13th Annual

IMPACCT Real World Evidence Summit

Exclusive Speaker Interview

Kelly H. Zou, PhD, PStat, FASA
Chief Excutive Officer, AI4Purpose
President, ASA NYC Metro Chapter

Looking back over the last decade, what do you see as the most significant shift in how real-world evidence is generated, interpreted, or applied across the product lifecycle?

The 21st Century Cures Act in the United States (US) was introduced exactly a decade ago in 2015 and become public Law on December 13, 2016, in which real-world evidence (RWE) was defined. Subsequently, the US FDA defines both real-world data (RWD) and RWE. Thus, it is the right time to reflect upon these ten years on the progression of RWE. RWE has undergone a transformative shift in its generation, interpretation, and application across the product lifecycle, driven by technological advancements, regulatory evolution, and growing demand for patient-centric data.

For example, in the context of Health Economics and Outcomes Research (HEOR), RWE reliant on retrospective claims and registry data. These days, since RWD is defined as "data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources," the types of data sources have become increasingly more diverse, high-volume sources such as electronic health records (EHRs), wearable devices, mobile apps, and social media. This expansion enables near real-time data capture and longitudinal tracking of patient outcomes.

The interpretation of RWE has become more rigorous and standardized. Methodological frameworks like propensity score matching and synthetic control arms help mitigate bias and confounding, improving the credibility of non-randomized data. Regulatory bodies, including the Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have issued guidance to ensure transparency and reproducibility in RWE studies, fostering greater trust in their findings.

The data lifecycle coincides with the biopharmaceutical product lifecycle from early development to post-market surveillance. In early phases, it informs trial design and feasibility. During regulatory review, it supplements clinical trial data, especially in rare diseases or underrepresented populations. Post-approval, RWE supports repurposing, label expansions, safety monitoring, and value-based reimbursement decisions. Payers and providers increasingly rely on RWE to assess effectiveness and safety to guide formulary inclusion. Finally, advanced data science and analytic capabilities, including artificial intelligence (AI), machine learning (ML), deep learning (DL), natural language processing (NLP), and large language models (LLMs) to help extract meaningful insights from complex datasets in a timely and comprehensive fashion.

Over time, RWE has evolved from being a valuable tool to being a strategic imperative to shape and reshape how stakeholders evaluate and optimize both novel and existing products.

Despite progress in data availability and analytical tools, what challenges in RWE generation or application still persist and why do you think they remain difficult to solve?

RWE is critical for decision-making, but there are several persistent challenges that still hinder its optimal evidence generation to gain actionable insights.

The first challenge is data quality. RWE relies heavily on RWD from EMRs, claims, registries, and wearables. These sources often lack standardization, contain missing or inconsistent entries, and vary in granularity. Unlike RCTs, RWD isn't collected with research in mind, making it difficult to ensure validity and reproducibility. Recently, the EMA has developed the data quality frameworks (DQFs), as well as several frameworks, including the ATRAcTR (Authentic Transparent Relevant Accurate Track-Record) screening tool that my coauthor and I have constructed to assess the potential for RWD and RWE for regulatory purposes.

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The next bottleneck is bias and confounding. RWD is inherently prone to selection bias, confounding variables, and reverse causality. While statistical methods like propensity score matching help, they can't fully replicate the rigor of randomized controlled trials. This limits the causal inferences that can be drawn from RWE.

The last obstacle is system interoperability and data privacy. Accessing comprehensive data from a variety of sources for patient journey mapping. is technologically complicated, compounded by data privacy regulations, including the General Data Protection Regulation (GDPR) in the European Union (EU) and the Health Insurance Portability and Accountability Act (HIPAA) in the US, fragmented health systems, data capture silos, and technologies for linkage. Integrating RWD across different platforms while maintaining patient confidentiality remains a hurdle.

Can you share an example where real-world data meaningfully influenced a decision whether clinical, commercial, or regulatory in a project you've worked on?

As a use case example when I was at Pfizer, I presented on behalf of the RWE collaborative team who used RWD to generate RWE to provide reference information to the Pharmaceuticals and Medical Devices Agency (PMDA), the regulatory agency in Japan, for lorazepam IV in status epilepticus among infants aged 3+ months in Japan (https://ww2.amstat.org/meetings/jsm/2019/onlineprogram/AbstractDetails.cfm?abstractid=304651).

