Package 'preference'

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Type Package

Title 2-Stage Clinical Trial Design and Analysis

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URL https://github.com/kaneplusplus/preference

BugReports https://github.com/kaneplusplus/preference/issues

Description Design and analyze two-stage randomized trials with a continuous outcome measure. The package contains functions to compute the required sample size needed to detect a given preference, treatment, and selection effect; alternatively, the package contains functions that can report the study power given a fixed sample size. Finally, analysis functions are provided to test each effect using either summary data (i.e. means, variances) or raw study data.

License LGPL-2

Suggests knitr, testthat, ggplot2,

VignetteBuilder knitr

RoxygenNote 6.0.1

LazyData true

NeedsCompilation no

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R topics documented:

`analyze_raw_data` *Analysis Function: Raw Data*

Description

Computes the test statistic and p-value for the preference, selection, and treatment effects for the two-stage randomized trial using provided raw data

Usage

```
analyze_raw_data(x1, x2, y1, y2, s11 = 1, s22 = 1, s1 = 1, s2 = 1,
  xi = 1, nstrata = 1)
```

Arguments

`x1`	vector of responses for patients choosing treatment 1
`x2`	vector of responses for patients choosing treatment 2
`y1`	vector of responses for patients randomized to treatment 1
`y2`	vector of responses for patients randomized to treatment 2.
`s11`	(if study is stratified) vector of stratum membership for patients choosing treatment 1. Should be a vector of the same length as x1 with the number of unique values equal to the number of strata.
`s22`	(if study is stratified) vector of stratum membership for patients choosing treatment 2. Should be a vector of the same length as x2 with the number of unique values equal to the number of strata.
`s1`	(if study is stratified) vector of stratum membership for patients randomized to treatment 1. Should be a vector of the same length as y1 with the number of unique values equal to the number of strata.

s2	(if study is stratified) vector of stratum membership for patients randomized to treatment 2. Should be a vector of the same length as y2 with the number of unique values equal to the number of strata.
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Rucker G (1989). "A two-stage trial design for testing treatment, self-selection and treatment preference effects." *Stat Med*, **8**(4):477-485. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
#Unstratified
x1<-c(10,8,6,10,5)
x2<-c(8,7,6,10,12,11,6,8)
y1<-c(10,5,7,9,12,6)
y2<-c(8,9,10,7,8,11)
analyze_raw_data(x1,x2,y1,y2)
#Stratified
x1<-c(10,8,6,10,5)
s11<-c(1,1,2,2,2)
x2<-c(8,7,6,10,12,11,6,8)
s22<-c(1,1,1,1,2,2,2,2)
y1<-c(10,5,7,9,12,6)
s1<-c(1,1,1,2,2,2)
y2<-c(8,9,10,7,8,11)
s2<-c(1,1,1,2,2,2)
analyze_raw_data(x1,x2,y1,y2,s11=s11,s22=s22,s1=s1,s2=s2,xi=c(0.5,0.5),
nstrata=2)
```

---

analyze_summary_data *Analysis Function: Summary Data*

Description

Computes the test statistic and p-value for the preference, selection, and treatment effects for the two-stage randomized trial using provided summary data

Usage

```
analyze_summary_data(x1mean, x1var, m1, x2mean, x2var, m2, y1mean, y1var, n1,
  y2mean, y2var, n2, xi = 1, nstrata = 1)
```

Arguments

x1mean	mean of responses for patients choosing treatment 1. If study is stratified, should be vector with length equal to the number of strata.
x1var	variance of responses for patients choosing treatment 1. If study is stratified, should be vector with length equal to the number of strata.
m1	number of patients choosing treatment 1. If study is stratified, should be vector with length equal to the number of strata.
x2mean	mean of responses for patients choosing treatment 2. If study is stratified, should be vector with length equal to the number of strata.
x2var	variance of responses for patients choosing treatment 2. If study is stratified, should be vector with length equal to the number of strata.
m2	number of patients choosing treatment 2. If study is stratified, should be vector with length equal to the number of strata.
y1mean	mean of responses for patients randomized to treatment 1. If study is stratified, should be vector with length equal to the number of strata.
y1var	variance of responses for patients randomized to treatment 1. If study is stratified, should be vector with length equal to the number of strata.
n1	number of patients randomized to treatment 1. If study is stratified, should be vector with length equal to the number of strata.
y2mean	mean of responses for patients randomized to treatment 2. If study is stratified, should be vector with length equal to the number of strata.
y2var	variance of responses for patients randomized to treatment 2. If study is stratified, should be vector with length equal to the number of strata.
n2	number of patients randomized to treatment 2. If study is stratified, should be vector with length equal to the number of strata.
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Rucker G (1989). "A two-stage trial design for testing treatment, self-selection and treatment preference effects." *Stat Med*, **8**(4):477-485. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
x1mean<-5
x1var<-1
m1<-15
x2mean<-7
```

```
x2var<-1.1
m2<-35
y1mean<-6
y1var<-1
n1<-25
y2mean<-8
y2var<-1.2
n2<-25
analyze_summary_data(x1mean,x2var,m1,x2mean,x2var,m2,y1mean,y2var,n1,y2mean,
y2var,n2)
```

`effects_from_means` *Calculate Effect Sizes from Means*

Description

Calculates the preference, selection and treatment effects given the means of each treatment group in the choice and random arms for the 2-stage randomized study.

