be unable to visit during a very important time in the patient treatment pathway. Accommodation and travel costs, time off work and childcare arrangements were barriers to participation that proved prohibitive.

Taking part in clinical trials for innovative medicines is always going to be fraught with worries. Patients in rural areas are disadvantaged in several ways concerning access to early-phase clinical trials, which may be running hundreds of miles away from home. Such participation would add huge logistical, financial and emotional burdens on the patient and their family when the outcome is uncertain and unknown.

There is ongoing work to explore how access to clinical trials can be improved by careful design of studies focusing on key demographics and tackling logistics and practical barriers, as the case study below demonstrates.

Case study 2

Eisai is working with international stakeholders, including patient advocacy organisations and leaders from ethnic minority communities, to develop strategies that will improve the diversity and representativeness of clinical trial participants. Examples of these strategies are:

- selecting study sites using demographic data to maximise a clinical trial's coverage of ethnic minority communities
- reducing barriers to patient access to research by using electronic consent, telemedicine, home health visits and mobile health units, and operating after business hours and at weekends
- tailoring patient education and recruitment campaigns to meet the needs of specific groups to better engage potential participants in those communities.

Adoption of innovation by the NHS

NICE and its devolved nation equivalents such as the Scottish Medicines Consortium (SMC) are responsible for undertaking robust evaluations of the clinical and cost-effectiveness of new medicines on behalf of the NHS.

Following these assessments, the NHS in England is required to make funding available within three months of NICE approving a medicine. NHS organisations across England need to add NICE recommended medicines on to their local formularies before they can be used. This process can take significantly longer than three months and there are opportunities to redesign local formulary processes to speed up decision making. Given NICE approval, additional evaluations of medicines should not be required at local level which reduces the burden on NHS organisations.

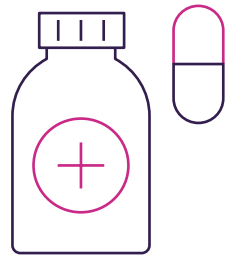
There are similar requirements in Scotland, Wales and Northern Ireland about reimbursement for products which have been approved by NICE or its devolved nation equivalents. The expectation is that local formulary adoption across the UK will be completed in an efficient and timely manner in order to ensure patients can access clinically proven innovations wherever they live.

However, the speed at which local decisions are made can be variable and slow. There are examples where local decisions either limit use or do not adhere to NICE guidance, creating inequity of access for patients, as illustrated in the following case study:

Case study 3

A drug developed to treat paediatric growth hormone deficiency – a rare condition affecting around 5,000 children in the UK – was recommended by NICE in February 2023 with a 30-day funding mandate. However, fewer than 30 per cent of NHS organisations updated their formulary within the timescale. Three months after the guidance was issued still more than half of the formularies had not been updated. While an increasing number of hospitals in England are starting to prescribe the product, only two hospitals have more than 10 patients. The reason for this slow and inequitable access would appear to be administrative delay and financial choices. The impact is felt every day by most eligible patients living with this condition but not able to access the treatment.

As part of the agreement reached under the 2024 voluntary scheme for branded medicines, pricing, access and growth (VPAG), NHS England has committed to the development of a local formulary national minimum dataset. This will increase the visibility of local variation in the implementation of NICE guidance.



The impact of locally made decisions can result in a delay to a nationally endorsed new treatment, leading to unwarranted variation at a system level and potentially impacting the outcomes for patients across the UK.

However, there are also examples where teams from across the system have worked together to develop solutions that genuinely reduce this variation.

Wales and Scotland both have specific funds to improve access to innovative medicines. This case study illustrates the approach in Wales, where the mechanisms of legislation, funding and accountability have been exercised together at the point of approval to improve access to innovative treatments across the nation.

Case study 4

The Welsh Government launched the New Treatment Fund (NTF) in 2017 to give people in Wales fast, consistent access to new and innovative treatments, regardless of where they live and irrespective of what condition is being treated. The Fund offered an additional funding component and changed some legislative directions and powers.

