

Research Proposals

how to communicate methodology

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Research Proposal: Methodology and Statistics

Convince your committee that your study is appropriately designed so that it can be succesful, effective and efficient. Keep in mind that not everyone in your committee is expert in your research domain, some are statisticians.

So you have to make clear

- what is the aim of your study
- how is your study designed to achieve that aim

This draft, based on shared experience in statistical consulting, highlights some issues and remains work in progress. Please share your suggestions for modifications and additions with ICDS (wilfried.cools@vub.be).

Consult the do's and donts page to avoid common misunderstandings.

Research Aim

Ask questions research can provide answers for !

FOCUS: specify -a limited number- of research questions, and be specific. Science is about finding what is looked for, not simply looking everywhere to describe what you see.

Make explicit

- what are the research questions (questions, not stories, not a description of results)
- what are the results -at a minimum- to consider the study successful (eg., significant difference)
- what are the expected results (eg., treatment outperforms control, an accurate estimate of the influence of age, ...)

The aim you specify has consequences for how you need to justify the study, what the implications and requirements are.

Maybe consider the following rough distinction in types of research that may help bring focus in your arguments. The types of design, for example experimental, observational, ... will be discussed in a later section.

The purpose of the research could be

- CONFIRMATORY
 - reason: confirm an expected difference, equality, relation, ... (you know what you are looking for)
 - focus: statistical test or parameter estimate with pre-specified confidence bounds

- requirement: sample size derivation and relation with cost/availability of observations
 - * decide what the result should minimally be
 - * specify the expected population variance
 - * discuss cost for the researcher / research unit / observation (eg., risks, money, time, ...)
 - * discuss availability of sample size (eg., could more be taken)
- EXPLORATORY
 - reason: to explore (collect data without a clear idea what the results should be)
 - justification: the need and importance for the extracted information on substantive grounds
 - focus A: data description and/or parameter estimation
 - * testing/accuracy is typically not the primary aim, but could be secondary
 - focus B: qualitative understanding
 - requirement: sample size -justification- in relation to costs
 - * balance -> value of new information vs. costs data collection
 - * sample size derivation may not be possible/meaningful
 - may not be possible/meaningful but remains required if significance is essential
 - consider sample size derivation based on confidence intervals
 - BEWARE: your study should be of interest without significant results !
- PREPARATORY
 - reason: to collect data (and monitor procedures) in preparation of -future- studies
 - justification: the need for such information for implementing succesful -future- studies
 - focus: small scale set-up to show the potential and/or detect issues
 - * phase I and II clinical designs
 - requires decision criteria to proceed or not
 - * pilot study: implementation of future study
 - not in itself of interest, no statistical testing, not intended for publication
 - requirement: minimal cost (material/physical/psychological) to get rough idea
 - * not necessarily including all aspects of future study
 - * eg., 3 observations per condition (allow for estimation of variance)
 - NOTE: qualitative research can be used preparatory too
- TECHNICAL - TECHNOLOGICAL advancements
 - reason: to design, engineer, create, ...
 - justification based on expected contribution versus costs, not statistics
 - * proof of concept: feasibility
 - * proof of principle: functionality (could it work in principle ?)
 - * development application
 - focus: rarely any statistics involved
 - requirement: methodology not related to quantitive research

Research Design

Garbage in... garbage out !

To answer your research question, information is required

- relevant information obtained from relevant observation
- with quality dependent on the
 - conditions of observation
 - eg., independent groups, cross-over, split-plot, ...
 - method of observation
 - eg., interview, survey, test result, ...
- with generalization dependent on the selection of research units
 - eg., large random sample, convenience sample, ...

Note: A poor design will make the study inefficient at best, and fully invalidate the study at worst.

From observation to information to conclusion

Books are written about design and its relation to statistics and inference.

common issues:

- how many observations (~ amount of information)
- what is the unit of analysis (eg., patient, test score at a given time, ...)
- how are the units of analysis allocated to the conditions (random, blocked, ...)
- how are the units of analysis selected from the population (random, stratified, convenience, ...)
- is the unit of analysis measured repeatedly (between / within)
- what are the possible observations (eg., positive continuous, low-medium-high, ...)
- which observations are most likely (eg., very many zero's, mostly low values occasionally high, ...)
- is the design balanced (same number of observations in each condition, at each time point)
- ...

All questions may be relevant to evaluate how to extract the required information and what can be concluded based on it.

Number of Observations: Quantity of Information

Observations provide information that is used to

- test (significance)
- estimate (confidence interval)
- predict (cross validation)
- understand (qualitative)

Observations typically involve a cost (money, time, ethical concerns, ...), so plan accordingly.

