Making Sense of Biostatistics: Efficacy vs. Effectiveness
By Melissa Pressman

Efficacy and effectiveness: These two related terms are often considered synonyms, but they actually have very different and specific definitions in clinical research and medicine.

**Efficacy**
Efficacy measures how well a study treatment produces a desired therapeutic result in an idealized setting like a randomized, controlled, double-blinded clinical trial with strict eligibility criteria. The more tightly controlled the clinical trial, the easier it may be to measure the therapeutic effect. However, the tighter the controls, the less likely the clinical trial findings are to translate into clinical practice.

**Effectiveness**
Efficacy does not always translate into effectiveness. Effectiveness measures how well a treatment works in the “real world,” with its diverse patient populations, wide variety of concomitant medications and illnesses, irregular regimen adherence, variable physician prescription writing and perceptions, off-label use, etc. For example, the New England Healthcare Institute (NEHI) has found that one-third to one-half of all patients do not properly take their medications due to financial constraints.

**Example**
A good example of the difference between these efficacy and effectiveness is the influenza vaccine. Current literature shows that the efficacy of the influenza vaccine is 50-70% in adults under the age of 65, while the effectiveness in this same population is 40-60%.

In this case, efficacy describes the prevention of illness among those vaccinated in controlled clinical trials, while effectiveness describes the prevention of illness in the vaccinated general population.

According to the CDC, there may be a difference between these two values for a number of reasons, such as age, additional confounding medication conditions, immune-competence of the vaccine recipient, the degree of similarity between the viruses in the vaccine and those in circulation, the outcome(s) being measured, and the accuracy and completeness of data collected for the general population.

**Summary**
The randomized clinical trial is the gold standard for determining the *efficacy* of a treatment. *Effectiveness* assesses the treatment response under “real world” settings. Therapies have to be *efficacious* to reach the market and *effective* to succeed in the market.

**References**


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