

## What is an Effectiveness Study?

Clinical trials can be designed to test **efficacy** (*whether an intervention delivers an effect under ideal conditions*) or **effectiveness** (*whether an intervention delivers an effect in the real world*). Effectiveness studies are often referred to as pragmatic trials. Efficacy studies are called explanatory trials. <sup>[1-9]</sup>

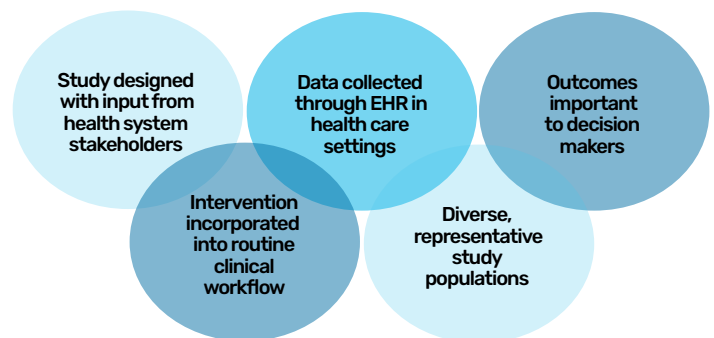
### Features of an Effectiveness Study

When a treatment is determined to be efficacious in an explanatory trial, it has usually been compared to a placebo or attention control group. Results help guide clinical decision making, but in everyday practice, there is a difference between the ideal conditions of the efficacy trial and the complexities present in health care practice (e.g., wide variety of patients who present for care, geographical and community differences, costs and economic factors, and more) <sup>[1, 6, 9]</sup>. Effectiveness studies attempt to fill this gap in knowledge for health care providers, patients, and other stakeholders (families, payers, government agencies, advocacy groups). These trials will usually involve the health care providers who normally attend to the patients on a daily basis <sup>[7, 8]</sup>. Results from a pragmatic trial are often more generalizable to the variety of patients typically seen in health care settings. Effectiveness studies are therefore on a continuum with efficacy studies <sup>[5, 9]</sup>.

### How to Design an Effectiveness Study

Effectiveness studies usually involve the comparison of two interventions that have established efficacy. They can also compare an intervention to standard care. Typically, participants are randomly assigned to a given treatment group. The researchers will need to identify the study population of interest and where they will be found (in health

facilities or in the community). Before designing a new study, it is important to review the findings and conclusions of similar studies conducted in other sites, as well as to include stakeholders (patients, families, clinicians) in the decision making about study design and study execution. This refines the research question(s) <sup>[1, 3, 5]</sup>.



When a number of efficacy trials and subsequent effectiveness trials have been completed for a particular clinical condition, health care providers, patients and families, and other stakeholders can incorporate available evidence so that treatment decisions can lead to optimal outcomes. Sometimes multiple studies demonstrate a clear treatment pathway, but there can also be mixed results. Only additional clinical research and experience in the real world of clinical care can eventually lead to more clarity about best clinical practices.

## References

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