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Overcoming Challenges in the Access and Launch of Innovative Healthcare Products

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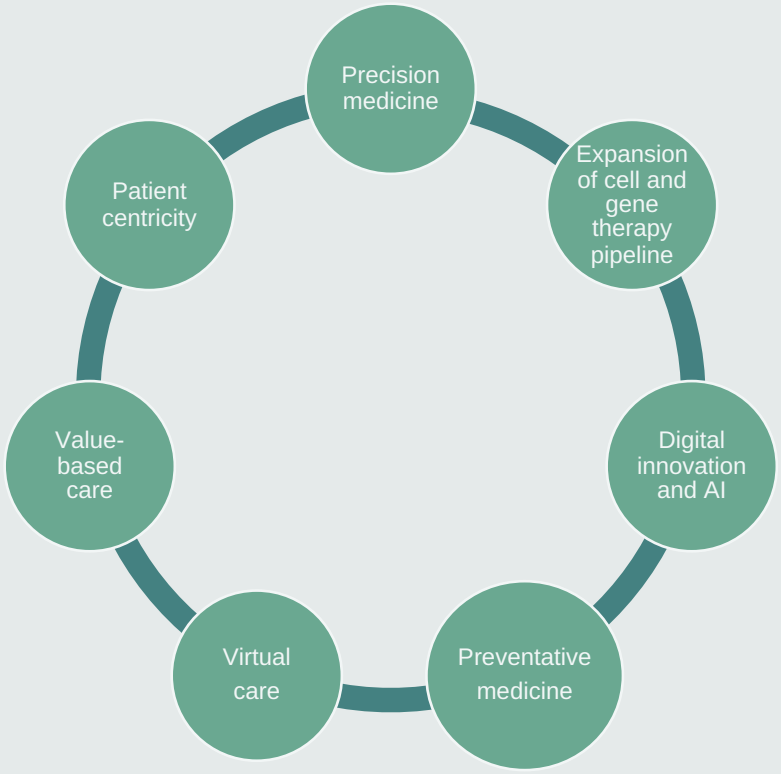
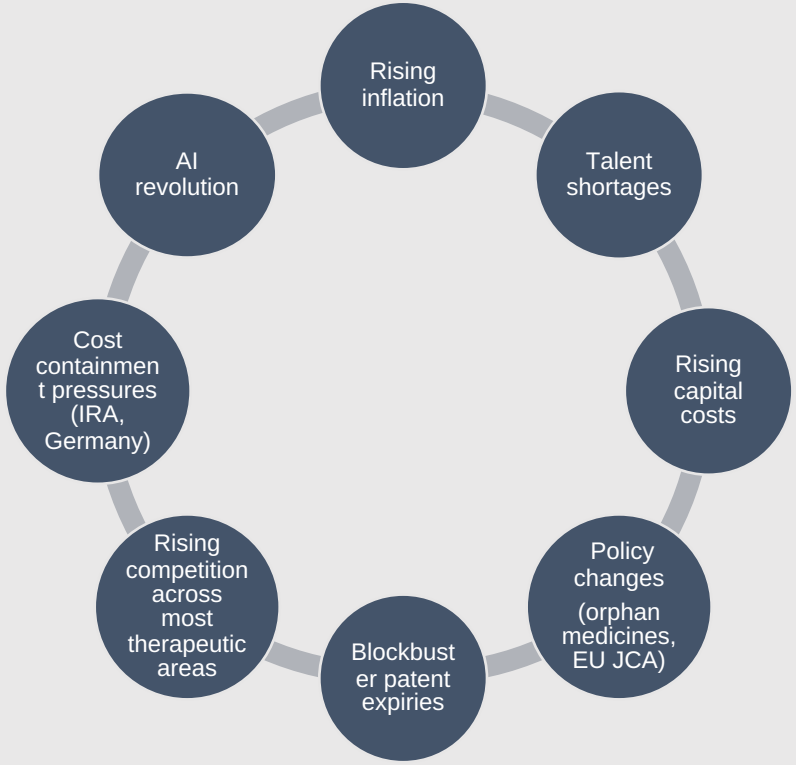
Manufacturers need to navigate a rapidly changing and complex access environment due to the changing healthcare landscape and disruptive macroenvironment

Innovative healthcare solutions present unique access challenges as the existing frameworks need to adapt



Disruptive healthcare landscape and macroenvironment

Challenging macroenvironment means manufacturers will need to operate with lower costs and shorter timelines



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Challenging access and launch landscape due to evolving regulatory and access pathways, policy changes, cost pressures, high evidence requirements and growing competition

Regulatory uncertainty and variability

Obtaining regulatory approvals, licenses, and certifications can be complex and time-consuming for innovative healthcare products (digital/AI, orphan medicinal products, cell and gene therapies) due to diverse regulatory frameworks

High payer requirements for reimbursement and cost pressures

Securing favourable reimbursement coverage and pricing and broad access for innovative healthcare products can be challenging due to high evidence requirements, evolving policies, cost pressures, and payer dynamics

Increasing competition and uncertain long-term benefits can impact adoption

Stakeholder engagement and differentiation are crucial for entering and maintaining a market presence in the face of growing competition across various therapeutic areas (TAs)

Navigating the regulatory pathways for innovative healthcare products can be complex, expensive and time-consuming due to the high evidence requirements

The regulatory pathways for orphan medicinal products (OMPs) and cell and gene therapies (CGTs), and meeting the stringent requirements for approval, can be complex and lengthy

- Lack of harmonized regulations for precision medicines and orphan drugs across countries
  - Need to understand specialized regulatory pathways with expedited timelines (e.g. FDA's breakthrough therapy designation for precision medicines or the orphan drug designation for drugs targeting rare diseases)
- Generating enough data for approval is challenging, as these therapies are designed for patient populations with limited numbers
- The guidelines for biomarker validation may not always be clear and vary across different agencies

