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 successful, 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).

Disclaimer: Current draft describes work in progress and its content should be treated as such. All provided information should be used as guidance but the researcher remains responsible for the actual choices and their communication.

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, check on the Net how to set up a good research question)
- 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.

Purpose

- 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)
  - minimum: statistical effect / sufficient accuracy for primary hypotheses
- 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
  - focus C: predictive modeling
  - 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
  - minimum: could be many things, as long as you can sufficiently argue its merit
  - 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 successful -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)
  - minimum: ensures information required for future study
  - NOTE: qualitative research can be used preparatory too, eg., mixed methods
- 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 quantitative research
  - minimum: particular state of advancement, improvement
  - NOTE: testing might involve additional statistics

Quantitative and Qualitative

Distinguishing between the how and the why

- Quantitative

  - focus on population, aim at generalization
  - uses (ideally) representative samples
  - can be descriptive and/or inferential
- Qualitative (holistic/subjective)
  - 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

Describe versus Infer

Distinguishing between the sample and the population

- Infer: 'population'
  - focus on population, aim at generalization
  - uses (ideally) representative samples
  - requires estimation of uncertainty
- Describe: 'sample'
  - focus on the observed data
  - present data as is
  - use no uncertainty of estimation, nor p-values

Note: inference usually is preceeded with descriptions

Research Design

Garbage in... garbage out !

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

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

To answer your research question, information is required

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

From observation to information to conclusion

Observations provide information that is used to

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

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 concluded based on it.

Quantity of Information: Number of Observations

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 statical 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, alpha 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 size are always obtained for future studies, never as a justification for used sample sizes (retrospective power analysis is meaningless).

Note: an equivalent notion in qualitative research is saturation, explain how you evaluate it

Quality of Information: Conditions of Observation

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

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 (randomization, 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

- randomization 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.

Generalizability: Selection of Research Units

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*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 analysis 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 successful prediction, ...

- example: test whether Treatment is not worse with 1 point on the scale compared to the Control, so p-value for non-inferiority $<.05$ with margin 1

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

- do not simply list all possible statistical techniques, 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 columns -before- data is collected.

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!