

Package ‘confidenceCurves’

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Type Package

Title Frequentist Confidence Analysis for Clinical Trials

Version 0.2.1

Description Frequentist confidence analysis answers the question: How confident are we in a particular treatment effect? This package calculates the frequentist confidence in a treatment effect of interest given observed data, and returns the family of confidence curves associated with that data.

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Imports cowplot (>= 1.1.3), DescTools (>= 0.99.54), ggplot2 (>= 3.5.1), rlang (>= 1.1.4)

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makeConfidenceCurves *Frequentist confidence analysis for any treatment effect*

Description

This function performs frequentist confidence analysis, given a point estimate and associated error estimation, to answer the question: How much confidence can we have in a particular treatment effect of interest?

Usage

```
makeConfidenceCurves(
  theta.estimator = NULL,
  estimator.type = NULL,
  treat.var = NULL,
  standard.error = NULL,
  confidence.lower = NULL,
  confidence.upper = NULL,
  sample.size = NULL,
  num.resp.ctrl = NULL,
  num.resp.trmt = NULL,
  num.ctrl = NULL,
  num.trmt = NULL,
  directory = "",
  show = "BENEFIT",
  pval = "TWO-SIDED",
  min.effect = -0.05,
  neutral.effect = 0,
  dir.benefit = 0,
  dir.min.effect = NULL,
  equiv = NULL,
  save.plot = FALSE,
  return.plot = FALSE,
  tag = ""
)
```

Arguments

theta.estimator	Enter the point estimate, assumed to follow a Normal distribution.
estimator.type	when entering binary data into inputs, specify "risk difference" or "odds ratio". For "odds ratio" option, the log odds will be used.
treat.var	Variance associated with the point estimate. Must be supplied with sample size.
standard.error	Standard error associated with the point estimate.
confidence.lower	Lower boundary of 95% confidence interval.

confidence.upper	Upper boundary of 95% confidence interval.
sample.size	Sample size. Can be calculated from num.ctrl and num.trmt.
num.resp.ctrl	When specifying binary input data; Number of responders in control group (who experienced the outcome).
num.resp.trmt	When specifying binary input data; Number of responders in treatment group (who experienced the outcome).
num.ctrl	When specifying binary input data; Number of subjects in the control group.
num.trmt	When specifying binary input data; Number of subjects in the treatment group.
directory	Character string expressing directory where you want to save the confidence curve family. Default is "".
show	On the confidence density function, which region to display in shaded blue: BENEFIT' (default), 'LMB' (lack of meaningful benefit), 'MB' (meaningful benefit) or 'EQUIV' (equivalence).
pval	Specify "ONE-SIDED" or "TWO_SIDED" test for returned p-value. Default is "TWO-SIDED".
min.effect	The minimally clinically interesting effect (meaningful benefit). Default is -0.05.
neutral.effect	Value corresponding to no effect. Default is 0. If using odds ratio, data should be on the log scale for a neutral effect of 0.
dir.benefit	Direction (0 or 1) around the neutral effect corresponding to benefit. 0: less than no effect value (default); 1: more than no effect value.
dir.min.effect	Direction (0 or 1) around the min effect that you're interested in. 0: less than min effect value; 1: more than min effect value. Default assumes LACK of meaningful benefit.
equiv	If interested in expressing confidence in treatment equivalence, you can specify two numbers as c(a,b) to bound the equivalency region. Default uses min.effect and -min.effect as a and b
save.plot	save the plot as png to directory, TRUE or FALSE (default).
return.plot	Return the plots from the function, TRUE or FALSE (default).
tag	phrase to append to the image filename as <directory>/confidence_curves_<tag>.png. Default is "".

Details

This is a function to perform frequentist confidence analysis on a observed treatment estimate. You may either supply a point estimate and associated precision estimate via standard error, variance and sample size, and 95% CI interval, or enter outcome data directly (the latter option is only available for binary data). Then, define a neutral effect, and a meaningful clinical effect, and the direction of interest (above or below these) and the function will calculate how much confidence one can have in the associated treatment effect (e.g., beneficial, lacking meaningful benefit). Also returned is the traditional frequentist p-value.

Value

Returns a list of values associated with confidence analysis (under \$text) and (if supplied TRUE to return.plot) four confidence curves

References

Equations used in this function are derived from Marschner, I. "Confidence distributions for treatment effects in Clinical Trials: Posteriors without Priors", Statistics in Medicine, 2024;43:1271-1289.

Examples

```
makeConfidenceCurves(
  theta.estimator = -0.22,
  confidence.lower = -0.36,
  confidence.upper = -0.07
)
```

testConfidenceCurves *Test confidence curves*

Description

A short function to test makeConfidenceCurves for binary data

Usage

```
testConfidenceCurves(
  num.ctrl = 50,
  num.trmt = 50,
  vary.ctrl = seq(16, 20, by = 2),
  vary.trmt = seq(26, 30, by = 2),
  vary.lmb = c(-0.05, -0.1),
  estimate.type = "odds ratio",
  return.plot = TRUE,
  save.plot = FALSE,
  directory = "./test"
)
```

Arguments

num.ctrl	Number of subjects in control group. Default is 50.
num.trmt	Number of subjects in treatment group. Default is 50.
vary.ctrl	List of numbers to vary response in control group. Default is c(16, 18, 20).
vary.trmt	List of numbers to vary response in treatment group. Default is c(26, 28,30).

vary.lmb	List of numbers to vary definition of meaningful benefit. Default is c(-0.05, -0.1).
estimate.type	Type of estimator, options are "risk difference" and "odds ratio" (default).
return.plot	Return and print the CFD plot. TRUE (default) or FALSE.
save.plot	Save the family of confidence curves to directory. TRUE or FALSE (default).
directory	Location to save images. Default is './test'.

Value

Returns a dataframe

Examples

```
# to fix control and treatment responders
testConfidenceCurves(vary.lmb = c(-0.05, -0.1), vary.ctrl = c(18), vary.trmt = c(28))
# to run without showing plots
testConfidenceCurves(
  vary.lmb = c(-0.05, -0.1),
  vary.ctrl = c(18),
  vary.trmt = c(28),
  return.plot=FALSE)
```

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