The pathways for digital and AI healthcare products can be lengthy, expensive, and complex, especially if there is no clear regulatory pathway in place

- Digital health products differ from established medical technologies and are subject to diverse regulatory frameworks across different countries or regions
  - Digital/AI healthcare products need to comply with regulations specific to medical devices, which includes a rigorous review process to ensure the safety and effectiveness of the product. Digital therapeutics do not fit into existing regulatory frameworks as they are not drugs or medical devices and need to provide robust evidence of safety and efficacy (i.e. well-designed clinical trials)
- Digital health technologies exist at the intersection of healthcare and technology, presenting regulatory complexities. Key considerations include data privacy, software validation, risk classification, clinical evidence requirements, and jurisdictional variations.

Securing favorable reimbursement coverage and pricing for innovative healthcare products can be challenging due to high evidence requirements, cost pressures, and evolving policies

High evidence requirements (clinical and economic) impacts reimbursement and pricing of combination therapies, OMPs and CGTs	• Orphan drug and CGT developers need to generate robust data on clinical outcomes, patient-reported outcomes, and health economic outcomes to justify their prices and secure reimbursement • Cost-effectiveness considerations may result in low reimbursement rates for orphan drugs and CGTs • HTAs often limit CGTs' price potential and do not recommend broad adoption due to a lack of comparative data, small patient populations, and a lack of long-term data (e.g., Bluebird struggled with HTAs in Europe) • Cost-effectiveness methodologies pose a significant barrier to accessing new oncology combinations
Implementing and scaling alternative payment models for innovative high-cost products can be challenging	• CGT and orphan drug developers need to consider innovative pricing and contracting models, such as risk-sharing agreements, outcomes-based contracts, and annuity payments, due to uncertainty in clinical/economic evidence
Unpredictable requirements for digital therapeutics/products	• There are challenges in the harmonisation of evidence requirements and lack of value assessment processes. No standardised or specific reimbursement pathway for DTx in most countries
Pricing pressure due to increased competition, cost containment measures and blockbuster patent expiries	• Pricing pressure due to: • cost containment measures in the US and European markets (e.g. the Inflation Reduction Act of 2022 in the US, the GKV Financial Stabilisation Act in Germany) • entry of generics/biosimilars (several blockbuster products, including Humira and Keytruda, are set to lose their patents) • Increasing competition in several therapeutic areas • increasing demand for pricing transparency from payers and patients
Implications of evolving policies	• Pharmaceutical companies need to understand the short and long-term implications of the evolving policies (IRA 2022, EU JCA, etc)

Effective stakeholder engagement and differentiation are crucial for successfully entering and maintaining a market presence in the face of increasing competition

Increasing competition in several therapeutic areas means differentiation is crucial for adoption	• There is increasing competition in several therapeutic areas (e.g., oncology, diabetes, rheumatology, cardiovascular disease, neurology, etc). • Differentiation in competitive therapeutic areas requires a combination of innovative product development, strong clinical evidence, value-based pricing and reimbursement strategies, targeted marketing, and collaborations with key stakeholders to understand their needs, to stand out from the competition.
Stakeholder engagement is critical to gaining support and adoption of innovative products (DTx, long-term benefits not known)	• Building relationships and engaging with key stakeholders, including healthcare professionals, opinion leaders, patient advocacy groups, and payers, is critical for understanding their needs, influencing decision-making, and gaining support for innovative products (e.g. cell and gene therapies and DTx)
Patient advocacy and engagement to support the adoption of orphan medicinal products	• Patient advocacy groups can help raise awareness about rare diseases and the need for effective treatments and actively engage with policymakers, healthcare providers, and drug manufacturers to promote the development, approval, and access to orphan medicinal products.
Not securing reimbursement/having a high price tag can impact adoption	• Reimbursement continues to be a challenge for gene therapy manufacturers, as high prices and uncertainty around long-term outcomes make it difficult for payers to cover the costs. • It is currently unclear how alternative payment models have helped in the adoption of gene therapies with high price tags (e.g., Luxturna and Zolgensma) • There is inadequate funding, and DTx reimbursement pathways do not ensure uptake

Manufacturers need to partner with key stakeholders to lead changes to value assessment and reimbursement models to improve accessibility to innovative healthcare solutions

Understanding regulatory and payer requirements for digital healthcare products	• Early engagement with regulators and payers is crucial to understand the evidence requirements for digital healthcare products. This will help prepare the necessary evidence required for approval and reimbursement.
Develop new value frameworks that align with the technology	• Collaborate with regulatory agencies and payers to create frameworks that facilitate the fast-track approval of CGTs (e.g., using surrogate or intermediate endpoints, incorporating innovative study designs, and real-world evidence) • Pharmaceutical companies should present strategies to create new value frameworks that can showcase the wider benefits of CGTs in comparison to traditional treatments • Increasing DTx uptake will require collaborative efforts between policymakers, HCPs, and companies and enhancing the education and experience of HCPs and patients.
Overcoming reimbursement/ pricing hurdles for high-cost therapies	• Collaborate with payers to develop innovative reimbursement models that align with product value and patient outcomes • Collaborate with key stakeholders to enhance the infrastructure necessary for data collection and outcome monitoring for cell and gene therapies
Understanding and adapting to the implications of evolving policies	• Pharmaceutical companies will need to carefully monitor uncertainties surrounding the implementation and interpretation of the EU JCA regulation • To be prepared for the implementation of IRA, manufacturers need to understand the short and long-term implications of the law