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ISPOR
Improving healthcare decisions

ISPOR Europe 2022 roundup

The onsite Envision team shares its key takeaways from ISPOR Europe 2022

ISPOR Europe 2022 in Vienna, Austria connected in-person and virtual attendees at a unique event that celebrated the theme of "Collaborating Across Borders: Building & Using Evidence to Enable Access."

Drawing on this theme, we recognize that embracing collaboration, inclusivity, and innovation, together with the highest standards of evidence, are essential to our success in enabling patient access to new health technologies.

KEY TAKEAWAYS

Collaborations to enable access

Central to the theme of the congress were collaborations across countries and stakeholder groups – including health technology assessment (HTA) organizations, regulatory agencies, patients and patient advocacy, healthcare professionals, and researchers – to support patient access to innovative health technologies. The theme aligns with the introduction of the joint European Union (EU) regulation on HTA, which will be applied as a new framework for joint clinical assessments and consultations from January 2025. Key to the success of the new framework will be close collaboration between HTA, regulatory bodies, and other stakeholders over the coming years.

Another major collaborative initiative is the **Data Analysis and Real World Interrogation Network (DARWIN EU)** that is being established by the European Medicines Agency to coordinate the collection and analysis of real-world data pertaining to disease, populations, and the use, efficacy, and safety of medicines across the EU.

For more information on DARWIN EU, click here

Real-world evidence in decision-making

The value of RWE to support both HTA and regulatory decision-making is increasingly recognized, including evaluations of disease burden and unmet needs, real-world effectiveness and tolerability, and the patient quality of life impact of new health technologies. Early engagement of manufacturers with HTA and regulatory bodies is essential to ensure the appropriate design of real-world studies that incorporate suitable endpoints and address key clinical requirements.

The use of real-world data to provide “external” or “synthetic” control arms for single-arm trials is also emerging as an important focus area. Single-arm trials are increasingly used to support regulatory approvals, particularly in oncology and rare diseases. Real-world control arms may be used to provide comparative evidence for these trials. Efforts are being made to continually improve methods to adjust for selection biases, residual confounding, and missing or misclassified data in real-world datasets to enable robust comparisons. Manufacturers should also consider RWE endpoints and comparability when designing single-arm trials.

Enhancing the quality and transparency of real-world evidence

Uncertainty around the quality of RWE remains a barrier to optimizing its use in decision-making. A number of initiatives have been undertaken to ensure the robustness of RWE. Among these, the **National Institute for Health and Care Excellence (NICE) RWE framework** was published in June 2022 and defines the methods, tools, and checklists to improve the quality and transparency of the design, conduct, and reporting of RWE.

An emerging approach to enhance the robust design of real-world studies is target trial emulation (TTE), which applies design principles from randomized clinical trials, linking the design and analysis of the real-world study to a target trial. TTE is central to the methods recommended in NICE's RWE framework, but there is a need to further disseminate the methodology of TTE and upskill the industry.

Additionally, a joint International Society for Pharmacoepidemiology (ISPE) and ISPOR task force recently published **HARPER**, a harmonized protocol to facilitate consistency in RWE study design and reporting.

For more information on the NICE RWE framework, click here

For more information on HARPER, click here

Patient centricity in real-world evidence and access

Patients and the patient experience must be central to all aspects of health research and decision-making. Research efforts are being focused on gathering patient-experience data from initiatives such as DARWIN EU, and the development of patient-centered core impact sets (PC-CIS) that identify health outcomes and other disease impacts most important to patients. Caregiver perspectives and the impact of new health technologies on caregiver burden are also a key consideration.

Patient-preference studies are increasingly considered in healthcare decision-making, and an **ISPOR joint task force has developed a roadmap** for increasing the usefulness and impact of patient-preference studies in the decision-making process.

For more information on the task force recommendations, click here

Top 3 takeaways

1. **Seek input** from HTA organizations and regulators to guide clinical trial and RWE generation and reporting, and ensure relevance for decision-making in an evolving landscape

2. **Build confidence** in the quality of RWE by applying the appropriate frameworks and guidance for robust and transparent study design, conduct, and reporting

3. **Collaborate** with patient partners in the design, conduct, and reporting of RWE and patient-reported outcomes to ensure meaningful, patient-centric evidence generation

How can Envision Value & Access help?

Envision Value & Access unites market access strategy and scientific communications expertise. Collaboration and partnership are at the core of our philosophy, bringing together experts from across Envision Pharma Group, patient collaborators, and client partners to support access to innovative health technologies.

For more information on how we can support your value and access strategy and communications in an evolving world, contact us at: **value@envisionpharma.com**

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