Usage

```
effects_from_means(mu1, mu2, mu11, mu22, phi, nstrata = 1, xi = NULL)
```

Arguments

`mu1`	mean response of the patients receiving treatment 1 in the random arm. For unstratified design, should be numeric value. For the stratified design, should be vector of length equal to number of strata with each entry corresponding to stratum- specific mean.
`mu2`	mean response of the patients receiving treatment 2 in the random arm. For unstratified design, should be numeric value. For the stratified design, should be vector of length equal to number of strata with each entry corresponding to stratum- specific mean.
`mu11`	mean response of the patients choosing treatment 1 in the choice arm. For unstratified design, should be numeric value. For the stratified design, should be vector of length equal to number of strata with each entry corresponding to stratum- specific mean.
`mu22`	mean response of the patients choosing treatment 2 in the choice arm. For unstratified design, should be numeric value. For the stratified design, should be vector of length equal to number of strata with each entry corresponding to stratum- specific mean.
`phi`	proportion of patients preferring treatment 1. For unstratified design, should be numeric value. For the stratified design, should be vector of length equal to number of strata with each entry corresponding to stratum-specific preference rate. All elements should be numeric values between 0 and 1.
`nstrata`	number of strata. Default is 1 (unstratified design).

`xi` a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. Should only be specified for stratified design.

References

Rucker G (1989). "A two-stage trial design for testing treatment, self-selection and treatment preference effects." *Stat Med*, **8**(4):477-485. (PubMed)

Examples

```
effects_from_means(mu1=1, mu2=2, mu11=1.5, mu22=2.5, phi=0.5)
```

---

imap *Summary data for the IMAP study*

Description

Data set is from the Improving Management of Abnormal Pap Smears study, which used a two-stage randomized preference trial design to evaluate psychosocial outcomes in women found to have atypical cells in a Pap Smear. Two systems for managing the atypical cells were tested (repeated Pap smears or HCV triage) and a doubly randomized design was used to evaluate the role of patient preference. The data set provides mean, standard deviation and sample sizes of the SF36 outcome for each treatment in both the choice and random arms.

References

McCaffery et al. (2010) "Psychosocial outcomes of three triage methods for the management of borderline abnormal cervical smears: an open randomised trial." *BMJ*, **340**:b4491. (PubMed)

McCaffery et al. (2011) "Determining the Impact of Informed Choice: Separating Treatment Effects from the Effects of Choice and Selection in Randomized Trials." *Med Decis Making*, **31**(2):229-236. (PubMed)

---

imap_strat *Stratified summary data for the IMAP study*

Description

Data set is from the Improving Management of Abnormal Pap Smears study, which used a two-stage randomized preference trial design to evaluate psychosocial outcomes in women found to have atypical cells in a Pap Smear. Two systems for managing the atypical cells were tested (repeated Pap smears or HCV triage) and a doubly randomized design was used to evaluate the role of patient preference.

In this data set, patients are stratified according to their baseline score on the six-item abbreviated Spielberger State-Trait Anxiety Inventory (STAI). This assessment is used to assess the level of

anxiety experienced by patients. Stratum 1 includes patients with low STAI scores (<=10 on STAI averaged across whole study), which indicates low levels of anxiety. Stratum 2 includes patients with high STAI scores (>10), indicating higher levels of anxiety. The data set provides mean, standard deviation and sample sizes of the SF36 outcome for each treatment in both the choice and random arms for both strata.

References

McCaffery et al. (2010) "Psychosocial outcomes of three triage methods for the management of borderline abnormal cervical smears: an open randomised trial." *BMJ*, **340**:b4491. (PubMed)

McCaffery et al. (2011) "Determining the Impact of Informed Choice: Separating Treatment Effects from the Effects of Choice and Selection in Randomized Trials." *Med Decis Making*, **31**(2):229-236. (PubMed)

Marteau TM, Bekker H. (1992) "The development of a six-item short-form of the state scale of the Spielberger State-Trait Anxiety Inventory (STAI)." *Br J Clin Psychol*, **31**:301-306. (PubMed)

`optimal_proportion` *Unstratified Optimized Theta*

Description

Calculates the optimal proportion of patients assigned to the choice arm in an unstratified two-stage randomized trial

Usage

```
optimal_proportion(w_sel, w_pref, w_treat, sigma2, phi, delta_pi, delta_nu)
```

Arguments

`w_sel`	weight assigned to the estimation of the selection effect. Each weight should be a numeric value between 0 and 1 and sum of three weights should be 1.
`w_pref`	weight assigned to the estimation of the preference effect. Each weight should be a numeric value between 0 and 1 and sum of three weights should be 1.
`w_treat`	weight assigned to estimation of the treatment effect. Each weight should be a numeric value between 0 and 1 and sum of three weights should be 1.
`sigma2`	variance estimate. Should be a positive numeric value.
`phi`	proportion of patients preferring treatment 1. Should be numeric value between 0 and 1.
`delta_pi`	overall study preference effect.
`delta_nu`	overall study selection effect.

