

Breakthrough to Bedside: Overcoming Market Access Challenges for the Next Generation of Healthcare Innovations

Adam Ben Taieb, Senior Consultant at Lightning Health explores why turning new healthcare innovations into accessible patient treatments requires more than scientific excellence - it demands strategic navigation of an increasingly complex market access landscape.



Breakthrough clinical and technological discoveries can be game-changing - whether it's a novel molecule targeting a rare debilitating disease with a high unmet need, or a new technology or software that transforms how and where patients receive care. However, innovation alone is not enough to guarantee patient access. As healthcare systems face mounting financial pressures and rising expenditure, securing patient access has become increasingly competitive and complex.

Manufacturers of innovative healthcare products face multiple hurdles to patient access, including navigating intricate and distinct health technology assessment (HTA) processes, meeting payer expectations for robust evidence that aligns both clinical and economic value, and overcoming potential infrastructure and clinical adoption barriers. This deep-dive explores key challenges manufacturers can encounter during the market access process and offers practical recommendations to help ensure groundbreaking healthcare innovations translate into accessible and sustainable patient care.

1. HTA and reimbursement processes can lag behind innovation

HTA bodies evaluate new healthcare technologies to ensure value for money and added clinical benefit, however, these frameworks often struggle to keep pace with rapid innovation. Traditional HTA processes and value frameworks were designed for conventional pharmaceuticals and are still evolving to accommodate cutting-edge digital health solutions, medical devices, gene therapies and advanced therapy medical products (ATMPs).

This can create uncertainty for innovators, particularly small and medium-sized enterprise (SME) organisations which may lack prior experience with the navigation of assessment pathways. Without clear guidance, evidence generation to demonstrate real-world clinical and economic value to drive sustainable patient access can be challenging to realise.

Case Study: France's Early Device Access Programme

A clear example of an attempt to adapt assessment frameworks is available in France, through the Commission Nationale d'Évaluation des Dispositifs Médicaux et des Technologies de Santé (CNEDiMTS), a dedicated HTA pathway for medical devices and telehealth services. France also has a "Forfait Innovation" programme, which grants early access to promising technologies while further evidence is generated. However, uptake remains limited - only 18 products have been enrolled since 2009 - and timelines even for early access remain long. The SonoCloud-9 device, a first-in-class medical device that uses therapeutic ultrasounds to treat severe brain disorders and has been in development for over a decade, was recently accepted into the programme.^{1,2}

Case study: Digital Therapeutics in Europe

Despite growing evidence of clinical and economic value, digital therapeutics face fragmented assessment and reimbursement across Europe, often falling outside of established HTA frameworks with limited clarity on evidence standards and available funding pathways. Even in markets where a more mature assessment framework is in place for digital therapeutics, consistent evaluation is limited. An analysis conducted between 2022 and 2024 found that only two patient facing applications have undergone evaluations across multiple countries; Deprexis (indicated for depression) in France, Germany and the UK, and Velibra (indicated for anxiety) in the UK and Germany and France.³

2. Pricing pressures and budget constraints

Although new therapies may offer improved outcomes and long-term cost offsets, upfront expenses and infrastructure investment can be significant. Payers and HTA bodies frequently benchmark innovations against cheaper alternatives, including generics and legacy devices, creating a "price squeeze" that often limits commercial viability.

Budget pressures are exacerbated by rising healthcare spending and global cost-containment policies, such as the US “Most Favoured Nation” pricing policy, which further impacts pricing negotiations and commercial potential.

Case study: Gene Therapy Pricing Challenges

This challenge is illustrated by the European access landscape for Bluebird Bio’s Zynteglo, a gene therapy for transfusion-dependent β -thalassemia. Despite the clinical promise, the US\$1.8 million price tag led to payer resistance in Europe, particularly Germany, where negotiations broke down after insurers offered less than half the list price, citing uncertainty about long-term outcomes and limited differentiation from the existing standard of care (SoC). Without commercially viable pricing, Bluebird Bio withdrew from the European market.^{4,5} Similar obstacles arose in the US for Lyfgenia for the treatment of sickle cell disease, priced at US\$3.1 million, competing against lower cost alternatives.⁶

Case Study: Robotic-Assisted Surgery Costs

Despite evidence of improved outcomes and efficiency, hospitals can spend between US\$1,000 and US\$4,000 more per robot-assisted surgery compared with endoscopic minimally invasive or open procedures, in addition to the initial purchase and ongoing maintenance costs of the robotic systems.⁷ Moreover, per-procedure costs for robotic surgery often only become cost-effective at high surgical volumes.⁸ Leasing models can help mitigate such financial barriers, alongside delivering robotic surgeries from specialist centres where economic gains align with high volume service.

3. Endpoint misalignment between regulatory and payer expectations

Clinical trial designs, including endpoint selection, comparator, and duration of follow-up, often remain tailored for regulatory approval, without consideration of likely payer/HTA expectations. While regulatory authorities may accept analysis that informs a positive benefit-risk ratio, HTA bodies typically require evidence that demonstrates clinically meaningful incremental patient value vs. SoC, alongside likely considerations of economic value. This misalignment can leave health economics and outcomes research (HEOR)/ value & access teams with datasets that do not align with the evidence requirements for HTA authorities, and may underrepresent the holistic value of an innovative new treatment.

Case study: Luxturna Gene Therapy

When developing Luxturna, a one-time gene therapy for inherited retinal dystrophy, Spark Therapeutics faced challenges due to the lack of existing clinical endpoints capable of adequately measuring functional vision improvements in this patient cohort. To address this, the Multi Luminance Mobility Test (MLMT), an innovative endpoint measuring functional vision, was developed and utilised as the primary endpoint in the pivotal trial, while also being validated in a concurrent study.⁹ The endpoint was ultimately accepted by both regulatory bodies and HTA authorities, supporting reimbursed patient access across a broad range of global markets.

