Patient access in the real world: Challenges and opportunities


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Is your portfolio future proof? When bringing a drug to market, having the right data for both regulatory and reimbursement assessments increases your chances of success. However, the outcome is also sensitive to external factors and recognising potential disruptions should be an integral part of your plan.

The pathway to patient access for new medicinal products is dependent on demonstrating a positive risk/benefit profile for regulators and communicating the value of the product for payers using the best available evidence. Health technology assessment (HTA) agencies aim to provide recommendations on reimbursement of new medicines and address uncertainty for payers to support decision making. Pharmaceutical companies benefit from coordinating the development programme to meet the needs of both regulatory and HTA agencies and ensuring that they can present the appropriate data to support both reimbursement and approval.

"Drug developers must apply a holistic stakeholder perspective to maximise product value, starting by addressing key commercial requirements early during clinical development," Mel Walker, Advisory Board Member at NDA says. "Think of it as future proofing your portfolio, ensuring that you have assets that can be successfully commercialised globally."

The regulatory agency focuses on the benefit/risk of medicines, which is based on results from clinical trials provided under ideal circumstances, whereas an HTA agency focuses on the additional value the product brings in the real world. The value assessments they carry out focus on the relative performance of a technology (such as safety, effectiveness, and cost-benefit) against currently available clinical options. While regulators may accept short-term or surrogate outcomes, payers prefer long-term clinical outcomes compared with appropriate standard of care options.

A high degree of uncertainty reduces the willingness to pay for new technologies. Decisions on reimbursement of medicines must strike a balance between maximising health outcomes in the context of constrained healthcare budgets. Healthcare decision-makers rely heavily on HTAs to achieve greater value for money, and assessments determine whether the intervention is relevant for the proposed patient population and what the clinical and economic benefits as well as the costs might be – evidence generation efforts must collect these data.

Macro-environmental challenges and opportunities

No article would be complete without mentioning the COVID-19 pandemic, which will of course drive increased healthcare budget pressures. This will likely place a great emphasis on cost and price, and development strategies will need to be even more attuned to demonstrating value recognised by payers. However, the global health crisis has proven to be an accelerator for collaborations and even more harmonised procedures to get medicines to market while preserving proper standards for efficacy and effectiveness. Several regional initiatives had already been established before COVID-19 and these are likely to develop further to strengthen capacities and reduce redundancies through HTA and payer collaboration.

In the UK, regulators, HTA, payers, and research groups have joined forces early on to advise companies on their 'target product profiles' and clinical trial designs to ensure that optimal data is generated for regulatory approval and to support favourable reimbursement outcomes. The Innovative Licensing and Access Pathway (ILAP) provides a platform for collaborative working between developers, authorities, and regulatory bodies in the UK and illustrates how collaboration may help to accelerate the path to market.

The move to introduce a single European process for the Relative Effectiveness Assessment (REA) of pharmaceuticals is another example of collaboration. REA is a framework to harmonise HTA information in multiple jurisdictions to establish a standardised HTA process supporting pharma companies to efficiently communicate the value of their products across healthcare systems. By combining resources, the hope is that the efficiency of the HTA assessment increases and national processes may be simplified but whether this is the reality remains to be seen.

The adoption of new technologies is changing the clinical trial landscape, and the digitalisation of data collection, patient recruitment, and adherence could significantly improve the process and avoid delays. The information technology infrastructure enables the storage and transfer of large amounts of data. By integrating health datasets from clinical research with biobanks, pragmatic trials can be conducted, increasing the value of the data, unlocking new avenues of research, and providing information that will be useful to regulators, HTA, and payers.

US drug pricing has been heavily debated over the last couple of years, as the cost of prescription drugs in the United States is considerably higher than in other developed countries. There is no agency with legal authority to regulate prices, and value-based pricing is not currently applied. Transformation of pricing approaches require changes to existing laws and regulations and would, if initiated, take a few years. The pharma industry will be following these changes carefully because the possible repercussions are potentially huge. If the US market trends move a little closer to Europe over time, meeting the needs of European markets will be important to ensure a future product launch that is globally viable.

Regulatory agencies have established several programmes to shorten the approval timelines for new therapies, which reduces the availability of data at launch. Drugs approved via an expedited pathway may have fewer data available at the time of initial approval, which is almost diametrically opposed to what HTA bodies require to inform decision making.

Awareness is the key

These environmental factors all emphasise the need to start thinking about HTA and commercial requirements early in the development process and build a strategy that meets the needs of both regulators and payers.

Yngve Mikkelsen, Advisory Board Member at NDA explains: "To successfully transform lives, pharma companies must formulate clear strategies laying the foundation for appropriate evidence generation and value communication addressing relevant stakeholders needs."

Developers need to shape target product profiles with a broader set of stakeholders in mind. A robust strategy is key for company success regardless of whether the goal is a planned exit, to partner, or to launch. Real-world evidence is increasingly important for answering payer challenges especially in the case of expedited regulatory approvals. Drug development is a lengthy process, and new drugs will be launched in uncertain future market environments. Evidence generation approaches need to consider future stakeholder requirements while including appropriate measures to capture both patient and economic benefits in addition to relevant clinical outcomes.

Recommendations

Start early by shaping your target product profile. By targeting the data needed for regulators and HTA bodies, you prepare for downstream success from the very beginning.

Engage early with payers as well as regulators. Remember the payer is dominant in most markets.

Be aware of existing clinical and economic guidelines and policies and how your product fits in.

Future proof your asset or portfolio. The external environment into which drugs are launched is also changing. Monitor the environment and plan accordingly.

Keep your options open. Launching in multiple jurisdictions may not be what you initially planned for but, by keeping a broad perspective, you will hit the ground running.

For any questions regarding how to future proof your portfolio contact:
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