Chapter 684

Pilot Study Sample Size Rules of Thumb

Introduction

This routine presents *rules of thumb* for finding an appropriate sample size for a pilot study in which the outcome is a continuous measurement. These results are for the total sample size of two-group studies in which the sample sizes of each group will be equal.

Pilot studies are conducted for many reasons including determining an estimate of the standard deviation. Unfortunately, if the outcome is a continuous measurement, little may be known about the standard deviation. This makes determining a sample size based on means and variances difficult. Eventually, a researcher just has to pick a sample size for the pilot study. This procedure generates reports which give the recommendations of statistical researchers as to the sample size of pilot studies.

The procedure is based on the information provided in Whitehead *et al.* (2016) and Machin *et al.* (2018).

Technical Details

Two Types of Rules of Thumb for Sample Sizes

Machin *et al.* (2018) explains that there are sample size rules of thumb may be categorized into two categories: *Flat* and *Stepped.* A *flat* rule of thumb is a single number that is suggested for every situation. For example, ‘30’ is a popular number.

A *stepped* rule of thumb is a number that depends on a limited amount of information about the main study such as the required power or the relative value of the effect size. The stepped rules are based on the NCT approach discussed by Whitehead *et al.* (2016).

These two categories have resulted to two sets of rules of thumb.

Procedure Options

This section describes the options that are specific to this procedure. These are located on the Design tab. For more information about the options of other tabs, go to the Procedure Window chapter.

Design Tab

The Design tab contains most of the parameters and options that you will be concerned with.

Solve For

Solve For

This option specifies the rules of thumb shown. The choices are *flat* or *stepped*.

Example 1 – Flat sample size rules of thumb

These example shows the report that is generated flat rules of thumb. It is based on Table 16.2 in Machin *et al.* (2018) found on page 266.

Setup

This section presents the values of each of the parameters needed to run this example. First, from the PASS Home window, load the **Pilot Study Sample Size Rules of Thumb** procedure. You may then make the appropriate entries as listed below, or open **Example 1** by going to the **File** menu of the procedure window and choosing **Open Example Template**.

Option	Value
Design Tab	
Solve For	**Flat Rules of Thumb**

Annotated Output

Click the Calculate button to perform the calculations and generate the following output.

Numeric Results

Numeric Results for Two-Group Studies

Reference	Recommended Pilot Study Sample Size	Comments
Birkett and Day (1994)	20	Suggested 20 for Internal pilot studies.
Browne (1995)	30	Mentions that the use of 30 is commonplace at the time
Kieser and Wassmer (1996)	20 to 40	Use when main trials are between 80 and 250 and using UCL.
Julious (2005)	24	Recommended minimum of 12 subjects per group.
Sim and Lewis (2011)	≥55	Use for small to medium effect sizes to minimize combined size.
Teare, et al. (2014)	≥70	Based on an extensive simulation study.

Note: These rules of thumb for two groups can be adapted for single-group or multi-group studies by multiplying the recommended pilot study sample size by an appropriate adjustment factor (e.g. 0.5 for a study with only one group or 1.5 for a study with three groups, etc.).

References

Machin, D, Campbell, M.J., Tan, S.B, Tan, S.H. 2018. 'Sample Sizes for Clinical, Laboratory and Epidemiology Studies, Fourth Edition'. John Wiley and Sons. Hoboken, New Jersey.
Whitehead, A.L., Julious, S, Cooper, C.L., Campbell, M.J. 2016. 'Estimating the sample size for a pilot randomised trial to minimise the overall trial sample size for the external pilot and main trial for a continuous outcome variable'. Stat Meth Med Res. Vol 25(3). Pages 1057-1073.
Birkett, M.A. and Day, S.J. 1994. 'Internal pilot studies for estimating sample size'. Statistics in Medicine. Vol 13. Pages 2455-2463.
Browne, R.H. 1995. 'On the use of a pilot study for sample size determination'. Statistics in Medicine. Vol 14. Pages 1933-1940.
Kieser, M and Wassmer, G. 1996. 'On the use of the upper confidence limit for the variance from a pilot sample for sample size determination'. Biometrical Journal. Vol 8. Pages 941-949.
Julious, S.A. 2005. 'Sample size of 12 per group rule of thumb for a pilot study'. Pharmaceutical Statistics. Vol 4. Pages 287-291.
Sim, J. and Lewis, M. 2011. 'The size of a pilot study for a clinical trial should be calculated in relation to considerations of precision and efficiency'. Journal of Clinical Epidemiology. Vol 65. Pages 301-308.
Teare, M.D., Dimairo, M., Shephard, N., Hayman, A., Whitehead, A, and Walters, S.J. 2014. 'Sample size requirements to estimate key design parameters from external pilot randomised controlled trials: A simulation study'. Trials. Vol 15. Page 264.

This gives various sample size suggestions for two-group studies found in the literature. These rules of thumb for two groups can be adapted for single-group or multi-group studies by multiplying the recommended pilot study sample size by an appropriate adjustment factor (e.g. 0.5 for a study with only one group or 1.5 for a study with three groups, etc.).

Example 2 – Stepped sample size rules of thumb

These example shows the report that is generated stepped rules of thumb. It is based on Table 8 in Whitehead *et al.* (2016) found on page 1071.

Setup

This section presents the values of each of the parameters needed to run this example. First, from the PASS Home window, load the **Pilot Study Sample Size Rules of Thumb** procedure. You may then make the appropriate entries as listed below, or open **Example 2** by going to the **File** menu of the procedure window and choosing **Open Example Template**.

Option	Value
Design Tab	
Solve For	**Stepped Rules of Thumb**

Annotated Output

Click the Calculate button to perform the calculations and generate the following output.

Numeric Results

Numeric Results for Two-Group Studies

Standardized Difference	80% Powered Main Trial Pilot Study Sample Size	90% Powered Main Trial Pilot Study Sample Size
Extra Small (δ/σ < 0.1)	100	150
Small (0.1 ≤ δ/σ < 0.3)	40	50
Medium (0.3 ≤ δ/σ < 0.7)	20	30
Large (0.7 ≤ δ/σ)	20	30

References

Machin, D, Campbell, M.J., Tan, S.B, Tan, S.H. 2018. 'Sample Sizes for Clinical, Laboratory and Epidemiology Studies, Fourth Edition'. John Wiley and Sons. Hoboken, New Jersey.

Whitehead, A.L., Julious, S, Cooper, C.L., Campbell, M.J. 2016. 'Estimating the sample size for a pilot randomised trial to minimise the overall trial sample size for the external pilot and main trial for a continuous outcome variable'.Stat Meth Med Res. Vol 25(3). Pages 1057-1073.

This gives various sample size suggestions for two-group studies based on the preliminary understanding of what power is used in the main study and what effective size will be targeted.