What Does "Real-World" Evidence Mean? A Review of 2017 Literature

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BACKGROUND

- The use of the terms "real-world data" (RWD) or "real-world evidence" (RWE) have become increasingly common in recent years.
  - RWD is all data collected outside traditional clinical trial settings.
  - RWE is derived from RWD and allows for insight into the actual setting of use.
- In 1984, one article used these terms; through 2005, fewer than 20 articles per year used the terms, and in 2017, more than 1,000 publications used them. Despite the increase in publications, it is unclear which types of studies are being presented as RWE.

METHODS

- A review of English-language 2017 titles and abstracts in PubMed and Embase was performed. The search was broad and limited to the term "real world." Titles and abstracts were reviewed, with the assumption that high-level study design should be adequately captured within the abstract of a peer reviewed publication.
- The following were extracted based on information in title/abstract: therapeutic area, exposure type, study design, primary outcome, timing of outcome, country, and data source.
- Descriptive analyses were performed.

OBJECTIVE

- To evaluate the use of RWE in publications from 2017.

RESULTS

- There were 1,045 hits for "real-world" publications in 2017. Of these, 315 were excluded because they lacked an abstract (n = 93) or were not related to provision of health care (n = 222); 730 remained in the analysis.
- Overall, most studies were retrospective (67%) versus prospective (31%); 67% evaluated outcomes of a drug; 15% evaluated devices.
- Almost half of the studies (44%) used existing data sources for RWD, and nearly one third used primary data collection (28%) (Figure 1b).
  - Existing data sources included medical records (32%) and claims data (12%) (Figure 1b).
- More than half of the studies (n = 415; 57%) focused on three therapeutic areas: cardiovascular (n = 168; 23%), oncology (n = 141; 19%), and infectious diseases (n = 106; 14%) (Figure 2).
- Almost all infectious disease studies (92%) and most oncology studies (76%) evaluated outcomes of drugs. However, cardiovascular studies evaluated both drugs and devices almost equally (43% and 40%, respectively).
- There was a wide distribution of oncology studies led by breast cancer (18%) and non-small cell lung cancer (14%). Example articles from real-world oncology studies are shown in Figure 2.
- Infectious disease studies were dominated by hepatitis C (70%), followed by hepatitis B (12%). Example articles from real-world infectious disease studies are shown in Figure 2.
- Arrhythmia (26%) and coronary artery disease (24%) comprised the bulk of studies in cardiovascular disease. Example articles of real-world cardiovascular disease studies are shown in Figure 2.
- The primary outcome among most infectious disease studies was effectiveness (70%). Cardiovascular and oncology disease studies primarily evaluated effectiveness (48% and 42%, respectively) and treatment patterns (14% and 18%, respectively) (Figure 3).

