Pharmaceuticals and Medical Devices Agency (PMDA)
独立行政法人　医薬品医療機器総合機構

Utilization of Real World Data

- PMDA's approaches -

23rd March, 2021

Health-related data are gathered and accumulated in the clinical practice day by day. These data are called Real World Data (RWD), and they include electronic health record, claims data, patient registry data, etc. RWD still provide valuable information related to the outcomes of using medical products, while RWD are not obtained in the same manner as well-designed clinical trials conducted to evaluate medical products.

At PMDA, we have already had some experiences of utilizing such existing data for evaluating benefit-risk balance in the regulatory process. For example, in the case of tacrolimus, RWD was utilized in its approval for an indication supplement of initial treatment for interstitial pneumonia associated with polymyositis/dermatomyositis. The indication was approved in 2013. Not only above case, but RWD has been utilized in some of new drug applications so far.

Although the PMDA has been making good use of RWD, it applied a case-by-case basis approach until recently. It might not be widely known RWD can be utilized for regulatory submission. In order to promote RWD utilization further by product developers, the PMDA has recently developed and finalized two guidelines below:

- “Basic principle for utilization of registry data for regulatory submission” in regards to patient population, endpoints, statistical analysis methodology, etc.
- “Points to consider for ensuring the data reliability” with regards to quality management and quality assurance of data collection, storage of records, registry operation system, and ethics/privacy, etc.

They were published on 23rd March, 2021, and the English versions will be available shortly. In addition, PMDA started operations of a couple of consultation categories for registry owners, database providers and developers of medical products in 2019, in order to provide scientific advice on feasibility of registry utilization and on registry data reliability. And within the PMDA, we will newly establish RWD Working Group on 1st April, 2021. It is a cross-cutting Working Group involving multiple offices within PMDA, and it will discuss topics to be considered in relation to RWD utilization throughout the product life cycle in a comprehensive manner.

Through these multi-dimensional approaches, the PMDA is building an environment that facilitates the use of RWD in the regulatory submission of medical products. By understanding the characteristics, advantages and limitations of RWD, we will plan to bolster RWD utilization in every stage of the medical product life cycle from the pre-approval development to the post-marketing phase, towards enhancement for early patient access.

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