

Chapter 5 Introduction to Study Designs

Types of Medical Research

	Primary Research	Secondary Research
Basic Medical Sciences	✓	✓
Clinical Research	✓	✓

- Primary Basic Medical Science Research
- Primary Clinical Research
- Secondary Basic Medical Science Research
- Secondary Clinical Research

Primary research vs. secondary research

Primary Research	Primary research presents original data that appear for the first time. Data are not published before.
Secondary Research	Data were published before, and the investigators are retrieving these data to summarize them, add them, re-analyze them, or synthesize new evidence from them. Secondary research includes a summary, collection, and/or synthesis of existing research.

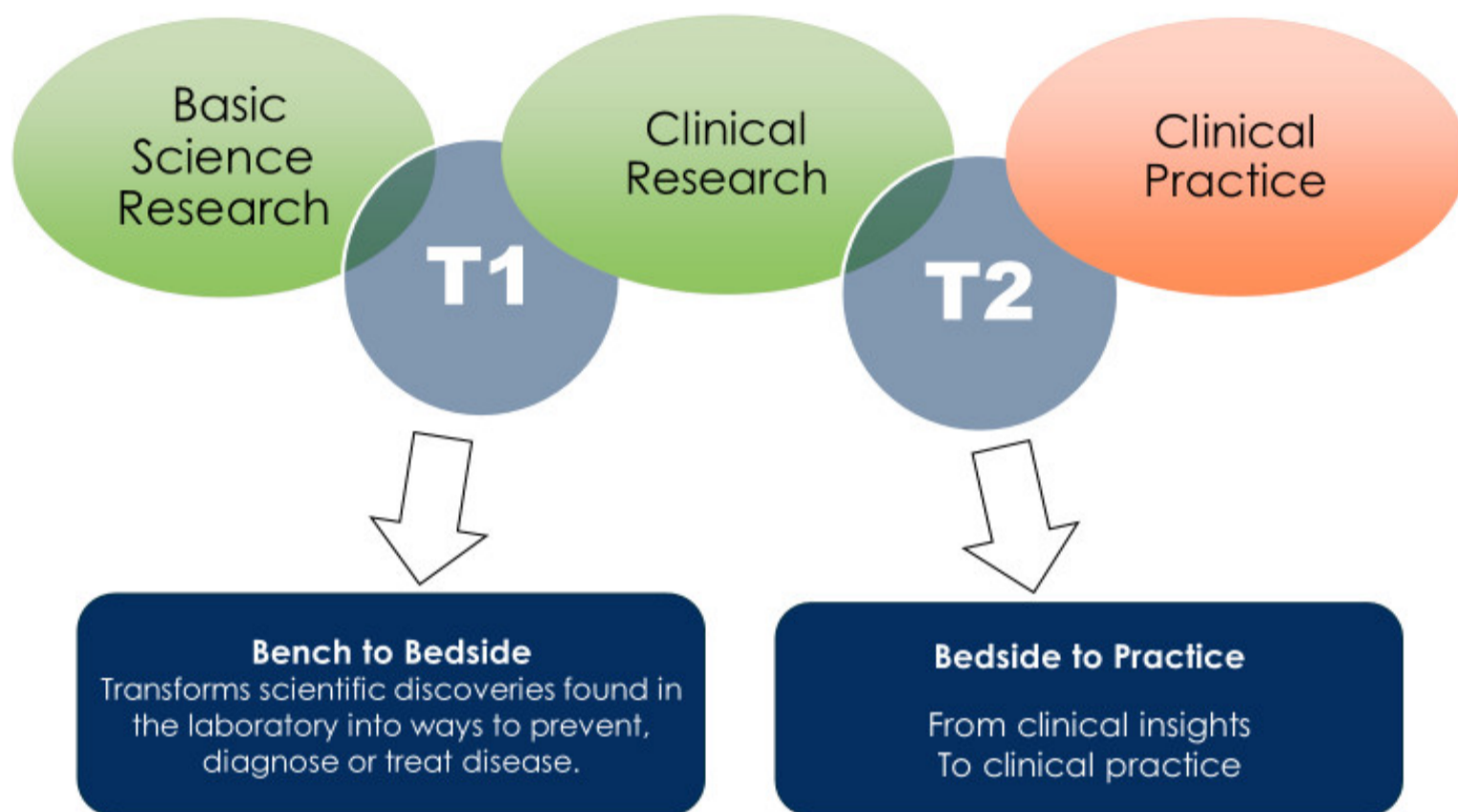
Basic science research vs. clinical research

