TOPRA Innovation Summit

19 April 2023 - NH Schipol Airport Amsterdam, The Netherlands

Programme

In these sessions, our expert panellists will brief us on current key topics and provide perspectives on what the future may hold over the next 3-5 years.

Panel session 1: Patient access to innovation: challenges and opportunities in a changing world

What is the value of innovation in the healthcare sector unless it changes patients' lives for the better? Patient access, therefore, should be the ultimate goal for innovators and yet this topic often remains largely ignored until developers start preparing for product commercialisation. As a result, many innovations only reach a small proportion of the patients who could benefit and this needs to change.

While discussions often focus on innovation, the key concept that drives patient access is value, specifically, value that is relevant to patients and demonstrated through evidence. Value is realised when an innovation reaches patients and provides benefits in terms of outcomes. Therefore, innovators should not only take care to ensure that regulatory requirements are met, but also that treatments are available, accessible, and affordable for eligible patients so that there is equitable delivery of health outcomes to all populations that may benefit.

This panel will explore some of the major barriers, and future opportunities when it comes to improving patient access to innovation with a specific focus on medicines. The discussion will focus on what may help us to improve future access to innovation.

Confirmed panellists:

- Dr Michael Berntgen, Head of Scientific Evidence Generation, European Medicines Agency
- Chiara Brouns, Policy Adviser/Strategic Buyer for Expensive Medicines, Association of Dutch Health Insurers
- Chris Hoyle, Head of Market Access, Pricing & HEOR, Europe-International, Immunocore
- Wija Oortwijn, President, Health Technology Assessment International (HTAi)
- Nathan Sigworth, Co-Founder and Chief Executive Officer, CCX
- Moderator: Dr Mel Walker, Biotech/Digital Health Advisor

Additional panellists will be confirmed shortly.

Panel session 2: Unlocking the potential value of RWE in regulatory decision-making – what does it take?

The consideration of real-world evidence (RWE) in regulatory decision-making is nothing new, and for decades such evidence has been used in the post-authorisation phase, predominantly to monitor post-marketing safety. What has changed dramatically, however, is the tremendous increase in both the supply and demand of RWE and RWD in support of regulatory decision-making.

Supply-wise, there has been a dramatic increase in the speed and connectivity of data acquisition, the volumes and the types of health data, all enabled by modern digital technologies. On the demand side, we are facing a rapidly changing evidence-generation landscape with increased relevance for RWE in support of regulatory decision-making, particularly in the pre-authorisation phase. With the increasing volume and complexity of 'big data' being captured across multiple settings and devices, it offers significant additional utility for drug development throughout the entire product lifecycle. However, the acceptance of this data in the regulatory context is still an evolving area.

This panel will explore current use of RWE in regulatory decision-making, some of the major barriers, and future opportunities/role of real-world data (RWD) to generate evidence complementary to traditional clinical research. Discussion will focus on what may help us to unlock the potential of RWD/E in regulatory decision-making.

Confirmed panellists:

- Ali Holland, Head of Decentralised Clinical Trials, Medable, Inc
- Almath Spooner, DARWIN EU board member
- David Wormser, Executive Director for Early Evidence Generation, Novartis
- Patrice Verpillat, Head of Real-World Evidence, EMA
- Moderator: Dr Chantal van Gils, Director Epidemiology & RWE, NDA advisory services

Additional panellists will be confirmed shortly.

Panel session 3: The future of Artificial Intelligence in Healthcare

Artificial intelligence (AI) has entered into our daily lives, ranging from general-purpose AI to AI with clear and intended purposes. And even while the healthcare industry always approaches changes and innovation with caution, this industry is not an exception.

There is no denying that AI has the potential to change healthcare, especially with the growing amount of healthcare data being collected. AI may lower the cost and effort associated with medical research, bring about solutions that could enhance patient outcomes, and broaden access to innovative solutions. However, there are several issues with its use in healthcare, including issues with performance, security and safety, regulatory and compliance, and ethics.

This panel session will explore some lessons learned as well as discuss opportunities and controversies in healthcare, including pharmaceutical development and medical devices. The discussion will focus on what may help us to improve the future of healthcare using AI.

Confirmed panellists:

- Francesca Cerreta, Scientific Administrator - Scientific Evidence Generation Department, European Medicines Agency
- Leon Doorn, Head of Regulatory Compliance, Aidence
- Moderator: Dr Célia Cruz, Head of Regulatory Affairs, Complear

More panellists will be confirmed shortly.

There will then be a final gathering of delegates after the panel sessions have finished where participants will gather into groups to discuss a range of topics with their peers in the regulatory affairs industry.