A small clinical trial was conducted with lorazepam IV in Japan for the indication of status epilepticus and the new drug application was approved in 2018. Unfortunately, the primary endpoint did not meet the pre-specified target efficacy criteria while the key secondary endpoint supported efficacy. The target number of nursing infants (3 months to < 1 year) was 3+ subjects, and only 1 patient was enrolled. The PMDA inquired the usage of lorazepam IV and diazepam IV outside of Japan to treat pediatric patients aged 3+ months using RWD.

The RWE collaborative team demonstrated usage of lorazepam IV for pediatric patients compared to diazepam IV among nursing infants outside of Japan. This was a successful example of the use of RWE to support regulatory decision making, and various assets may leverage this experience in future regulatory interactions.

Besides the above example in Japan, my recent co-edited and co-authored book (https://www.taylorfrancis.com/books/edit/10.1201/9781003017523/real-world-evidence-patient-centric-digital-era-kelly-zou-lobna-salem-amrit-ray), published by Taylor & Francis, reviewed key analytical practices and data science methods for generating RWE, with an emphasis on pharma. It covers bioethics, regulations, and compliance, highlights emerging trends, and offers best practice guidelines. There were several examples and software recommendations to support evidence-based decision-making and healthcare innovation around the world.

What developments in the RWE space are you most excited about, whether it's an emerging methodology, data source, or cross-functional mindset shift?

Complex data sources and holistic evidence generation continuously evolve, partly driven by technological advances and shifting regulatory expectations. Three key trends that I am excited about are as follows, AI-powered analytics, integrated evidence planning (IEP), and the use of unstructured data, which are reshaping how biopharmaceutical companies develop and evaluate therapies.

AI is revolutionizing RWE by enabling deeper, faster insights from complex datasets. AI/ML algorithms can identify patterns in patient outcomes, predict disease trajectories, and optimize designs and outcomes. AI also enhances pharmacovigilance by detecting safety signals.

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IEP has become a cornerstone of modern drug development. Companies are increasingly combining data from RCTs, patient-reported outcomes (PROs), EHRs, and claims databases to build a comprehensive evidence base. This approach supports regulatory submissions, informs payer negotiations, and accelerates market access. RWE can supplement traditional clinical trial data.

Unstructured data unlock new and rich dimensions of patient insights and journeys. LLM and NLP tools extract valuable information from clinical notes, radiology reports, and PROs. Moreover, data from wearables, mobile apps, and social media provide real-time perspectives on treatment adherence and quality of life. By integrating structured and unstructured data, companies gain a richer understanding of therapeutic impact across diverse populations.

Alongside regulatory and payer advancements in big data and AI, these three trends are transforming RWE from a retrospective tool into a proactive driver of innovation, precision medicine, and regulatory success.

What are you most looking forward to at the 13th IMPACCT Real-World Evidence Summit?

This will be the 13th IMPACCT Real-World Evidence Summit, and as the Co-Chair once again, I welcome many returning and new speakers, panelists, discussants, and attendees. Held in Boston annually, this meeting is like a step into the epicenter of innovation in end-to-end lifecycles of innovative products and solutions.

As someone deeply invested in advancing how we use data to improve patient outcomes, it will be a convergence of experts from leading biopharmaceutical and health tech companies that share cutting-edge strategies and use-case examples.

What excites me is the focus on integrating AI/ML, predictive modeling, and unstructured data into the product lifecycles and workflows. Studies and tools will be shared on how the biopharmaceutical and healthcare industries can collaborate and partner to generate insights, evaluate outcomes, and maximize values. These learning sessions can be both hands-on and forward-looking to help participants assess and refine their end-to-end evidence generation strategies.

Furthermore, professional networking via speed networking is a wonderful feature. Connecting the dots to tackle challenges, such as data quality, payer alignment, or cross-functional collaboration, just to reemphasize a few, can spark new ideas for patient care. Last year, there was also an innovative hackathon to design RWE and digital health solutions for specific healthcare challenges globally.

Overall, this meeting can be essential for those of us who harness RWD and generate RWE to drive timely and impactful decisions through patient-centricity.

Disclaimer: The views expressed in this interview are the author's own and may not necessarily reflective of her employer's views.

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