Basic Science Research	It is called "test-tube research" because it requires well-prepared laboratories. This type of research studies fundamental functions in biology as molecular mechanisms, cell cycle, receptors, and genes. This type of research might involve animal models and tissue cultures. It is usually more powerful and has more scientific rigor since it generates more knowledge. However, it has no direct clinical relevance.
Clinical Research	This type of research deals with patients, and it requires patients not animals. It relies on patient data, and therefore, it has direct clinical relevance (unlike basic science research).

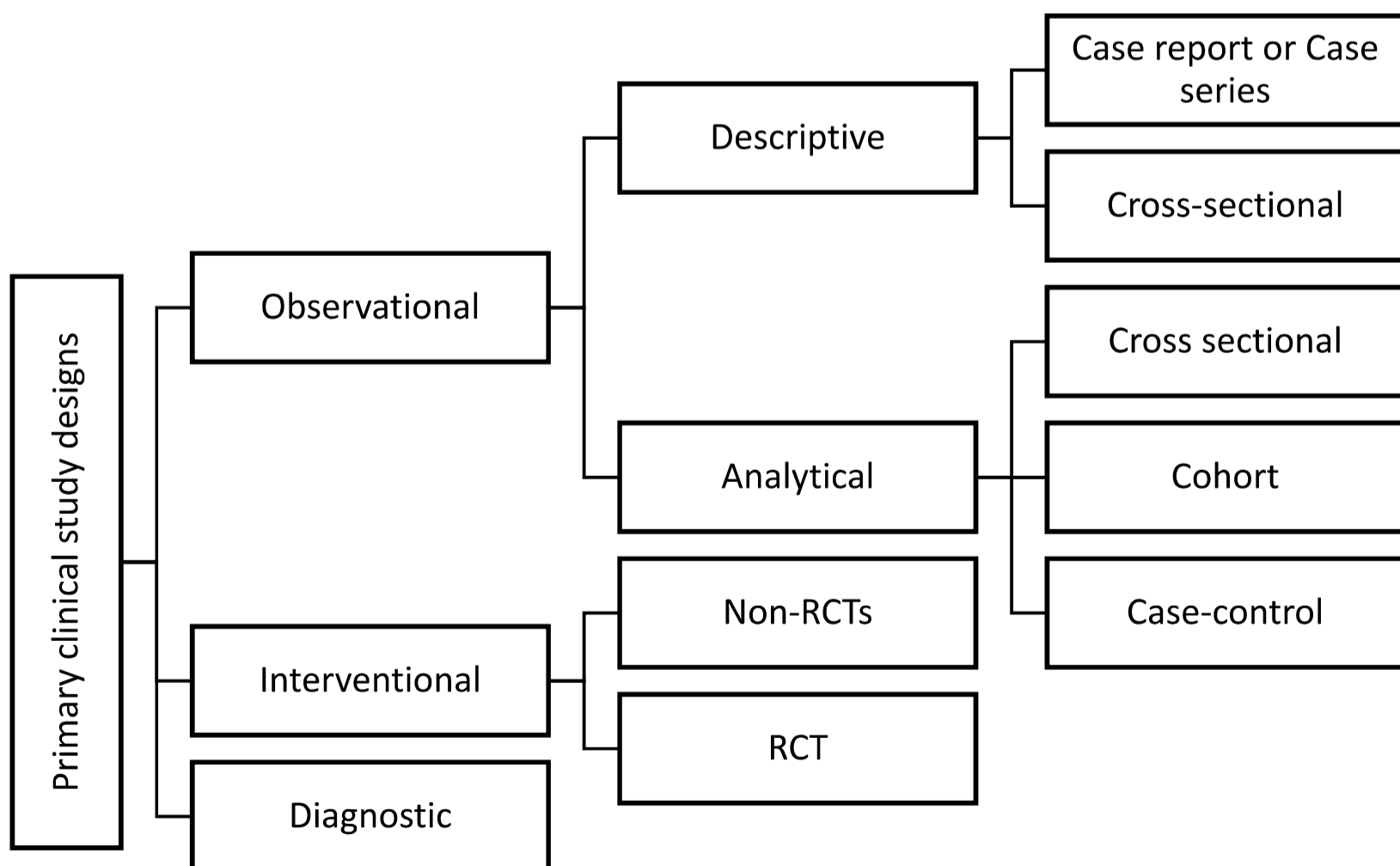
Translational research

It is a type of medical research that translates the findings of basic science research into clinical research (T1) or translates findings of clinical research into clinical practice (T2). For example, basic science research showed that VEGF is implicated in the pathogenesis of renal cell carcinoma. Subsequent basic science research studies

involved developing the drug that can bind to VEGF and testing this drug on the molecular level. The next step is to test this drug in animal models. All these steps are classified as basic science research. When enough preclinical evidence exists about the safety and efficacy of this drug, we will move to clinical evaluation of this drug in humans. This is called "the first in-human trial." Research studies that transfer basic science research findings to clinical evaluation in humans are called translational research (T1).



What are the primary clinical study designs?



Types of Primary Clinical Research

Primary Clinical Research	
Observational	Interventional
<p>This type of studies does not involve any intervention or experiment. The researchers are observing some variables in the patient without exposing the patients to a specific drug or intervention in the context of the research study.</p>	<p>This type of studies include an intervention or an experimentation. This intervention might be a surgical operation, a drug, or any other form of treatment. The investigators manipulate this risk factor to examine its effect on this population.</p>

What is the importance of observational studies?

- Unlike interventional studies where the investigators are controlling the experiment, observational studies evaluate the efficacy of drugs in real-life practice without influencing the current practice.