Sample sizes are determined depending on the type of research (see above)

- calculate how many observations are required for a successful study (significance / high enough accuracy)
- justify the sample size in relation to the cost of observation and value of information
- take the minimum that gives a rough idea

Note: determine sample size for primary research questions only (take highest when multiple exist = conservative)

The resulting sample size depends on:

- the statistical test in focus (eg., t-test for independent groups at the end of the study)
- the effect size aimed for (eg., .5: difference > 2, assuming pooled population standard error of 4)
- the operational characteristics (type I error α and type II error β)

Note: include all these elements in a proposal (do not simply state sample size, α and power)

The effect size aimed for is based on an effect and an uncertainty:

- effect: ideally specified on substantive grounds or at least with reference to common practice or earlier research
- uncertainty: should ideally be based on earlier data/research or pilot

Issue: if you are interested to detect a difference between two groups when you have more than two groups, do not derive sample sizes for the omnibus F-tests. Issue: sample sizes are always obtained for future studies, never as a justification for used sample sizes (retrospective power analysis is meaningless).

Allocation to Conditions of Observation: Quality of Information

Different types of research have different consequences on the quality of the collected information and its processing.

- QUANTITATIVE
 - main question is ‘how’
 - focus on population, aim at generalization
 - uses (ideally) representative samples
 - can be descriptive and/or inferential
- QUALITATIVE (holistic/subjective)
 - main question is ‘why’
 - focus on understanding of object of research: reasons, opinions, motivations, ...
 - typically explorative / hypothesis generating

Note: there is a continuum between the two and both can be used jointly/consecutively

For quantitative research:

The conditions under which observations are made determine their informational value, referred to as design.

A general principle is to

- experimentally control the confounding variables (randomisation, blocking, ...) as good as possible
- minimize non-systematic variability (eg., use a reliable tool for measurement)
- maximize systematic variability (eg., use conditions that are as different as possible given the hypothesis)

Control on confounding variables

- randomisation to balance out various disturbances
- enhance comparability (control for unwanted influences)
 - blocking (compare observation with others within a block)
 - repeated measures (compare observation on the same unit, but at different time points)
 - matching (create similar groups, except for the main effect in consideration)
 - cross-over designs (alternate treatments within a unit of observation)
 - ...

Note: the underlying reasoning always is to isolate the effect of interest either by avoiding or measuring unwanted influences.

Selection of Research Units: Generalizability

Generalization (inference) is dependent on the research units under study and how they are sampled.

For generalization, a big enough representative sample is required.

- probabilistic (sampling: random, stratified, cluster, multi-stage,)
- non-probabilistic (sampling: convenience, diversity, expert,)

As an example,

a random sample of elderly patients that you know personally strictly does not allow for generalization to

- young people
- non-patients
- people you do not know personally

or can you assume that they would show equivalent observations ?

Example

The aim is to show that the proposed treatment is an improvement over the current standard method.

Participants are randomized into two groups, the treatment and the control group. The control group is given a dummy treatment that mimics as good as possible the actual treatment. A post experiment survey will

address whether participants were aware about the use of a dummy treatment. Each participant is measured once, immediately after the (dummy) treatment was administered which results in one score per patient, continuous on a 0 to 10 scale. A t-test for independent means is used to evaluate whether the scores in the treatment group are higher than the scores in the control group.

A sample size was derived for the t-test to detect a minimal difference of 2 in favor of the treatment which was decided upon by our expert panel. The standard method is described in literature to have a population standard deviation of about 4 when used for our type of patients. Because no information is available on the new treatment it is assumed that the same population standard deviation applies. Therefore, a sample size of 51 patients in each of both groups is required for a one-sided test, type I error of .05 and power of .8. Because earlier experiments did show a drop-out of about 10%, $51/9 \times 10 < 57$ patients in each group are included. Note that there is no reason to expect serious deviations from normality. A small but possible risk that is unforeseeable is that there may be much more variance in the treatment group, which would complicate the analyses and increase the required number of participants slightly.

Statistics

Introduce the statistics that are planned

- in agreement with design
- able to answer the research questions vital for your study

Specify what is of primary interest and how it answers your questions

- p-value, confidence interval, probability of succesful prediction, ...
- example: test whether Treatment is not worse with 1 point on the scale compared to the Control, so p-

Reflect on likely challenges and how to deal with them for the analysis of main importance

- do not simply list your full statistics course 101, but explain why certain issues could arise
- example: deviations from normality often observed, dealt with using log-transformations.

Best practice (in the ideal world)

- create the data you expect using simulation during the design phase -before you have your data-
- verify whether your analysis would be able to extract the required information.

At least (in the practical world)

- give the final datafile as expected some serious thought and build the framework by specifying rows and

Note: do not state that you will be working with SPSS, R -and- Prism, choose only one if possible, it shows you do not know what you are doing.

Remember: even the most complex and advanced statistics would not make up for a poor design!