

Preparing a Research Study and Proposal



Simple Guidance in the Process:
Elizabeth S. Moore, Ph.D.
Mark A. Smith, M.S.
Vickie Dacey, M.S.
Robert Lubitz, M.D.



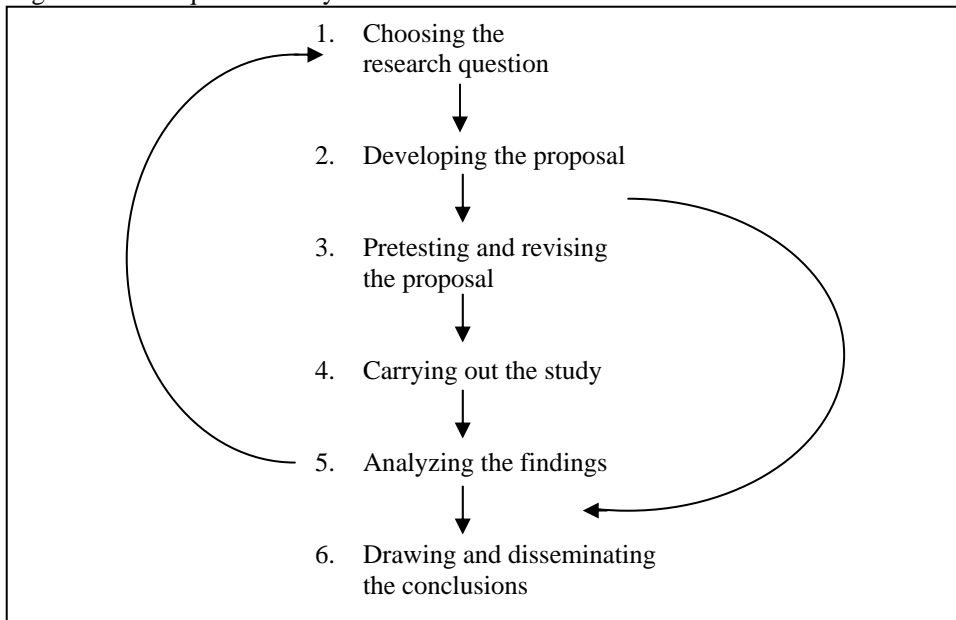
The following is a brief guide to help those interested in conducting health care research projects at St. Vincent Hospital and Health Services. This is meant to be a very general guide. For more specific information you may want to contact Elizabeth Moore, PhD, Mark Smith, MS or Bob Lubitz, MD.

Before we begin, let's consider one very basic question:

What is Research?

Research is finding out something you don't know through the use of scientific inquiry. Research can improve clinical practice by providing a process that can (1) provide answers to clinical questions, (2) evaluate effectiveness of interventions and patient care protocols, (3) expand physician knowledge by testing theories relevant to medicine and, (4) provide a link between education and practice (evidence-based medicine). The steps involved in a research study are shown in Figure 1. This guide will cover steps 1 and 2.

Figure 1: The sequence and cycle of research.



What is a Research Proposal?

A research proposal is a written presentation of a proposed study. It can be a short document (1-2 pages) that identifies and outlines the main components of the proposed research study, or it can be an elaborate document written for the purpose of obtaining funds from granting agencies or approval from an institutional review board.

Why Bother With a Research Proposal?

The research proposal basically lays out your ideas and intentions in a clear, concise manner. It also acts as a guide throughout your research, and helps keep you on course. The research proposal is not set in stone. The idea behind research is exploration, and delving into the unknown. If it is set in stone, why bother with research? Your proposal doesn't even have to be perfect. In fact, for those who aren't quite sure what your focus will be, the research proposal can be a space to explore options. It also can be an effective starting place to discuss your project with faculty and fellow researchers at the Academic Affairs Research Forum.

How Do I Get Started?

Working out a research study and proposal is a demanding task, one that transforms the research idea from a vague, but exciting, prospect into a set of actions that are manageable – and should be even more exciting. There are several questions you can answer to get the research area of your brain engaged:

What is your area of interest?

What do you want to focus on?

What resources are available?

What type of research project is feasible in terms of the time and energy you have available?

Now What?