- Observational studies are useful if the experimentation was unethical, e.g., to study the effects of heavy smoking, we could not allocate the study participants to smoke or not to smoke.
- Observational studies are useful if the experimentation was difficult to implement, e.g., to test whether adding chloride to water affect population health, we have to compare the population of two areas that are naturally supplied by water with vs. without chloride. In this example, the researchers can not control which individuals will be supplied by drinking water that includes chloride.
- Clinical trials might include atypical subjects, which makes their results not generalizable on the target population. Observational studies rely on real-world data, which makes their results more generalizable to the diseased population. For example, patients who agree to participate in clinical trials are usually different from the diseased population in the clinic (for example, they will be more motivated to the experiment and are more likely to adhere to the treatment, unlike patients who receive the drug in real-life).
- For initial evaluation and generation of hypotheses, we can use observational studies. If the hypothesis was found to be possibly correct through observational studies, clinical trials might be followed. For example, some investigators suggested that diabetes mellitus share some pathophysiological pathways with Parkinson's disease, which means that antidiabetic medications might be effective in treating Parkinson's disease. In order to evaluate this hypothesis, observational studies

were data and data suggested that patients who take that antidiabetic medications of piloglitazones are at less risk of developing Parkinson's disease. The next step was to conduct randomized controlled trials testing the efficacy of piloglitazones for patients with Parkinson's disease.

The table shows the definition of the clinical research designs

Study design			
Case report	Describe	Clinical picture	One case
Case series	Describe	Clinical picture	> 1 case
Cross sectional	Describe	Prevalence	Study population
Cross-sectional	Analyze	Variables	within population
Cohort study	Compare	Risk	Between two groups
Case-control	Compare	Risk	Between two groups
Clinical Trial	Implement	Experiment	In a group of patients
	Evaluate	Efficacy/safety	
Randomized controlled trial	Evaluate	Efficacy/ Safety	of intervention in two groups of patients

What are the secondary clinical research designs?