One of the objectives was to drive down the time taken for Welsh health boards to add new treatments onto their formularies. Until the launch of the NTF, health boards had up to 90 days to include a medicine onto local formularies. With the NTF in place the time taken for newly approved medicines to become available for patients in Wales has dropped to an average of just 13 days. One factor in this shift was the 'accountability mechanism' principle established to support the NTF. This introduced a 'comply or explain' requirement for boards not meeting the new standard to notify the Welsh Chief Medical Officer in writing within three weeks of the All Wales Medicines Strategy Group recommendations or NICE Final Appraisal Determination (FAD) recommendation.

By 2020, 226 medicines had been made available under the NTF for more than 100 different health conditions, with very few reports of non-availability, unlike the position before its introduction when there were numerous media reports of postcode prescribing.

The change to processes following the launch of the NTF in Wales has addressed the unwarranted variation in access to newly authorised and Health Technology Assessment-approved medicines to a large extent. This reduced inequity in access demonstrates how national leadership at health system level can reduce health inequalities.

Patient access

Even when a treatment has been approved for use and access to it agreed upon in theory, how it is delivered affects the extent to which access is ensured or restricted. This case study shows the impact of very limited access.

Case study 5

Spinal muscular atrophy (SMA) is a rare neuromuscular disease affecting around 1,340 people in the UK. It has no cure, but in 2019 the first disease-modifying treatment became available through a managed access agreement (MAA) between its maker (Biogen) and NHS England. A multidisciplinary approach plays a crucial part in realising the benefits of the treatment, along with the provision of specialised paediatric and adult SMA centres with the capacity for intrathecal administration (injection into the spinal canal).

However, the tendering process for adult treatment centres only began when NICE published its full guidance in July 2019, leading to a significant delay in set-up. This has been compounded by limited access to the services of radiologists, physiotherapists, occupational health therapists, and speech and language therapists.

All this has limited patient access to the treatment, which is only available at considerable distances – more than 100 miles – or not at all. In a survey, half of the patients surveyed reported that they did not have consistent access to the services required for their treatment plan.

Two years on from July 2019, when the MAA was agreed, only one centre in the south of England had treated a patient. In April 2023, there was still no adult or paediatric centre set up in Wales. While specialised centres for SMA have now been established in Salford, Sheffield and London, these encountered logistical challenges, and although COVID-19 added to the strain on services, many of the issues predate the pandemic.

The impact of this slow and inequitable access is that people with SMA still have no certainty that they will receive any disease-modifying treatment or, if they can access specialised centres, that their treatment will be consistent.

Once an innovative therapy is established as one of the accepted and effective treatment approaches, systemic inequity remains stubbornly present, with geographical disparity challenging the concept of a 'national' health service, as the following case studies on CAR-T and inflammatory arthritis illustrate.

Case study 6

CAR-T cell therapy is a highly specialised cancer treatment. In 2019 it was initially made available for patients in England with a form of leukaemia, then extended to be available to patients with a particular type of non-Hodgkin lymphoma, and subsequently made available for use in Scotland and Wales. The UK currently has 18 specialist sites delivering CAR-T therapies for adult patients.

However, significant variation in access remains for patients from areas of high deprivation, with data showing that in the three years to 2022 only 11.7 per cent of CAR-T-treated patients came from the most deprived quintile of the population, compared with 23.8 per cent of the least deprived quintile. CAR-T contributes to the efforts of the long-term cancer strategy – the NHS's ambition to have 55,000 more people surviving five years or more by 2028 – which is why we need to address the inequitable access to these innovative treatments.

Case study 7

For some people with certain types of inflammatory arthritis, advanced therapies (ATs) can dramatically improve their quality of life. NICE criteria recommend the use of these for patients with severe or resistant disease. Despite CCGs in England at the time being legally obliged to fund advanced therapies recommended by NICE, research undertaken in 2018 found that there was huge variability in decisions on advanced therapies. This has meant that patients at the same stage of disease who should have access to the same NICE-approved AT have not experienced equitable access, simply because of where they live in the country. The full 2018 research paper can be found at:

https://www.researchgate.net/publication/356113872_Inequality_of_access_to_advanced_therapies_for_patients_with_inflammatory_arthritis_a_postcode_lottery

A further study is currently underway to see if any progress has been made since 2018 in improving equity in access to advanced therapies for inflammatory arthritis.