Case study: Eteplirsen for Duchenne Muscular Dystrophy

Eteplirsen received Food and Drug Administration (FDA) approval in the US based on increased dystrophin production, a surrogate biomarker, rather than increased muscle function or quality of life (QoL) data. Many US insurers deemed this endpoint insufficient to prove clinically meaningful benefit, resulting in restrictive coverage policies and limited access.¹⁰ Additional video evidence demonstrated patient function improvements; however, this was not validated meaning its use was limited, underscoring the need for endpoints that resonate with both regulators and payers.¹¹

4. Infrastructure and service provision gaps

Innovative healthcare solutions often require new care pathways, specialised infrastructure and/or trained personnel. Lack of readiness in healthcare systems can delay or limit adoption, even after reimbursement is secured. Budget constraints and cumbersome funding processes may also prevent providers from investing in new facilities, equipment, or staff required to deliver the innovation effectively. This is particularly acute for rare or orphan indications where clinical services can be non-existent, for novel ATMPs with complex distribution and storage requirements and for digital technologies dependent on robust IT infrastructure.

Case study: Alzheimer’s Disease Therapies

Despite a high clinical need, the National Institute for Health and Care Excellence (NICE) in the UK did not recommend lecanemab or donanemab due to perceived limited clinical benefit and high costs associated with infusion and monitoring infrastructure, increasing the anticipated incremental cost-effectiveness ratio (ICER) beyond the accepted threshold.^{12,13} The Haute

Autorité de Santé (HAS) also declined to grant early access for lecanemab in France, due to (amongst other reasons) operational constraints around the process required to ascertain eligibility, including genetic tests, and safety monitoring requiring frequent magnetic resonance imaging (MRI) scans.¹⁴

Case study: Continuous Glucose Monitoring Devices

Initial uptake of continuous glucose monitoring for diabetes in Italy and Spain was hindered by lack of trained healthcare professionals to interpret data, limited integration with existing IT systems and fragmented frameworks for evaluating and funding digital health technologies. This highlights the importance of system readiness for digital and monitoring innovations.^{15,16}

5. Clinical adoption barriers

Regulatory approval and reimbursement do not guarantee immediate clinical uptake. Provider inertia, lack of familiarity or confidence in clinical data, absence of updated clinical guidelines and insufficient incentives often delay adoption. This is especially pronounced in rare diseases where clinicians may lack specialist knowledge required to administer a new treatment or for new medical equipment or software that can require a high standard of technological dexterity and training for implementation.

Case Study: Cardiovascular Device Uptake in Global Markets

The WATCHMAN Left Atrial Appendage Occlusion device and the Impella Percutaneous Ventricular Assist device experienced faster adoption in the US than Europe, driven by financial incentives aligned with physician and hospital reimbursement. While higher reimbursement is available for performing atrial fibrillation ablation compared with WATCHMAN implantation, WATCHMAN devices are more efficiently implanted, allowing physicians to perform increased billable procedures with subsequent reimbursement.¹⁷

Case Study: Multiple Sclerosis Therapies in Germany

Despite several high-efficacy disease modifying therapies (DMTs) for multiple sclerosis receiving favourable benefit assessments, many clinicians continued to prescribe lower-efficacy treatments due to concerns about safety and long-term outcomes. Analysis of retrospective claims-data between 2017 and 2022 found only 14.8% of new patients started on high-efficacy DMTs for multiple sclerosis.¹⁸

Think ahead - align stakeholders from discovery to delivery

Navigating the journey from breakthrough innovation to patient access requires a strategic, multi-faceted approach. While the road to access for healthcare innovations that do not fit the standard framework for evaluation and reimbursement is challenging, the accelerating pace of innovation and the realisation of concepts previously considered science fiction, demonstrates the necessity of defining and implementing feasible pathways for patient access.

At core, the requirement to demonstrate the clinical and economic value of a new product that satisfies market specific evaluation frameworks is critical. Solidifying the initial demonstration of a clinical/safety benefit with a data generation strategy that supports the patient relevant incremental clinical, QoL and economic value for a new healthcare technology compared to existing treatment options (over a clinically relevant duration considering the natural history of the disease) will be highly important.

Furthermore, prior stakeholder engagement is essential to ensure sustainable pricing models that are commercially viable for the manufacturer, whilst being economically sustainable for healthcare systems. This will require careful pre-launch planning to determine feasible pricing potential/ models for national payer/insurer negotiations (including innovative contracting models that mitigate financial risks for healthcare systems, whilst minimising bureaucratic complexity). In this context, a collaborative approach from the manufacturer to offer a range of financial or outcomes-based managed entry options for payers to consider at the point of launch (based on feasibility/affordability within their healthcare system) is likely to be increasingly important.

Finally, country specific launch planning for innovative new treatments must consider the available funding pathways, logistics requirements and infrastructure for treatment delivery (covering patient diagnosis, treatment delivery, and ongoing follow-up/management). Where new pathways/ services may be required (or significant adaptations made to existing routes), pre-launch engagement between manufacturers, clinical stakeholders, and budget holders will be highly important to ensure alignment regarding the practical requirements for adoption.

By anticipating and addressing challenges related to evidence generation, pricing, healthcare infrastructure and clinical adoption, healthcare innovators can improve their chances of successful and sustainable market access, ensuring transformative therapies reach the patients who need them most.

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