

TODAY AT ISPOR

Our overview of the plenary session from Wednesday 20th November 2024

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Plenary 321b: Reality Check - Are We Bridging the Evidence Gaps for Patients?

SPEAKERS: Patrice Verpillat, Nikos Dedes, Peter Mol, Yannis Natsis, Almath Spooner, Anne Willemsen

INTRODUCTION

In the complex healthcare landscape, stakeholders across the pharmaceutical, regulatory, and health technology assessment (HTA) sectors continually face challenges in ensuring that innovative medicines reach patients effectively. The final plenary session at ISPOR 2024 sought to tackle this critical issue, discussing how evidence gaps impact access and exploring solutions to ensure patients benefit from advancements in medicine as early as possible.

The panel, comprising representatives from regulatory agencies, HTA bodies, industry, and patient advocacy, examined these gaps through diverse lenses. Their discussion underscored the need for collaboration, innovation, and patient-centred approaches in evidence generation.

WHAT ARE EVIDENCE GAPS, AND WHY DO THEY MATTER?

Evidence gaps arise when the data needed to evaluate a drug's benefit-risk balance or effectiveness for real-world application is incomplete or unavailable. These gaps can delay regulatory approvals, complicate HTA assessments, and hinder access to life-saving treatments.

For example, traditional randomised controlled trials (RCTs), often considered the gold standard in evidence generation, may not be feasible in certain diseases due to ethical constraints, low patient populations, or evolving treatment paradigms. Moreover, ageing populations with increasing comorbidities demand evidence that better reflects diverse patient groups, beyond the controlled environments of RCTs.

These gaps affect all stakeholders, including patients, payers, and manufacturers, and addressing them requires a concerted effort to build robust evidence frameworks.

PERSPECTIVES FROM THE PANEL

The patient perspective

Nikos Dedes, chair of the Greek Patients Association, highlighted the inequities in drug access across Europe and globally. Drawing from his experience as an HIV patient advocate, he stressed the importance of quality-of-life data and evidence on drug tolerability, especially for older populations with complex health needs. Dedes advocated for better integration of patient voices in designing and implementing data collection initiatives, such as real-world data registries.

The payer perspective

Yannis Natsis, representing the European Social Insurance Platform, underscored the challenges payers face in scrutinising treatments with limited evidence. While robust RCTs remain essential, he argued for stronger commitments from manufacturers to provide post-launch evidence through real-world data. Natsis also pointed out that better evidence could drive faster drug access and help negotiate fairer contracts with manufacturers.

The HTA perspective

Anne Willemsen, co-chair of the HTA CG JCA subgroup, focused on the need for early alignment between regulatory and HTA evidence requirements. With the Joint EU HTA on the horizon, she emphasised the importance of addressing divergent evidence needs early in a drug's development. Willemsen advocated for proactive strategies to ensure success at both regulatory and HTA levels.

The regulatory perspective

Peter Mol, a member of the CHMP, discussed the limitations of traditional endpoints, such as overall survival, for innovative therapies. He called for increased use of patient-relevant outcomes and innovative trial designs, such as registry-based randomised controlled trials (R-RCTs) used in Nordic countries, to strengthen data reliability and relevance.

The industry perspective

Almath Spooner, representing AbbVie and EFPIA, highlighted barriers to accessing high-quality real-world data, despite initiatives like DARWIN EU. She called for greater predictability in the acceptance of innovative trial designs and improved transparency in data-sharing processes. Spooner also stressed the need for convergence among methodologies and stronger collaboration across all stakeholders.

Bridging the gaps: what's next?

The panel's discussion revealed a shared recognition of the critical need to address evidence gaps, yet a unified path forward remains elusive. Key solutions discussed included:

- **Harnessing real-world data (RWD):** expanding the use of RWD to complement RCTs, particularly for diseases where traditional trials are not feasible
- **Aligning evidence needs:** encouraging earlier and more frequent dialogue between regulators, HTAs, and industry to harmonise evidence requirements
- **Strengthening patient involvement:** integrating patient perspectives more effectively into evidence-generation efforts to ensure data reflects real-world health challenges

While progress has been made, such as through initiatives like registry-based RCTs, the path to bridging these gaps will require sustained collaboration, trust, and innovation.

THE 3 KEY TAKEAWAYS

- 1 Stakeholders must invest in initiatives like R-RCTs and DARWIN EU to complement traditional trial data and address unmet needs
- 2 Regulators, HTAs, and industry should collaborate from the outset to harmonise evidence requirements and improve decision-making
- 3 Greater integration of patient voices ensures evidence generation aligns with real-world challenges and improves access to innovative therapies



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