Once you have answers to the questions above you can begin to design a study. Remember there are bound to be many changes between the first outline idea and the final fleshed out version and it is important to leave plenty of flexibility to adjust the components, so as to preserve consistency among them. Maintaining this consistency can be difficult if you write too soon: it is better to keep to notes or a draft until the study design is reasonably firm.

Designing a Study

Ask these questions:

1. What is the focus and aims of the study?
2. Why are these questions important?
3. What type of design should be used?
4. Who are the subjects and how will they be selected?
5. What measurements will be made?
6. How large is the study?
7. Is it adequate to answer the study questions?
8. How will the study be analyzed?

Determining the study focus

The very first step in designing a research study is to decide what area you are going to focus on. This will lead to a first draft of some research questions that, although likely to be changed later, are the basis for thinking further ahead.

A research question is the uncertainty about something in the population that you want to resolve by making measurements on study subjects. To come up with some research questions, many investigators have brainstorming sessions or discussions with friends, colleagues and/or faculty. The characteristics of a good research question can be remembered by using the mnemonic **FINER**.

Feasible

- Adequate number of subjects
- Adequate technical experience
- Affordable in time and money
- Manageable in scope

Interesting to the investigator

Novel

- Confirms or refutes previous findings
- Extend previous findings
- Provides new findings

Ethical

Relevant

- To scientific knowledge
- To clinical and health policy
- To future research directions

Aims of the Study

The research question provides the starting point from which an investigation is organized. However, a hypothesis is essential for all studies that compare an intervention and control. If you are going to conduct an analytic study you will need to formulate your research question into a research hypothesis. A hypothesis is a version of the research question that has the purpose of providing the basis for testing the statistical significance of the findings. Descriptive studies don't require a hypothesis because their purpose is to describe how variables are distributed (e.g., prevalence of AIDS virus antibodies) rather than how they are associated with each other.

A specific, rather than general, research hypothesis is very important because without well-defined hypotheses, it is more difficult at the end of the study to tell whether the study aims were met. Also, statistical significance testing is not reliable unless there is a study hypothesis to answer. Failure to clarify the hypothesis being tested makes it difficult to choose the appropriateness of the study design. An example of a non-specific hypothesis follows:

The degree of end-organ damage will correlate with the degree of hypertension. Riegelman, RK, Studying a Study and Testing a Test, 4th edition, 2000, p. 13.

This hypothesis is not specific enough to study. A more specific hypothesis might be:

An increased degree of narrowing of the retinal arteries, as measured on retinal photographs after 3 years of observation, will be associated with an increased level of diastolic blood pressure taken as the average of three blood pressure measurements at the beginning of the study. Riegelman, RK, Studying a Study and Testing a Test, 4th edition, 2000, p. 13.

This provides a specific hypothesis that can be addressed by an investigation. Another example of a specific hypothesis follows:

An increased degree of narrowing of the retinal arteries, as measured on retinal photographs after 3 years of observation, will be associated with an increased level of diastolic blood pressure taken as the average of three blood pressure measurements at the beginning of the study. Riegelman, RK and RP Hirsch, Studying a Study and Testing a Test, 1996, p. 59.

Importance

What is known about the topic at hand, why is your research study important, and what kind of answers will the study provide? Answers to these questions will set your study in context and give its rationale. In addressing this area you will want to conduct a literature search to find any previous relevant studies. In your critical review of prior studies, you will want to find any problems with the research and determine what questions remain. You will use this information in your research proposal to make clear how the findings from your study will help resolve uncertainties and influence clinical and public health policy.

