

Current Status, Challenges, and Future Perspectives of Real-World Data and Real-World Evidence in Japan

Real-world data and real-world evidence are terms widely used in the field of health care. In fact, various stakeholders are showing increasing interest in using real world data and real world evidence. Pharmaceutical companies, for example, use real world data for various purposes—from the early stages of development to post-launch. One hot topic focuses on using real world data and real world evidence to support regulatory decision making to deliver drugs faster to patients with high medical needs. This has been the subject of active discussion in the US, Europe, Japan and other countries, leading to regulatory reform and improvement of the implementation environment.

However, there are barriers to the regulatory acceptance and use of both real world data and real world evidence. Real world data should be evaluated not only for quality but also for data relevance. There is a lack of universally accepted methodological criteria.

We propose that the use of real world data and real world evidence in the development of pharmaceuticals and medical devices be discussed by industry, government, academia and patients and that clear international guidance be issued from regulatory authorities. As the demand for real world data and real world evidence increases, we need to focus on the quality of data, data relevance, study design, the quality of analysis, and the transparency of the entire process and reporting to ensure credibility and acceptance by decision makers, while protecting patient privacy.

While the expected benefits of using real world data and real world evidence are high, challenges in Japan persist, such as limited access to real world data and difficulty linking useful databases. Indeed, many countries other than Japan face these same challenges.

In the absence of global definitions of real world data and real world evidence, the following have been proposed by the Pharmaceutical Research and Manufacturers of America Japan Medical Affairs Committee.

As defined by the FDA, “Real-world data are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.” Since the FDA's definition is the broadest one and covers the purposes for which we use real world data, we propose the same definition. We propose that real world evidence be defined as the evidence

derived from the analysis of real world data. This definition is simpler and broader in scope than the FDA's definition. That's because the FDA defines real-world evidence as the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of real world data, whereas our definition applies to Health Technology Assessment and disease burden as well.

In Japan, opt-in is required to use data in research under the Act on the Protection of Personal Information, but this does not apply to the use of data for research purposes in academia. In addition, such consent acquisition is often not performed in routine medical care. As a result, the private sector only has access to commercially available anonymized data. This may result in a loss of research opportunities for the private sectors that could benefit patients and society.

Another challenge is the difficulty in linking different databases. Linking data from several databases pertaining to an individual provides valuable insight. But laws and regulations concerning patient anonymity and consent, and the fact that there are multiple database custodians, limit data linkage.

In 2018, the Next-generation Healthcare Infrastructure Act was implemented to improve data linkage and governance. As a result, the Japanese regulatory authority has allowed business operators certified by the government to create anonymized data from medical information. This move is promising because it could increase the amount of data available, while protecting personal information.

However, in terms of data use, stakeholders would lose immediacy and valuable information. And in terms of resources, new issues that arise would require tremendous amounts of people, money, and time.

The aim should be to create a Japan where necessary medical information can be effectively used anytime, anywhere without omission after ensuring data security and the prompt return of data and evidence to patients and the public.

To realize this vision, we propose that the Japanese government implement measures such as educating the public on personal information and research ethics, as well as on the use of research, which could increase understanding and cooperation. We further propose that after education by the government, consent from the public to use personal medical information be obtained for research and medical care, and that these data sets be linked using the Japanese national identification number, My Number.



This solution would lead to high-quality real-world datasets that are richer and less biased and would significantly reduce the resources required to create anonymized data. Of course, the utilization of advanced IT and innovative technologies and the assurance of data security are essential for realizing these goals.

We propose the realization of a society in which innovative technology provides a comprehensive system for instant access to real world data while maintaining data security, and in which all Japanese people can fully benefit from the use of digital technology and real world data, thereby improving their quality of life.