Using Real-World Evidence for Label Expansion

Presented by the MAPS Evidence Generation FAWG

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- This presentation is intended for general informational purposes only. It is not intended to provide legal advice and should not be relied on for that purpose. You should consult an attorney for advice regarding your individual situation.

Educational Objectives

This session will provide a learning opportunity for our audience by:

- Defining RWE/RWD and how are they different
- Understanding RWE product expansion legal and regulatory considerations
- Discussing RWE with FDA and Payors

Overview

- Introduction to RWD/RWE
- How did we get here?
- Examples of Successful Uses of RWE for Label Expansion
- Interacting with FDA regarding RWE
- Communications with Payors
- Questions

Introduction to RWD/RWE

Real-World Data (RWD):

- Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources (examples: electronic health records, medical claims, product and disease registries, patient-reported data)

Real-World Evidence (RWE):

- Clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

How did we get here?

Sentinel program (2008)

- Post-market surveillance

21st Century Cures Act (2016)

- Congressional mandate to evaluate RWE for new indications and satisfying post-approval study requirements

FDA Initiatives

- Final Guidance on RWE and Devices (2017)
- RWE Framework (2018)
- 4 draft guidance documents (2021)
- Advancing RWE (early meeting) program (2022)

Examples of Successful Uses of RWE for Label Expansion

Cases Label Expansion

- Edwards Life Sciences Sapien 3 TAVR
  - Registry data supports new indication for device.

- Ibrance® (Pfizer) for treatment of male breast cancer
  - FDA extrapolates from clinical trials safety and efficacy data from women, and RWE from use with men.

- Prograf® (Astellas) – suppression of organ rejection in lung transplants
  - Registry data showed significant improvement in cases where Prograf had been used (in combination with other drugs) compared with natural history studies of cases where it had not been used.

Causes of Rejected RWE Submissions

- Lack of transparency
- Post-Hoc analysis
- Statistical errors
- Non-generalizable data
- Missing data
- Inadequate sample size
- Failure to discuss early with FDA

Interacting with FDA regarding RWE

- Approach FDA early!!
- Listen to FDA feedback

RWE-Real World Evidence

Communications to Payors

FDAMA 114 (1997) amended by Section 3037 of 21st Century Cures Act

- Permits discussion of drug health care economic information (HCEI) regarding drugs to payors and formulary committees, subject to the following:
- Relating to an approved indication
- Competent and reliable scientific evidence
- Prominent statement describing material differences between HCEI and approved labeling

The above does not protect communications relating only to non-approved indications.

Thank You