Inequitable access can impact in other ways beyond medicines and equipment. Funding pressures and particular government policy choices can also have a huge impact on an individual's quality of life.

Case study 8

A patient with brittle asthma since childhood was diagnosed with COPD in his twenties. Following a severe infection in November 2019, he had to retire from his job. The patient relied on three machines to help him breathe – an oxygen concentrator switched on 24/7; a CPAP (continuous positive airway pressure) machine used overnight and at certain times throughout the day to prevent carbon dioxide poisoning; and a nebuliser used six times a day for 15-minute periods. The huge increase in the cost of energy in the past year brought the patient fresh worries, doubling his monthly electricity bill and forcing him to switch off other appliances to try to make ends meet. Turning off his medical machines was not an option because he would not survive.

The only financial help available was getting back a small percentage of the cost of energy used from running the oxygen concentrator. There was no financial support available to help with the cost of running the other medical machines that kept him alive, even though they were as vital as medicines and an integral part of his ongoing treatment.

Despite these persistent barriers to innovative treatments, there are examples of how patient access can be made more equitable. Using data to better understand future need and plan deliberately for appropriate and equitable access is one approach taken to tackle the problem.

Case study 9

Lilly was aware of regional variation in the delivery of cancer treatments in England. Its investigations showed that staff shortages – including oncologists, clinical specialists and oncology pharmacists – were a significant barrier to enabling access to patients who would need care in secondary settings. This was coupled with funding constraints in the current operating model (integrated care systems) and a lack of accurate data about the eligible patient population, which made estimating future demand difficult. These challenges are preventing the NHS from fulfilling equitable care to the whole eligible patient population, in line with statutory NICE guidance.

Lilly partnered with The Christie NHS Foundation Trust in Manchester to pilot a new service for breast cancer patients, developing a new model of care to cope with the increased resourcing demand that innovative and complex treatments for breast cancer place on services. They modelled estimated numbers of future patients and identified a need for additional clinic appointments every week. The work then looked at different delivery options, including digital and other technology solutions for areas such as blood testing, toxicity scoring and prescribing, and evaluated them alongside existing delivery options. Healthcare professionals and patients were consulted on their preferences, and costs and efficiency also came into the equation.

While the results of the project are not yet available, they should provide detail on the required capacity to implement a patient pathway that offers equitable access for all eligible patients.

Our case studies show that even once a treatment is well established, speedy and equitable access can still be hard to achieve. Yet improving swift access to the treatment pathway can have a significant positive impact, as the following case study shows.

Case study 10

A gastroenterology service was experiencing increasing referrals and follow-up appointments. The impact was long and frustrating delays for patients with suspected inflammatory bowel disease (IBD). Someone could be stuck in primary care waiting for a referral to be made by the GP, then waiting for the first appointment with a consultant, and then waiting again for an investigation. It was taking over 18 weeks for many patients to get into the system, meaning that many patients were going back to their GP multiple times with flare-ups during the waiting periods, and possibly going to A&E as well. A further impact is that delayed diagnosis of IBD can lead to a higher likelihood of surgery and a poorer prognosis.

As part of the Elective Care 100-Day Challenge Programme, the IBD team established a rapid access clinic led by specialist IBD nurses but also involving other disciplines. The model allows GPs to run early algorithm-based diagnostic tests and refer their patients quickly and directly to the clinic, which runs seven clinics each week. Patients are booked in to attend within two weeks for initial triage with a specialist IBD nurse. A flare-up clinic is also available, and the service includes the facility for patients to have direct telephone access to the specialist nurse.

The clinic took just three weeks to set up and open, and patients have experienced a drop in the time from referral to diagnosis, from 30 weeks to nine weeks. The impact for the NHS is one of decreased costs, better use of nurses and much-improved patient access and provision.

