Use of Real-world Evidence for Drug Assessment in China

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Protect and Promote Public Health

RWE guidances published by NMPA

- **Guidance on Using Real-world Evidence to Support Drug Development and Decision-making (For Trial Implementation), Jan 7, 2020**
- **Guidance on Real-world Data Used to Generate Real-world Evidence (For Trial Implementation), Apr 15, 2021**
- **Guideline on Real-world Study to Support Pediatric Drug Development And Review (For Trial Implementation), Aug 31, 2020**

Case Study 1

Fluticasone Propionate Inhalation Aerosol

- Sponsor have applied for expanding the scope of applicable population in China from the approved children ≥4 years old to the children ≥1 years old by using the clinical research evidence of overseas which are from the children ≥1 years old.
- As a post approval requirement, the sponsor completed a post marketing observational study for the purpose of monitoring the drug safety of children aged 1-4.

Case Study 2

- A cardiovascular drug that has been marketed overseas and is in urgent clinical need in China. It was approved for marketing with local clinical trial waiver.
- In order to further clarify the effectiveness in Chinese patients, it is planned to carry out prospective observational real-world study after marketing.
- To evaluate the preventive effects of the drug combined with standard treatment vs. standard treatment alone on major cardiovascular events in Chinese patients with cardiovascular disease.

Points of consideration using RWE for regulatory decision-making

- **Pre-communication of study protocol**
  - Real-world data
    - Relevance: The inclusion of important variables and information related to clinical outcomes, sufficient sample size and follow-up duration , etc.
    - Reliability: data completeness, accuracy, transparency and quality assurance
  - Rationality and rigor of study design and statistical methods
- **Transparency in implementation: avoid selection bias**
- **Whether study results are reproducible, robust and replicable**
- **Risk-benefit evaluation**

Challenges

- **Fit-for-purpose real-world data**
  - The purpose of data collection and curation was not to support the research
  - Accuracy and reliability
  - Patient privacy protection
  - Data submission meets regulatory reproducibility requirements
- **Lack of Common data model**
- **Methodology and analytical technology**