Remap

Day One
World EPA 2025

Our overview of:

the keynote and insights emerging from the global market access landscape

Highlights

1

Following the EU HTA launch, manufacturers remain concerned about uncertainties in the JCA process, especially for orphan drugs. Limited resources make meeting tight timelines and complex PICO requirements challenging. Insights from initial JCA submissions can inform planning and strategy for future launches.

2

There's a growing need for early stakeholder engagement to optimize planning and streamline scientific advice generation. Proactive engagement helps manufacturers anticipate PICOs and facilitates smoother assessments. For details, see Charlie Hewitt’s presentation summary on the website.

3

AI has significant potential to transform market access, though current tools aren't fully mature. Rapid progress is ongoing, so manufacturers should start preparing foundations now.

4

Trust, validation, and human expertise remain essential for successful AI adoption and integration.

The Key Takeaways

RARE DISEASES

- The JCA process presents significant uncertainties for orphan drugs and rare disease, as many orphan drug manufacturers often lack the resources to navigate the tight timelines and complex evidence requirements imposed by the JCA.

- Whilst specific markets do have flexibilities for orphan drugs, many markets still assess orphan drugs with standard HTA methodology, resulting in payer uncertainties that impact pricing and reimbursement outcomes.

- Healthcare pressures go beyond rare disease treatment, proactive engagement between payers, policymakers and manufacturers is needed to ensure healthcare sustainability.

The Key Takeaways

MARKET ACCESS

- As AI becomes essential to market access, manufacturers that effectively use these technologies will achieve a strategic advantage in analytics and decision-making, leading to improved efficiency.
- Market access: Early engagement in the EU HTA is essential to avoid bottlenecks. Manufacturers should focus on proactive strategic planning, participate in JSCs, and collaborate with local KOLs to respond to JCA deadlines in a timely manner.

HTA

- Early engagement with payers can have a significantly positive impact on market access, and early scientific advice can facilitate the optimisation of pricing and reimbursement through alignment with payer expectations.

Observations

RARE DISEASES

- Manufacturers are developing a growing focus on the utilisation of AI and machine learning to enhance the understanding of rare diseases and optimise factors like RWE.
- Funding for rare diseases in specific markets, whilst necessary, may impact available funding for other treatments, impacting pharmaceutical companies without relevant treatments in their pipeline.

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- Value stream mapping can enhance efficiency by identifying the most effective tools to streamline processes, reducing resource strain and improving efficiency in market access.
- Manufacturers can use early PICO simulations to validate evidence requirements and shape assessment parameters. Proactive participation mitigates the risk of misalignment with HTA expectations, thereby facilitating timely reimbursement.

For The Future

RARE DISEASES

- There is a risk the JCA reports will be used opportunistically as they are non-binding. They may only be used when they meet the expectations of local bodies. However, there may be a fairer way to take these into consideration; for example, some countries (e.g., Sweden) are binding themselves to the JCA outcomes.
- As AI continues to develop and gain more widespread use, different use cases, level of utilisation of AI within the rare disease space, and the limitations of said AI within these use cases can be evaluated.

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- The first wave of JCA outcomes is anticipated in 2026. The initial wave of JCA assessments will establish important precedents. submissions anticipated in 2026, early assessments will establish important precedents. It's crucial for manufacturers to closely monitor these outcomes to refine their strategies and navigate potential pitfalls effectively.

Manufacturer Considerations

RARE DISEASES

- Consider the development of active partnerships with payers and healthcare systems through mechanisms such as value-based agreements to ensure ongoing sustainability of healthcare and positive payer perception.
- Continue to monitor the use of AI within the rare diseases space by competitors and national stakeholders to facilitate beneficial future outcomes for orphan drugs.
- There is a high degree of uncertainty in rare diseases and orphan drugs. A flexible mindset is required, including a flexible value assessment framework, a risk-sharing agreement and a dynamic pricing scheme.

MARKET ACCESS

- It is essential for manufacturers to build partnerships, adopt new technologies, and anticipate evolving access challenges.

Manufacturer Considerations

MARKET ACCESS

- Investing in education, cross-functional collaboration, and digital tools, manufacturers can successfully navigate the evolving EU HTA landscape.

HTA

- Consider the use of scientific advice to ensure optimal HTA outcomes and the choice of scientific advice process based on factors specific to the manufacturer, such as the depth of evidence required, timelines, resource availability and eligibility.

Ready for day 2?
Meet our team at booth 36