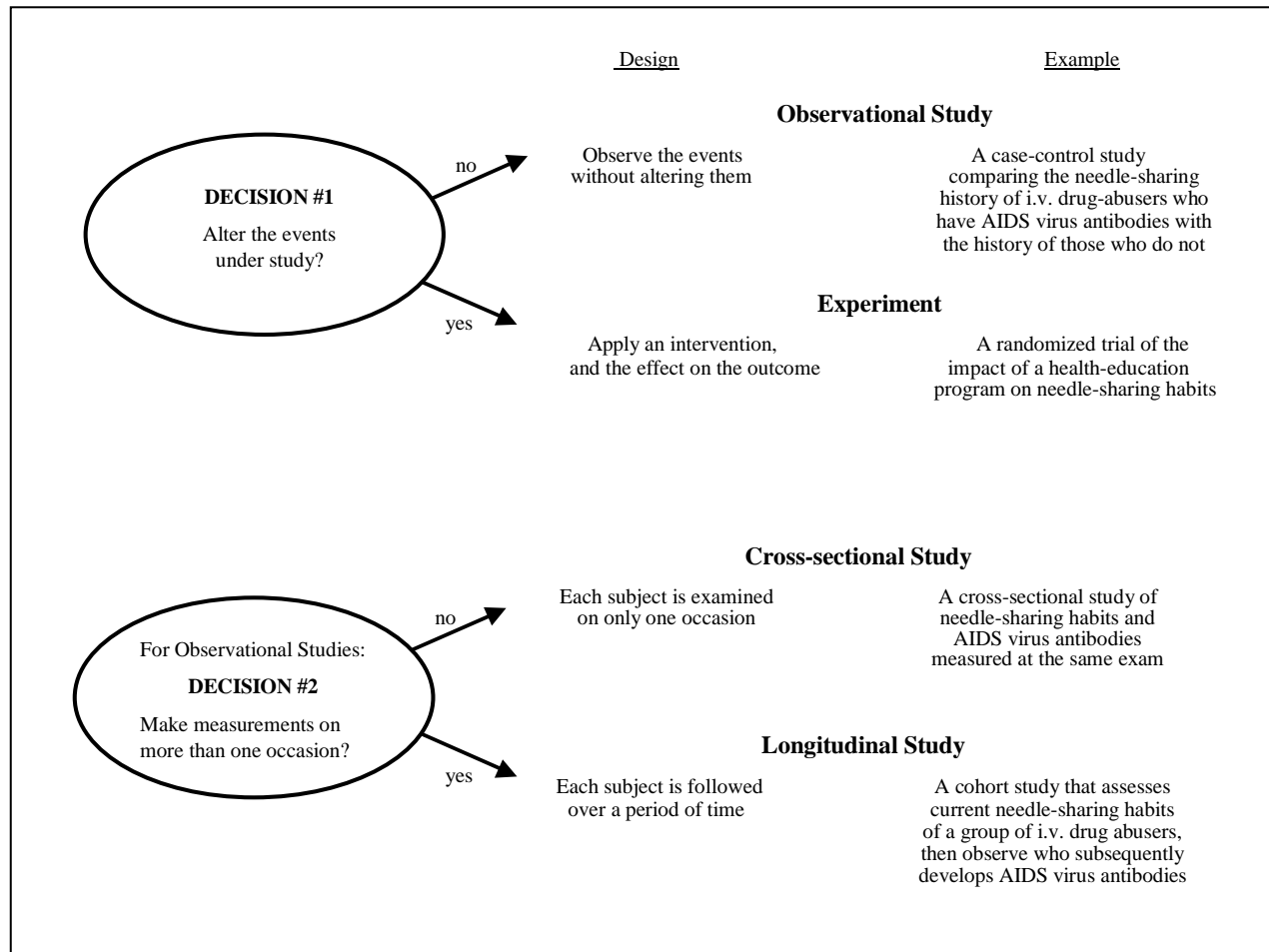
You now need to think about the type of research design you will use, and the statistics power and analysis that will be required for your study results to be valid.

What Type of Design Should I Use?

The design of a study involves a number of decisions (see Figure 2). The design of the study sets the rules for selecting subjects and measurement tools and determining the study size and the type of analysis to be conducted.

The most fundamental decision regarding the design of your study is whether to stand apart from the events taking place with the study subjects (**observational study**), or to test the effects of an intervention on these events (**experiment**). If you choose an observational design, your next decision is whether to make the measurements on a single occasion (**cross-sectional study**) or over a period of time (**longitudinal study**). A third aspect of the design decision (not shown in Figure 2) is whether to deal exclusively with past and present events in a **retrospective study**, or to follow study subjects **prospectively** for events that have not yet occurred when the study begins.

Figure 2. Some design decisions



No one approach is always better than the others; for each study a judgment must be made as to which design is the most efficient way to get a satisfactory answer. Figure 3 presents 4 of the most common research study designs.

