How Companies Are Using Real-World Evidence for Regulatory Purposes

Presented by the MAPS Evidence Generation FAWG

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- The following faculty and planning members do disclose financial relationships- *(Cerise James MD, MPH; Neil Belson M.S., J.D.)*

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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 the different ways companies are using RWE for regulatory purposes
- Learning about “external control arms” and natural history studies
- Discussing challenges in using RWE

Significance of Real-World Evidence

- 78% of new drug approvals in 2020 included RWE in submissions*
- 10 therapeutics areas (oncology and infectious diseases had the most such approvals)
- #1 Trend in Health Economics in 2022-2023 (ISPOR)**

*Source The Role of Real World Evidence in FDA Approvals - 2021 Update - Aetion;

** Source: ISPOR - Top 10 HEOR Trends

Overview

- Introduction to RWD/RWE
- Recap from Webinar 1
- Discussion of different regulatory uses of RWE
- 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 or treatment derived from analysis of RWD

21st Century Cures Act (2016) – Mandated consideration of RWE in regulatory approvals

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

Regulatory Uses of RWE

1) Post-Approval Safety Monitoring

2) Clinical Trial Design

3) Obtaining FDA Approvals (focus on Regulatory)
   - External Control Arms and Natural History Studies
   - Label Expansion
   - Post-Market Studies

Post-Approval Safety Monitoring

- One of RWE's oldest uses
- FDA Sentinel System launched 2008
- Over 50 collaborating health systems, hospitals, universities and analytics firms

Clinical Trial Design

- Hypothesis generation
- Identify sub-populations of interest, treatment patterns
- Enhance clinical trial diversity
- Identify optimal sites
- Identify meaningful endpoints
- Learn about disease and its progression
- Identify appropriate inclusion/exclusion criteria

RWE-Real World Evidence

Case Study: Brineura* (cerliponase alfa)

- Approved 2017 for a rare form of Batten disease (an inherited disorder)
- Single "treatment" arm with 22 symptomatic patients
- Control used natural history cohort of 42 untreated patients
- Patients receiving Brineura suffered fewer declines than natural history control
- A post-approval safety study (≤ 10 years) required

* Source: https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm555613.htm

Some challenges with RWE

- It's new
- Often uses data collected for other purposes (and may be in unstructured form)
- Standards still being developed (and likely evolving on a case-by-case basis)
- Data privacy issues (watch my next presentation! ☺)

Thank You

- Question 7: (After two slides, "Label Expansion") What about some of the other regulatory uses for RWE that you listed on your slide?

- Question 8 (After two slides, "Challenges with RWE"): What do you see as some challenges with using RWE?

- Question 9: (Next slide) "Any concluding thoughts?"