TODAY AT EPA

Our overview of:

the keynote and insights emerging from the global market access landscape

WORLD EPA CONGRESS
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Remap

KEYNOTE

CHANGING LANDSCAPES AROUND MARKET ACCESS AND PRICING: GLOBAL PERSPECTIVES

During Tuesday's keynote presentation by a Vivian Mendonca, Senior Vice President and Global Head of Value, Evidence and External Affairs at Menarini Group, crucial challenges and opportunities in market access and pricing for innovative medicines were discussed, with a special focus on evolving landscapes in biomarker complexity within oncology and strategies to combat global health threats in healthcare.

The presentation highlighted four key challenges faced in pricing innovative treatments:

- Balancing 'fair' innovation rewards with cost-effectiveness considerations.
- Adapting pricing strategies amidst intense competition and evolving market dynamics.
- Addressing uneven funding distribution and changing governmental priorities.
- Managing the shortening of exclusivity periods.

In the realm of rare diseases and interventions combating antimicrobial resistance, a lack of harmonisation in reimbursement pathways across different markets emerged as a significant barrier to patient access. Furthermore, current investment levels were deemed insufficient to drive necessary innovation and product launches in these critical areas where effective treatments are urgently needed to mitigate patient suffering.

Biomarkers also emerged as a pivotal area experiencing rapid development. However, there exists a substantial lack of awareness and understanding regarding the potential value biomarkers offer, leading to access challenges. Biomarker testing, particularly in oncology and cardiovascular diseases, is gaining importance. Yet, significant regulatory and reimbursement variations across markets complicate matters.

To improve patient access to biomarker testing, it is imperative to develop cost-effective testing methods and demonstrate the long-term cost savings associated with biomarker utilisation, thereby showcasing their value to healthcare systems. Collaboration and partnerships are necessary to educate stakeholders and promote access to biomarker testing, with patient advocacy groups playing a crucial role in this endeavour.

In conclusion, effective coordination among internal value teams, early engagement with key stakeholders, and proactive involvement in shaping supportive policies are key strategies to maximise pricing and access opportunities for innovative treatments. This proactive approach is essential in navigating the evolving landscapes of market access and pricing while ensuring patient needs are met with timely and affordable access to life-saving innovations.

The key takeaway from the presentation on market access and pricing challenges for innovative medicines is the critical need for stakeholders to align innovation rewards with cost-effectiveness considerations. Balancing the fair rewards for innovation with the affordability and accessibility of treatments is paramount in ensuring sustainable access to life-saving therapies for patients worldwide.

RARE DISEASE TRACK

PATIENT ACCESS IN RARE DISEASES: ADDRESSING CHALLENGES THROUGHOUT THE PATHWAY

INTRODUCTION

Investment in rare diseases is a topic marked by shifting priorities and complex dynamics. While there is a growing inclination towards equitable distribution of funds between rare and common diseases, the reality often sees a tilt towards financing common diseases when costs escalate. This shift is influenced by challenging negotiations on pricing between payers and the pharmaceutical industry, where payers seek optimal value for their investment. Thus, it is crucial to consider factors such as disease severity and life-threatening nature alongside other considerations.

THE DISCUSSION

Currently, among EU nations, Germany, Italy, and France lead in orphan drug approvals by the EMA, with an average of 84% success. Germany notably stands out with a swift average time of 89 days between approval and patient access, followed by Denmark at 243 days. However, this success also highlights a significant challenge associated with orphan drugs that demands immediate attention.

Looking forward, there is optimism surrounding an upcoming reform set for parliamentary debate in May 2024. This reform aims to foster innovation and improve access to medicines, particularly addressing high unmet medical needs in the rare disease space.

Several data challenges exist along with potential solutions:

- Study design issues, such as insufficient randomised trials, could be addressed by leveraging more real-world evidence.

- Lack of defined clinical measures or validated endpoints might be mitigated by using surrogate endpoints.
- Absence of comparative data due to heterogeneous practices and lack of existing treatments could be addressed through indirect treatment comparisons.

Accurately capturing the patient journey accurately is another critical yet neglected aspect in the rare disease domain. Patient perspectives are vital for empowering and improving their quality of life, especially in scenarios where a 10-day waiting period for drug access is deemed too lengthy. Some rare diseases exhibit daily progression, leading to irreversible damage by the time the 10-day mark is reached.

Collaboration among stakeholders is essential to integrate patient advocacy at an earlier stage. While clinicians require education, a deeper comprehension of patient challenges is crucial identifying the appropriate clinician for diagnosis and subsequently referring them to the right specialist.

THE KEY TAKEAWAYS

1. Supporting orphan drugs incurs higher costs compared to drugs for common diseases, leading to challenges in attracting investments due to perceived lower returns on investment.

2. Across the European Union, a significant number of member states require over 400 days for patients to gain access to their orphan drugs. This indicates that market access for rare diseases lags considerably behind that of common diseases.

3. Resolving data challenges, such as study design issues and lack of comparative data, has the potential to accelerate patient access to orphan drugs, benefiting both patients and stakeholders.

