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presize: An R-package for precision-based sample size calculation in clinical research

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Software

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Summary

Sample size calculation is a crucial step for planning a clinical study. A study that is too small leads to inconclusive results; a study that is too large is a waste of resources. Either case might be unethical. Furthermore, Bland (2009) called for a focus on the width of confidence intervals rather than the power of the test in sample size calculations. Indeed, many research projects aim to estimate a quantity rather than test a hypothesis, sample size calculation approaches for which are largely missing from other software packages. There are many software packages for hypothesis-based sample size calculation, such as Stata, PASS, G*Power, including many R packages, such as `pwr` (Champely (2020)) and `TrialSize` (Zhang et al. (2020); see other packages detailed on the CRAN Clinical Trials taskview). To the best of our knowledge, only Stata provides precision-based approaches, and only then for a small number of statistics. We have, therefore, developed an R package for precision-based sample size calculation, `presize`, which can be used within the R-environment or a shiny application.

Development

`presize` is programmed in the R programming language (R Core Team (2020)), and offers sample size calculation for estimation-based research. We implemented the most common measures used in descriptive research, including descriptive, absolute and relative differences, correlation and diagnostic measures. There are two approaches for each measure. Firstly, based on a given sample size, e.g. for a retrospective data analysis, the precision of an expected measure can be calculated. Precision is expressed as the confidence interval around the measure. The level of confidence can be specified; the usual 95%-confidence interval (CI) is the default. Secondly, based on a given precision (i.e. CI), the sample size can be calculated. This is mainly of use in the planning of prospective studies, where the aim is to estimate a measure of interest with enough confidence. The available functions in the package require common input arguments; either the sample size or the width of the CI, and the level of the CI. Depending on the function, further specific input arguments are required such as the expected area under the curve (AUC) for test accuracy.

For ease-of-use, we have also implemented a Shiny application which can be used online or from within the R-environment. Values of parameters can be entered using rulers and numeric fields. Based on the given parameters, the application will either display the sample size for a given precision, or vice-versa, the precision for a given sample size. Moreover, the application also displays the corresponding R-code, which allows copying the command into the R-environment for further exploration as well as reproducibility.

Usage

presize is available on CRAN or GitHub and can be installed and loaded into the R session using

```
# installation:
# install.packages("presize") # CRAN
# remotes::install_github('ctu-bern/presize') # development version on GitHub
library(presize)
```

As a brief example, suppose we want to estimate the proportion of hospital admissions with diabetes. Diabetes has a prevalence of approximately 10% (Emerging Risk Factors Collaboration et al. (2010)). We assume a slightly higher proportion of diabetics, 15%, as diabetes is a risk factor for a wide range of conditions. We want to estimate the prevalence of diabetes to within 5% (plus/minus 2.5%). With presize, this is simple. We use the prec_prop (precision of a proportion) function and pass our 15% and 5% as arguments p and conf.width:

```
prec_prop(p = 0.15, conf.width = 0.05)
```

```
     sample size for a proportion with Wilson confidence interval.

     p      padj        n conf.width conf.level       lwr       upr
1 0.15 0.1517077 783.4897       0.05       0.95 0.1267077 0.1767077
```

In the n column, we see that we would need to ask 784 (rounding 783.5 up) patients to achieve the desired CI width. It is also possible to calculate the CI width with a given number of participants, for the case that we know roughly how many participants we could include:

```
prec_prop(p = 0.15, n = 600)
```

```
     sample size for a proportion with Wilson confidence interval.

     p      padj   n conf.width conf.level       lwr       upr
1 0.15 0.1522266 600 0.05713404       0.95 0.1236596 0.1807936
```

Discussion

We have developed a comprehensive and easy-to-use software package for precision-based sample size calculation. As far as we know, presize is the first package that comprises the most common summary measures used in estimation-based clinical research. A limitation of the package is that it does not allow calculating the probability of a CI, i.e. the probability that a future confidence interval will have at least the desired precision. The functions currently return the average CI width. In practice, 50% of trials will yield narrower CIs and 50% will yield wider CIs due to sampling variation. Providing a method to specify the coverage probability is one possible avenue for further development.

We often observe in our consulting activity that researchers try to implement a hypothesis-based approach into a project that is in fact purely descriptive. Reasons for this might be a lack of methodological understanding, but also a lack of appropriate tools. To conclude, we believe that our software package will facilitate the appropriate use of estimation-based sample size calculation in descriptive research projects.

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