

Research Minute

Research Design

Issue 4

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The purpose of *research design* is to systematically answer questions about how things work, while controlling bias and ruling out alternative explanations. Over many years of scientific discovery, researchers have developed a variety of methods for answering research questions. Each design has its own strengths and weaknesses. *None are perfect. All have bias.* The researchers' job is to find the methodology that best answers the question while ruling out bias and considering ethical standards, costs, and feasibility.

Research design can be classified as retrospective or prospective. *Retrospective* studies rely on gathering information from pre-existing information, while *prospective* studies make a plan for data collection that carries forward in time.

Design is also classified as observational or interventional. *Observational* designs simply mean that the researcher is not performing an intervention. *Intervention* designs can test a variety of interventions, including medicines, devices, procedures, group visits, education programs, psychotherapy, or other special programs.

What is Research Bias?

Anything that produces systematic (but unexpected) variation in a research finding

Observational Designs

Descriptive Studies.

Most research is about the associations between 2 or more phenomena—but not this. A *Descriptive Study* examines one phenomenon at one point in time. This design is good for simple prevalence studies. Examples: the prevalence of foot amputations, or prevalence of patients' access to the internet, or patients' use of complementary/alternative medicines.



Even more interesting are *Analytic Studies* that examine the association between Two Phenomena.

Cross-Sectional Designs.

This design represents a one-pass collection of data in a particular period of time. Unlike *Descriptive Studies*, the researcher gathers information about two phenomena, and analyzes the associations between them. For example, are foot amputations associated with poverty? Is access to the internet associated with health literacy? Is CAM use associated with health status? **Cross-sectional studies** cannot show cause and effect, but they do underscore important relationships between two phenomena.

Case Control Studies.

This is a *retrospective design* that often uses medical records to determine the differences between people with and without a particular condition. One starts by first identifying the presence/absence of the **outcome** (Phenom 2), then tracing backward in time to identify the **predictors** (Phenom 1) of that outcome. Consider "Cases" with foot amputation due to diabetes. Identify a "Control" group of similar people with no amputation (similar gender, age, time since diagnosis). Then ask, "what are the differences between these two groups that might account for Cases having amputations?" This method is excellent for examining rarer conditions.



Cohort Studies.

This is a *prospective design* that follows a "cohort" of people over time, measuring predictors (Phenom 1) and outcomes (Phenom 2) along the way. The researcher does not intervene, but does periodic assessments to determine changes over time. This is a good design for following the natural history of a condition, but it is a bad design for rare conditions or conditions that slowly develop. Example: the Residency Research Network of Texas followed back pain patients over 3 years to assess changing levels of pain, functioning, depression and medication use. One finding: long periods of narcotic medicine use predicted new onset of depression.

Intervention Designs

PreTest/PostTest Intervention Design

By design, Intervention Studies are prospective and analytic. Phenomenon 1 is the intervention (the subject is exposed or not). Phenomenon 2 is the change in outcomes.

Pretest/Posttest designs are the simplest types of intervention research designs. The investigator draws one sample group, pretests them, conducts an intervention, the posttests them to see if they changed after the intervention. You use this method for **Quality Improvement projects**. (1) Pretest residents' and staff knowledge, attitudes, skills, and do a baseline measure of the clinical documentation. (2) Conduct an intervention which includes education about the topic plus changes in clinical procedure. (3) Posttest everyone, revisit the clinical documentation and assess for improvement.

This design does not demonstrate cause-and-effect. Other things may have happened to change people's behavior or condition—which is why clinical researchers use control groups. In a controlled trial, if both groups change, *something else* might be driving the change.

Pragmatic Clinical Trials

These clinical trials measure an intervention's effectiveness in *real world settings* by testing the intervention in a full range of people—those who have variable compliance, comorbidities and polypharmacy. Inclusion and exclusion criteria are less strict than other clinical trials.

Controlled Clinical Trials

Clinical Trials come in different shapes and sizes. The Gold Standard of clinical trials are **Double-blind Randomized Controlled Trials**. The investigator *randomly* assigns a subject into either a Control or Treatment group. The subject does not know whether she receives the treatment or the placebo; the person administering the treatment also does not know which is which—hence, double-blinding. Subjects in both groups complete pretests, receive the treatment or control condition, then complete posttests.

This method is excellent for showing cause-and-effect, because it controls several types of bias. Strict subject inclusion and exclusion criteria rule out extraneous influences on the intended outcome. Control groups address maturation bias (where change might occur even without treatment). Blinding the patient controls for Hawthorne effect. Blinding the physician controls for investigator bias. Randomization controls for other types of bias we cannot anticipate.

Some interventions cannot be blinded, such as trials of surgical interventions, procedures, educational interventions, or massage. One can still identify control groups, however, and randomly assign subjects to control or intervention groups.



Stages in Testing New Therapies

You may have read about FDA trials of new medications or medical devices. This table defines the different levels of testing for FDA-approved treatments.

Pre-Clinical	Studies in cell cultures and animals
Phase I Trial	Unblinded, uncontrolled studies in a small sample to test safety
Phase II Trial	Small samples, randomized & blinded, to test tolerability and initial effect of dose intensity on clinical outcomes
Phase III Trial	Large double-blind randomized controlled trials to test the effect on clinical outcomes
Phase IV Trial	Large trials after FDA approval to assess rate of serious side effects & evaluate additional therapeutic uses.

Comparative Effectiveness Research

Comparative Effectiveness Research (CER) is the direct comparison of existing health care interventions to determine which work best for which patients and which pose the greatest benefits and harms. The core question of comparative effectiveness research is: *which treatment works best, for whom, and under what circumstances?*

Systematic Reviews and Meta-analysis

Systematic Reviews identify completed studies about a specific research question and evaluate them as a group to understand the body of research. Investigators identify all relevant studies, display the results of eligible studies and calculate a summary estimate of the overall results. **Meta-analysis** is the statistical approach to calculating a composite effect of all the studies' results. The Cochrane Collaboration is well-known for systematic reviews of medical tests and treatments. Most **Systematic Reviews** summarize the effects of interventions, but they are not limited to that.