

Webinar Title: Considerations when collecting real world data (RWD) and evaluating real world evidence (RWE) from Expanded Access programs

Presenters:

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More information on ERWE and CUPA can be found [here](#).

Session Description: While randomized controlled trials (RCTs) are considered the gold standard for evaluating the efficacy of an investigational product, sometimes RCTs are not feasible due to cost, practicality, or timeliness. Furthermore, strict inclusion/exclusion criteria for entry into an RCT may limit the generalizability of study results. In 2016, the U.S. Food and Drug Administration passed the 21st Century Cures Act, which endorsed the use of real world data (RWD) in regulatory decision making. RWD are defined as any data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources, e.g., electronic health records, claims and billing activities, product and disease registries, home-use settings, and mobile devices outside the confines of a traditional RCT. RWD may, in some situations, generate real world evidence (RWE), i.e., insights allowing for decision making regarding the usage and potential benefits or risks of a medical product. While RWD is intended to supplement RCTs, in situations where trials are not possible (e.g., rare diseases), RWE may be a useful substitute. RWE might also be useful as an adjunct to trial data, for example in understanding how trial findings translate to a more diverse population of patients than participated in the study.

An example of where RWD collection might be particularly desired is Expanded Access, historically referred to as “compassionate use.” Expanded Access is a regulatory pathway that allows patients to use an investigational product outside of a clinical trial when four conditions are met: the patient has a life-threatening or serious disease, no comparable or satisfactory alternative treatment options are available, clinical trial enrollment is not possible, and non-trial use does not pose a threat to timely clinical development. Data collection from this non-trial use of unapproved medical products offers an opportunity to learn about the products’ safety and efficacy in situations where there is no ongoing clinical trial or in patients who are outside the parameters of the trial of the product.

Recently, the notion of RWD from Expanded Access being used to generate RWE has increased in popularity. However, assessing the validity of RWD collected from Expanded Access programs can be challenging, as we will demonstrate by reference to two cases. Furthermore, Expanded Access is intended as treatment, not research; when the thin line between research and treatment dissolves, as in the case of treatment use of unapproved medical products, it can be difficult to determine how much and what data should be collected, how long patients should be followed-up,

what is reasonable to expect of clinicians with regard to reporting and monitoring, and whether patients must consent to the data collection to obtain access to the desired investigational medical product. In this session, we will discuss key ethical and design considerations for evaluating RWD collected from Expanded Access programs and highlight some of the reasons that stakeholders ranging from sponsors to payers to patient advocates are excited about the possibility of collecting RWD from Expanded Access.

Time Allocation of Session: 60 minutes total:

- Defining RWD, including how it is used in research (5 minutes)
- Defining Expanded Access, and how RWD collected from Expanded Access programs differ from other observational studies or clinical trials (5 minutes)
- Opportunities and limitations for collecting RWD from Expanded Access programs (10 minutes)
- Potential benefits and harms of collecting RWD from sponsor, payer, and patient advocacy perspectives (10 minutes)
- Ethical and research regulatory standards when collecting RWD from Expanded Access (10 minutes)
- Statistical considerations for RWD collected from Expanded Access programs that yield high-quality and impactful research (10 minutes)
- Audience Questions (10 minutes)

Target Audience: This session will target a diverse audience as we anticipate the content would be relevant to trialists, statisticians, industry representatives, patients and/or patient advocates, ethicists, and any individual interested in learning more about RWD generated both within and outside of Expanded Access programs, and how to evaluate RWD from Expanded Access programs as a potential source of RWE.