While early access programs assist vulnerable patients and generate real-world evidence, they also pose challenges by potentially extending access times for non-participating patients. Balancing these factors is crucial for devising effective solutions that benefit all stakeholders.

DIGITAL TRANSFORMATION TRACK

IMPROVING ACCESS TO DIGITAL THERAPEUTICS IN EUROPE

INTRODUCTION

Digital Therapeutics (DTx) offer evidence-based therapeutic interventions through software programs aimed at preventing, managing, alleviating, or treating diseases. They have transformative potential for patients, empowering them with at-home treatment options, fostering engagement, and enabling holistic management. Healthcare providers (HCPs) benefit from improved or alternative therapeutic options and enhanced data sharing with patients. From a healthcare system perspective, DTx target unmet needs, reduce overall burdens, and integrate generated data seamlessly.

THE DISCUSSION

Despite their immense potential, DTx adoption in Europe remains limited. Only Belgium, Germany, and the UK have established national value assessment frameworks, with reimbursement pathways currently operational only in Belgium and Germany. Germany stands out for pioneering clear pricing and reimbursement frameworks for Digital Health Applications (DiGA).

Four primary barriers hinder DTx market access:

1. Lack of harmonisation in regulatory requirements across EU Member States, leading to varied interpretations and a lack of precedent for risk classification.
2. Challenges in evidence requirements and the absence of standardized value assessment processes for DTx across most countries.

3. Absence of a standardized access pathway, complicating market entry strategies.
4. Insufficient funding for DTx, coupled with low awareness and recognition of their potential value, further hampering uptake.

THE KEY TAKEAWAYS

1. Value assessment processes for DTx must be tailored, predictable, and consistent, incorporating a comprehensive evidence portfolio, including real-world data.

2. Payers should adopt flexible approaches allowing provisional access while additional data is collected, facilitating faster market entry.

3. Collaboration among policymakers, healthcare providers, and companies is essential to drive DTx uptake, streamline regulatory processes, and improve overall access for patients.

By addressing these key takeaways, stakeholders can work towards unlocking the full potential of DTx, ensuring they become integral components of modern healthcare systems across Europe.

HTA TRACK

EU HTA - HOW TO ENSURE STAKE-HOLDER ALIGNMENT ON A NATIONAL LEVEL TO ACCELERATE BROAD PATIENT ACCESS

INTRODUCTION

The recent publication of the first implementing act of the joint EU Health Technology Assessment (HTA) has brought about significant uncertainties among stakeholders. One of the main challenges and surprises has been the substantial volume of data required, raising questions about the actual reduction in workload that was initially anticipated. Additionally, the extremely tight deadlines are a major concern, especially for manufacturers navigating this new landscape.

THE DISCUSSION

Further acts are expected to be published in the 3rd or 4th quarter of 2024, posing challenges for manufacturers. It appears that Germany and France will bear the brunt of the workload, with assessment processes resembling those seen in AMNOG (the German pharmaceuticals market access system).

Manufacturers express concerns about the lack of collaboration in the EU HTA process and question whether there will be meaningful opportunities for engagement moving forward. Unlike EUnetHTA, which provided clear stakeholder engagement instructions, the joint EU HTA process lacks such clarity.

Small and mid-size companies face difficulties in gathering local clinical data, particularly if they lack a presence in specific localities. Coordinating local adoption is challenging, although these companies may find it easier to restructure to align with EU HTA requirements. Dependence on consultancies for knowledge and assistance in navigating the EU HTA is also noted.

The European market may become less attractive for investment due to the evolving and challenging environment. Questions arise about the timeliness of the JCA (Joint Clinical Assessment) report if manufacturers opt to launch their products in different countries at varying times.

THE KEY TAKEAWAYS

1. **One voice, big impact**
   The joint EU HTA creates a unified approach across all 27 member states, offering a streamlined process that can significantly impact manufacturers' ability to create global clinical development plans.

2. **Market access planning**
   Manufacturers must consider market access strategies early in their clinical trial planning to align with EU HTA requirements effectively. This strategic approach becomes increasingly crucial with the introduction of the joint EU HTA.

3. **Local affiliates and adaptation**
   Establishing and leveraging local affiliates is essential for manufacturers navigating the EU HTA landscape. Focusing on local capabilities can help limit the number of product information and clinical evidence queries (PICOs) encountered during assessments, streamlining the overall process.

MARKET ACCESS TRACK

ACCELERATING ACCESS TO INNOVATIVE MEDICINES IN THE UK – KEY STRATEGIC THEMES FOR GLOBAL ACCESS LEADERS

INTRODUCTION

Today's panel discussion on access to innovative medicines in the UK brought together experts representing payer, clinical, company, and health economist perspectives. Delving into the complexities of the UK market, the panel explored challenges and opportunities for launching new medical products effectively.