Likewise, taking a patient-centred approach to improving access to care involves planning service delivery in a holistic way, actively supporting patients to benefit from treatment in a setting that works for them.

Case study 11

One hospital provides services to people across a wide area, much of it rural. The IBD service at the hospital was experiencing significant challenges with slow access to clinics and treatment, with many patients having to travel long distances for consultations and their annual review.

The IBD team had established a telephone advice service provided by IBD nurses, which proved very successful. The team continued to develop its services by establishing outreach clinics run by the specialist IBD nurses, still in hospital settings but outside the main hospital site, meaning patients were able to access services much closer to home. The outreach clinic introduced video conference appointments during COVID-19, which also helped patients unable to attend in person. The IBD team felt that holistic care was critical to patients, recognising that different life events – from mental health and family planning to moving locality and care – needed to be tailored to each patient, and were able to offer holistic care as part of its IBD service. Accessing specialist support in this way has proved popular with patients and has reduced hospital admissions and the need for operations, easing pressure on consultant time. This in turn has enabled consultants to focus on more complex cases.

The IBD team now offers follow-up care tailored to and changing with the needs of patients. The service offers regular consultant-led clinics alongside nurse-led clinics, as well as patient-initiated follow-up. Patients can access the care they need wherever they live, in the way that works for them and their condition.

Equitable access to treatments can become more difficult to deliver when a patient has more than one health condition, but the impact of not doing so can increase the risk of worse outcomes. Treating patients with multi-morbidities in a medically joined-up way, ensuring equity of access, is possible, as this case study shows.

Case study 12

About a quarter of people who suffer a heart attack also have type 2 diabetes, and the presence of diabetes more than doubles the risk of death compared to heart attack patients without the disease. Leeds Teaching Hospitals NHS Trust worked with Boehringer Ingelheim to set up an innovative cardiometabolic clinic to tackle this issue. The one-stop clinic provides pharmacist-led clinics, with patients invited to attend six to eight weeks after their heart attack. Patients receive a mix of health education and medicines management support in a clinic setting tailored to their multi-morbidities.

The clinic aims to provide better, more accessible support for patients, helping them to understand their medication and self-manage their diabetes more effectively. The clinic intervention was designed to reduce the need for treatment escalation and hospital stays. The model upskills pharmacists and enables consultant time to be freed up to serve more complex patients.

Patients are given tools to help manage their condition and encouraged to adopt a healthier lifestyle, including using blood pressure monitors and accessing smoking cessation therapy. The clinic offers virtual and in-person support and has led to a reduction in patients needing to access both GP and secondary care for their diabetes. Patients using the service have reported satisfaction that their concerns relating to both heart and diabetes health were being addressed.

Conclusion

As these case studies show, inequity, variation, and delay in access to and participation in clinical trials, the uptake of uptake of treatments approved by NICE and its devolved nation equivalents by local NHS systems, and knowledge of and access to innovative treatments is not an academic challenge for policy discussion. It remains a real issue for thousands of UK people living with a wide variety of common and rare health conditions. When left unaddressed it has a negative impact on the quality of life and health outcomes for patients. By working together, there is the opportunity to overcome many of the barriers and ensure equitable access for all.

With thanks to the following charities and pharmaceutical companies for sharing case studies:

Action Kidney Cancer

Asthma + Lung UK

Biogen

Boehringer Ingelheim

Eli Lilly

Eisai

Gilead

National Rheumatoid Arthritis Society

Pfizer

Takeda

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About the ABPI

The ABPI exists to make the UK the best place in the world to research, develop and access medicines and vaccines to improve patient care.

We represent companies of all sizes which invest in making and discovering medicines and vaccines to enhance and save the lives of millions of people around the world.

In England, Scotland, Wales and Northern Ireland, we work in partnership with governments and the NHS so that patients can get new treatments faster and the NHS can plan how much it spends on medicines. Every day, our members partner with healthcare professionals, academics and patient organisations to find new solutions to unmet health needs.

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