Figure 3. Basic description of the 4 most common research study designs

Study Design	Type	Advantages	Disadvantages
Case-control study	Observational study	<ul style="list-style-type: none"> Useful for studying rare conditions/diseases Short duration Examine multiple variables that might be associated with a disease or condition, i.e., alcohol and/or cigarette use, diet, age, etc. Useful for generating a hypothesis about new disease or an unusual outbreak Sometimes the only method for establishing a prior association Relatively inexpensive Yields odds ratio (usually a good approximation of relative risk) 	<ul style="list-style-type: none"> Prone to a variety of methodological errors and biases (such as reporting or recall bias) Does not yield incidence, prevalence, or excess risk Limited to 1 outcome variable Does not establish sequence of events
Cohort study	Observational study	<ul style="list-style-type: none"> Establishes sequence of events Avoids bias in measuring predictors Avoids survival bias Can perform nonconcurrent cohort studies to examine characteristics over time. Yields incidence, prevalence, and excess risk Able to look at various consequences from a single risk factor 	<ul style="list-style-type: none"> Can be expensive and time-consuming Often requires large sample sizes Not feasible for rare outcomes

Cross-sectional studies	Observational study	<ul style="list-style-type: none"> • May study several outcomes • Study characteristic and outcome measured at the same point in time • Control over selection of subjects • Control over measurements • Usually inexpensive and short time frame • Useful when time between exposure and disease is short • Yields prevalence, relative prevalence 	<ul style="list-style-type: none"> • Doesn't establish sequence of events • Potential bias in measuring predictors • Potential survivor bias • Not feasible for rare conditions • Does not yield incidence or true relative risk
Randomized clinical trials	Experiment	<ul style="list-style-type: none"> • Gold standard • Can be used for large studies • Subjects randomized to study or control group • Often use masking or blinding • Establishes treatment efficacy • Establishes associations and prior associations • Demonstrates that altering the cause can alter the effect 	<ul style="list-style-type: none"> • Sample population doesn't often represent the target population • Subject loss to follow-up • Limited ability to assess secondary effects of intervention

What Should Be Measured?

Chose variables that will be needed to answer your research questions (or accept or reject your hypothesis).

Variables are characteristics of the study subjects such as blood pressure, ejection fraction, BMI. In a descriptive study individual variables are looked at one at a time. In an analytic study you will analyze the relationships among 2 or more variables in order to predict outcomes and to draw inferences about cause and effect. If your study includes an intervention, you need to decide who will be responsible for the intervention, where it will take place, what activities will be performed, and what frequency and intensity.

Characteristics of the Population Being Studied

What are the characteristics of the individuals in your study? Who will be included and who will be excluded? To answer these questions you will want to define your study population. The inclusion and exclusion criteria need to be carefully defined and these definitions will help you determine how to recruit or obtain subjects for the study. Inclusion criteria serve to identify the types of individuals who should be included. Exclusion criteria serve to remove individuals from eligibility because of special circumstances that may complicate their treatment or make interpretation more difficult. You will also want to consider the specific characteristics of the sample, (will age be a factor, race, previous medical history of the condition or disease under study, etc.).

Size of the Study Sample

All studies should have a sample size estimate. It is important to estimate the sample size early in the design phase. Waiting until the last minute to prepare the sample size can be disastrous. The goal of sample size planning is to estimate an appropriate number of subjects for a given study design. Sample size planning is best thought of as a scientific way of making a ballpark estimate. It often reveals that the research design is not feasible, or that different variables are needed.

You will also need to determine if a sample of this size will answer the questions posed by the study and will it allow you to demonstrate statistical significant differences between the intervention and control groups. What you really want the sample size to do is: estimate of the number of subjects required to detect an association of a *given effect size at a specified likelihood of making Type I (false-positive) and Type 2 (false-negative) errors* To give this estimate, the statistical power of the study needs to be determined. At this time you may want to consult with a statistician. Mark Smith is the statistician for St. Vincent, and is available for consultation.

Is the sample size adequate to answer the study questions?