OPENING REMARKS AND MARKET PERCEPTION:

The panel unanimously acknowledged the UK market's perceived complexity, often deemed a 'painful' landscape for manufacturers developing launch strategies. However, amidst these challenges, there was a shared optimism about the market's potential. The robust data infrastructure and a growing desire for collaboration among stakeholders—companies, payers, healthcare professionals, and patients—were highlighted as key factors offering opportunities for successful product launches.

PAYER PERSPECTIVE AND POLICY IMPROVEMENTS

Rob Kettel from NHS England provided an optimistic outlook, citing policy improvements that facilitate timely patient access to innovative medicines. Noteworthy advancements include shortened timeframes between NICE assessments and final guidance publication, as well as enhanced capabilities in creating innovative commercial agreements with manufacturers. Initiatives such as the VPAG and Innovative Medicines Fund aim to foster innovation and competition, benefiting both patients and taxpayers.

HEALTH INEQUITY & COVID-19 IMPACT

The panel identified health inequity across UK regions as a significant barrier to uptake, exacerbated by the COVID-19 pandemic. Christopher Kipps discussed the introduction of secure data environments (SDEs) to streamline national and regional data collection, aiding in identifying access disparities. Strategies leveraging collected data can effectively boost uptake across diverse regions.

HEALTH ECONOMIC PERSPECTIVE & COST-EFFECTIVENESS

A focus on cost-effectiveness thresholds and strategies like preventative medicine was emphasised from a health economic standpoint. While cost-effectiveness thresholds vary regionally based on quality-adjusted life years (QALYs), they are applied nationally to ensure efficient resource allocation. Innovative approaches such as vaccines and genomics hold promise in mitigating healthcare inequities.

EU HTA PROCESS AND ACCELERATING ACCESS

The potential impact of the EU Health Technology Assessment (HTA) process on UK access was discussed. Clear frameworks for real-world evidence (RWE) and managed access agreements were identified as accelerators for access in the UK compared to the rest of Europe. However, caution was advised, emphasising the need for robust evidence packages to maintain confidence among stakeholders.

KEY TAKEAWAY

In conclusion, while the UK market poses challenges, initiatives like the VPAG and Innovative Medicines Fund incentivise innovation and collaboration across disease areas. Ongoing education of healthcare professionals and patients is crucial to mitigate healthcare inequities and ensure swift uptake of new therapies.

The panel discussion highlighted the importance of a cohesive approach among stakeholders to navigate complexities and drive positive outcomes in accessing innovative medicines in the UK.

MARKET ACCESS TRACK

EU HEALTHCARE REFORMS AND IMPLICATIONS FOR LOCAL MARKET PRICING

INTRODUCTION

Delving into the potential ramifications of the proposed revisions to EU pharmaceutical legislation, specifically focusing on the reduction of the data protection timeline, is crucial. Currently, Europe invests a staggering 39 billion EUR in Research and Development (R&D), showcasing a thriving R&D landscape. However, when compared to powerhouses like the US and China, Europe still lags behind.

Manufacturers are expressing concerns about the proposed legislation, viewing it as a possible threat to ongoing R&D efforts in Europe. The reduction of the data protection period from 8 years to 6 years implies that manufacturers will have a shorter timeframe to recoup their investments, which are vital for sustaining further R&D endeavours.

THE DISCUSSION

The looming question revolves around whether manufacturers will continue to invest in lengthy clinical trials akin to those in cardiology or in complex and expensive trials typical in rare disease research. There's speculation about a potential shift towards developing more intricate products, which are harder to replicate and often come with a higher price tag.

The EU aims to incentivise manufacturers to invest in combating anti-microbial resistance (AMR) by offering an additional year of data protection. This move could potentially stimulate R&D activities in this critical area. However, despite such incentives, access remains a significant hurdle. Introducing access incentives for anti-microbials, akin to orphan drug designation, could pave the way for tailored AMR access pathways within Europe.

Data protection duration guarantees manufacturers a specific timeframe to generate revenue essential for ongoing R&D endeavours. With reduced data protection, the logical response might be to increase drug prices. However, this approach faces challenges in Europe due to a decreased willingness among payers to pay higher prices compared to other regions. Manufacturers are unlikely to raise prices without corresponding reimbursement adjustments. This situation contrasts with the US, where increased data protection could potentially lead to price hikes.

Many pharmaceutical products are initially developed with the US market in mind, and while this mindset also extends somewhat to Europe, the reduced data protection timeline could alter this dynamic in the future.

THE KEY TAKEAWAYS

1. Manufacturers must familiarise themselves extensively with the revised EU pharmaceutical legislation to navigate potential challenges effectively.

2. The looming question remains: will manufacturers sustain their R&D investments in Europe amid these regulatory changes?

3. Furthermore, will the EU introduce additional incentives to bolster anti-microbial research and development?

By addressing these critical points and considering potential solutions, stakeholders can better prepare for the evolving pharmaceutical landscape in Europe.

We always welcome your thoughts and opinions on the topics raised at EPA.

If you'd like to share anything from your Amsterdam experience or hear how we can support you in getting your product to market, email our leadership team today at contact@remapconsulting.com or reach out personally by clicking their email below.

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