Final Business Plan

Establishment of a new ICH guideline on "General principles on plan, design, and analysis of pharmacoepidemiological studies that utilize real-world data for safety assessment of medicines"

23 March 2022

Endorsed by the Management Committee on 5 April 2022

1. The issue and its costs

While the number of pharmacoepidemiological studies utilizing Real-World Data (RWD) in a regulatory context have increased globally, currently, there are no ICH guidelines that focus on how to generate fit-for-purpose Real-World Evidence (RWE). Although many regions (Canada, China, EU, Japan, and US) have published guidelines related to general principles of planning and designing such studies, mainly for the purpose of medicine safety assessment, a lack of harmonisation in this area can cause challenges for sponsors and regulators.

2. Planning

Establishment of a new harmonized guideline entitled "General principles on plan, design and analysis of pharmacoepidemiological studies that utilize real-world data for safety assessment of a medicine."

For this guideline, medicines refers to drugs, vaccines and other biologics. This guideline will focus on non-interventional studies using RWD. Studies with treatment assignment are excluded, including randomized clinical trials or single arm studies. However, the basic principles presented in this guideline may be applicable to these studies when RWD elements are included.

The Expert Working Group (EWG) will include regulators and industry representatives with innovative thinking, adequate expertise and experience in technical and regulatory issues relating to pharmacoepidemiology utilizing RWD for safety assessments of drugs, vaccines and other biologics. Regulatory, industry, and observer experts with experience and expertise relating to pharmacoepidemiology utilizing RWD for studying the safety of these products are needed for the development of these guidelines. The core disciplines include: pharmacovigilance, epidemiology, biostatistics, data curation and management, and ethics.

The anticipated time to complete the establishment of the guideline will be 2-3 years (by January 2025).

3. The impacts of the project

A lack of harmonisation in this area can cause challenges for sponsors and regulators and the fundamental issues and overarching principles of following topics will be addressed in this guidance.

- Definitions and associated requirements
- Format and content of regulatory filings and reporting of study materials and results (e.g. protocol and report templates or formats)
- Data source generation and/or selection, and fit for purpose requirements
- Outcome identification by study design and data type
- Selection of proper methods
- Safety reporting requirements, in line with regional regulatory frameworks

The proposed guideline would result in harmonisation in these areas, minimize issues of the conduct of multiple studies on the same safety concern for submission to multiple regulators, result in improved efficiency and transparency in the development, submission and review of pharmacoepidemiological studies and resultant regulatory actions.

The proposed guideline will outline recommendations on general considerations when utilizing RWD for drug, vaccine and other biologic product safety assessments, including defining the research question, data source selection/generation, study design, target populations, exposure and outcome(s), covariates, data source fit-for-purpose evaluation, sources of and methods to address confounding and bias, analytic approaches, and reporting.

4. Post-hoc evaluation

A post hoc evaluation plan will be proposed after development of the guideline