Figure 1. Location and Data Sources for Studies

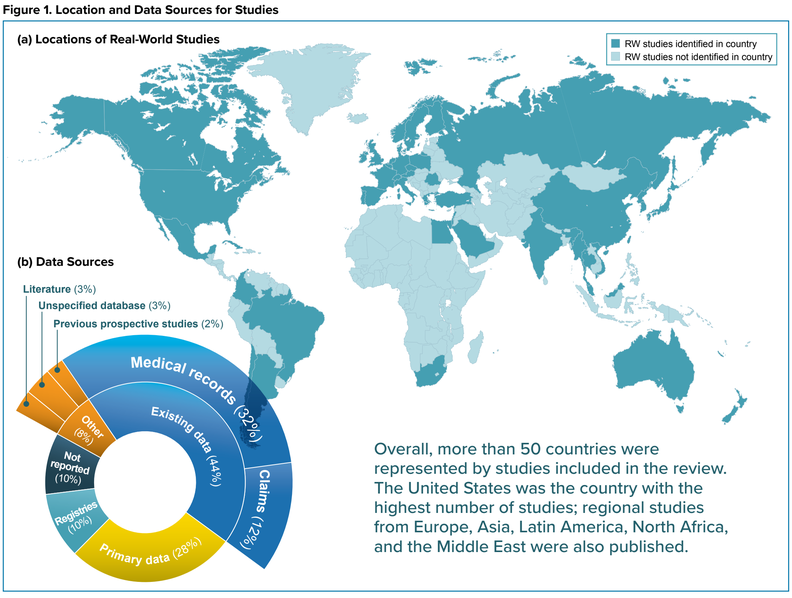

Figure 2. Types of Real-World Studies

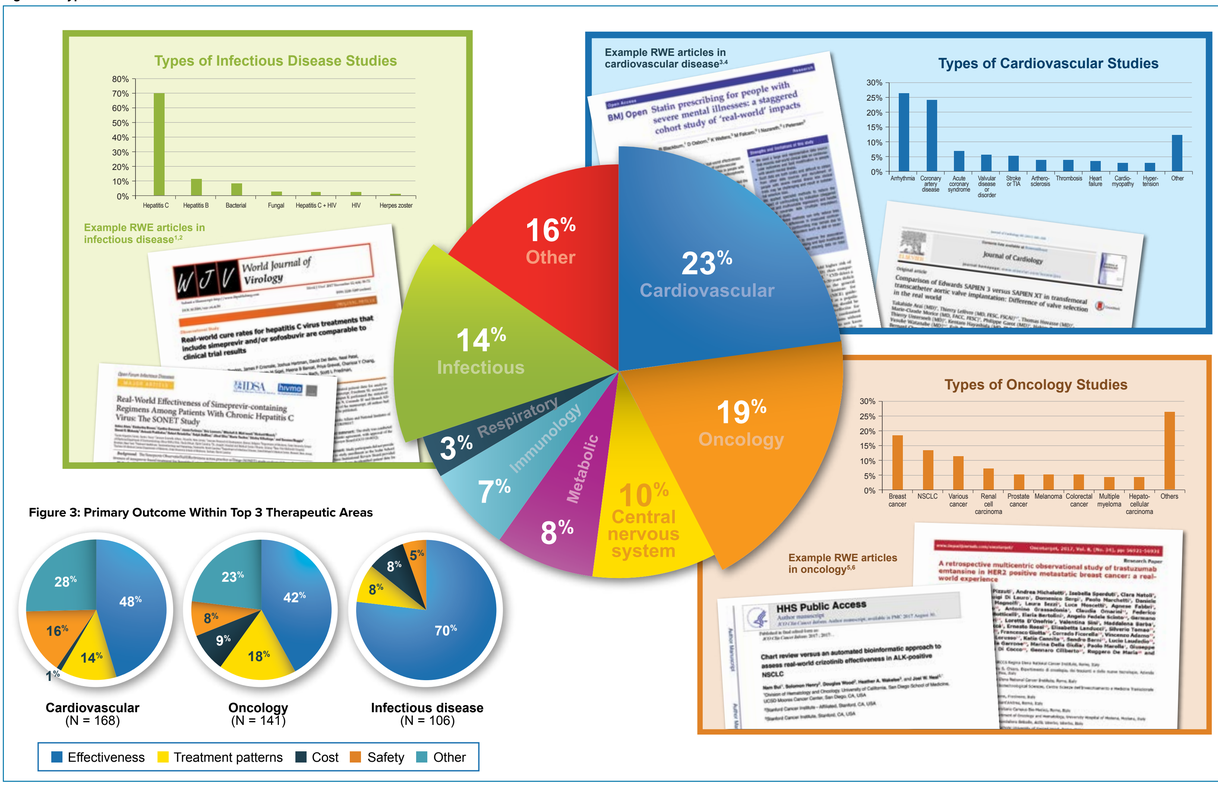

Figure 3: Primary Outcome Within Top 3 Therapeutic Areas

DISCUSSION

- RWE covered a broad array of observational studies. RWD were most often existing data from health care interactions. However, primary data collection and registries also were used.
- Studies of effectiveness were most commonly referenced as "real world," but treatment, cost, and safety studies were also present.
- In infectious diseases, these outcomes covered more than 90% of all "real-world" studies, primarily due to the increased proportion of effectiveness studies compared with cardiovascular and oncology therapeutic areas.
- Inference was made for more than 30% of abstracts regarding whether a study was retrospective and the type of study design. Clarity of reporting is needed. The number and diversity of publications from 2017 reinforce the interest and importance of gathering these data.

LIMITATIONS

- We assessed only the titles and abstracts of studies; additional details on study design, outcomes, and therapeutic area would be available within the full article.
- This high-level analysis implies that differences in reporting exist across therapeutic areas. Further investigation is warranted to understand nuances of reporting RWE by therapeutic area.

CONCLUSIONS

- RWD is crucial to demonstrate the utilization, safety, and effectiveness of health technologies outside clinical trials.
- As the number of RWE studies is expected to increase over the coming years, we recommend that reporting guidelines (such as the ISPOR/ISPE guidelines) be adopted and utilized.

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