FDA Guidance: Using Electronic Health Records and Medical Claims Data to support regulatory decision-making for drug and biological products

Purpose of the guidance

FDA has released guidance as part of the FDA's Real World Evidence Program for industry in using Electronic Health Records (EHR) or Medical Claims Data (MCD) in clinical studies to support regulatory decision making on the effectiveness or safety of Drug and Biological products

Considerations and recommendations

- Before conducting a study using EHRs or MCD:
  - Sponsors should submit protocols and statistical analysis plans to the FDA
  - Predefine essential elements of study design, analysis, conduct, and reporting

Data sources

- Thoroughly consider and document the relevance and reliability of data sources.
- Data sources should address the study question by clearly identifying the study population, the factors being tested (exposures), the outcomes, and key variables that might distort the relationship between exposure and outcome (confounders) or change the strength or direction of the relationship (effect modifiers).
- Demonstrate that data sources are detailed and complete enough to capture the study population. Consider continuity of coverage and care, data linkage and synthesis, handling of unstructured data, and dealing with missing data.

Data quality

- Maintain high data quality throughout the course of the data lifecycle: collection, curation, and transformation into the final study-specific dataset.
- Generate comprehensive documentation for the quality assurance and control plan, and the data management process.

Study design elements that should be clearly defined:

- Time periods relevant to the study, including periods for identifying the study population, assessing exposures and outcomes, and follow-up.
- Methods for applying inclusion and exclusion criteria and defining how to identify the study population from the data source.
- Define exposure, including the data source, duration, dose, any considerations for specific populations, and validation methods used to ensure the data's accuracy and reliability for measuring exposure.
- Define the outcome of interest, create operational definitions, and validate them while addressing any potential issues with outcome misclassification.

Validation

- Validation involves ensuring the study variables (e.g. exposures and outcomes) are correctly measured, usually according to a reference standard.
- Misclassification and its impact on study validity should be considered, using appropriate validation approaches described in the protocol such as quantitative bias analyses.

Source: FDAs Real-World Data: Assessing Electronic Health Records and Medical Claims Data to Support Regulatory Decision-Making for Drug and Biological Products – Guidance for Industry