To answer this question you need to decide what size of Type I and Type II errors you will tolerate. (Type II error is the probability of failing to demonstrate a statistically significant difference when a true difference does exist in the larger population from which the study and control groups were drawn.) For studies with prior hypotheses, this means estimating the number of subjects needed to consistently observe the expected difference in outcomes

between study groups. For descriptive studies, an analogous approach considers the number of subjects needed to produce descriptive statistics (means, proportion, etc.) of adequate proportion. The formulas used to calculate sample size are based on certain mathematical assumptions, which differ for each statistical test, which depend on the statistical approach to analyzing the data. The steps for conducting a statistical significance test are listed in Appendix A. Because the process of determining the sample size can be involved, it is recommended that you contact a statistician early in the study design process.

Data Collection and Analysis

A sometimes overlooked part of research planning is the preparation for data collection and analysis. You need to have a good plan for data collection and analysis. Based on your hypothesis and types of variables you chose, you need to determine how the variables will be measured and how they will be presented (quantitative and/or qualitative), indicating the analytical models and techniques (statistical, non-statistical, or analytical techniques for non-numeric data, etc.). You should also have a preliminary scheme for tabulating the data. It is recommended that special attention be given to the key variables that may be used in the statistical models.

How and Where To Begin?

Now that you have an idea of what will be in your research, you need to determine how and where you will get the information you will require. Some suggestions:

- Contact the St. Vincent Librarians
- Do a quick scan through the Internet for resource-rich websites
- Make contact with experts or people knowledgeable in your area of research.

Note: St. Vincent is blessed to have two very efficient and knowledgeable librarians, and the icing on the cake is that they are nice and willing to help. Contact the librarians to see how they can help you with conduct a literature search and obtain materials and other resources you will need to write your research proposal.

Preparing the Research Proposal

At this junction, your research proposal will be short written presentation (1-2 pages) of your study. It will identify and outline the main components of your research. Follow the outline below when writing your research proposal.

1. Title

Give your proposal a working title.

2. Specific Aims and Hypothesis(es)

Briefly set the context of your research interest, aiming to indicate the significance of your research topic, and state the nature of the research problem you wish to examine. This section is generally one page.

3. Background and Significance

In this section you should:

- Review appropriate literature to indicate the significance of your research questions and hypothesis
- Describe any preliminary studies that you have completed, and
- End with a brief statement of what is now known and what is not yet known.

4. Research Design and Methods

Explain how you will conduct your research in as much detail as possible. Discuss kinds of sources you hope to consult and the methods you will use to extract and process the information you gather in as much detail as is possible at this stage. Throughout this section, clearly indicate what specific question is addressed by each proposed experiment, potential difficulties, what results you expect to find, and how they would be interpreted. This includes a tentative sequence for the study and the statistical procedures by which the data will be analyzed.

5. Adverse Event Reporting

Adverse event data collection and reporting are required as part of every clinical trial and are reported in a routine manner at scheduled times.

6. Literature Cited

Present the complete citations for all the factual material you refer to in the text of your proposal. You can also make a list of texts your plan to consult. A complete citation includes the names of all authors, titles, book or journal, volume number, inclusive page numbers, and year of publication.

7. Appendix

Include copies of all materials that will be provided to study participants and any material that contributes to the proposal.

Final Comments

It is quite common to feel that, in order to write a good proposal, the research has virtually to be carried out! Indeed for large and expensive research the planning does sometimes require that a pilot trial is conducted. In general though, it is an exaggeration, but it indicates the effort that is required.

Remember, the planning, preparing, and presenting of the research proposal is not in any way a wasted effort, for research must at some point be meticulously planned if it is to have the rigor necessary to add to knowledge. This will not be the case unless we take note of what is already known, define our questions to take this into account and use appropriate methods of data collection and interpretation. It is much better to do this at the start than to be forced to change course when the research is underway because of events that should have been foreseen.

Further Questions?

Confused? Need more help? Have specific questions? There are several individuals that are available to help you design a research study and proposal.

Elizabeth S. Moore, PhD (Research Scientist)	338-2436	esmoore@stvincent.org
Mark Smith, MS (Clinical Statistician)	338-3924	masmith@stvincent.org
Bob Lubitz, MD (Director Internal Medicine and Academic Affairs)	338-2172	rlubitz@stvincent.org