References

Walter et. al. (2011). "Optimal allocation of participants for the estimation of selection, preference and treatment effects in the two-stage randomised trial design." *Stat Med*, **31**(13):1307-1322. (PubMed)

Examples

```
optimal_proportion(w_sel=0.2, w_pref=0.4, w_treat=0.4, sigma2=1, phi=0.5,
delta_pi=1, delta_nu=0.5)
```

`overall_power` *Power Calculation from Sample Size*

Description

Calculates the study power to detect a set of effects given a particular sample size in a two-stage randomized clinical trial

Usage

```
overall_power(N, phi, sigma2, delta_pi, delta_nu, delta_tau, alpha = 0.05,
  theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

`N`	overall study sample size.
`phi`	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
`sigma2`	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
`delta_pi`	overall study preference effect.
`delta_nu`	overall study selection effect.
`delta_tau`	overall study treatment effect.
`alpha`	desired type I error rate.
`theta`	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
`xi`	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
`nstrata`	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
overall_power(N=300, phi=0.6, sigma2=1, delta_pi=1, delta_nu=0.5,
delta_tau=1.5)
# Stratified
overall_power(N=300, phi=c(0.6,0.5), sigma2=c(1,1), delta_pi=1, delta_nu=0.5,
delta_tau=0.5, xi=c(0.5,0.5), nstrata=2)
```

`overall_sample_size` *Overall Sample Size*

Description

Calculates the sample size required to detect a given set of effects in a two-stage randomized clinical trial. Returns the largest of the required sample sizes for a given set of treatment, selection, and preference effects.

Usage

```
overall_sample_size(power, phi, sigma2, delta_pi, delta_nu, delta_tau,
  alpha = 0.05, theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

`power`	desired study power. Should be numeric value between 0 and 1.
`phi`	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
`sigma2`	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
`delta_pi`	overall study preference effect.
`delta_nu`	overall study selection effect.
`delta_tau`	overall study treatment effect.
`alpha`	desired type I error rate.
`theta`	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).

xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
overall_sample_size(power=0.8, phi=0.5, sigma2=1, delta_pi=1, delta_nu=0.5,
delta_tau=1.5)
# Stratified
overall_sample_size(power=0.8, phi=c(0.5,0.4), sigma2=c(1, 1), delta_pi=1,
delta_nu=0.5, delta_tau=1.5, xi=c(0.3,0.7),nstrata=2)
```

---

preference *Design and Analysis of Two-stage Randomized Clinical Trials*

Description

The **preference** package is used for the design and analysis of two-stage randomized trials with a continuous outcome measure. In this study, patients are first randomized to either a random or choice arm. Patients initially randomized to the choice arm are allowed to select their preferred treatment from the available treatment options; patients initially randomized to the random arm undergo a second randomization procedure to one of the available treatment options. The design has also been extended to include important stratification variables; the functions provided in this package can accommodate both the unstratified and stratified designs.

In this study, there are three effects that may be of interest. The treatment effect captures the difference in outcome between patients randomized to treatment A and treatment B (similar to a traditional RCT). The selection effect captures the difference in outcome between patients that prefer treatment A and patients that prefer treatment B, regardless of the treatment that is actually received. Finally, the preference effect compares the outcomes of patients who receive their preferred treatment (either treatment A or treatment B) and patients who do not receive their preferred treatment.

To aid in the design of these two-stage randomized studies, sample size functions are provided to determine the necessary sample size to detect a particular selection, preference, and/or treatment effect. If the sample size is fixed prior to the start of the study, functions are provided to calculate the study power to detect each effect. Finally, the `optimal_proportion` function can be used to

determine the optimal proportion of patients randomized to the choice arm in the initial randomization.

To analyze the data from the two-stage randomized trial, two analysis functions are provided. The function `analyze_raw_data` computes the test statistic and p-value for each effect given provided raw study data. The function `analyze_summary_data` uses provided summary data (mean, variance, and sample size) of each study group to compute the test statistic and p-value of each effect.

Sample Size Function calls

- selection_sample_size: required sample size to detect a given selection effect
- preference_sample_size: required sample size to detect a given preference effect
- treatment_sample_size: required sample size to detect a given treatment effect
- overall_sample_size: required sample size to detect a given set of selection, preference, and treatment effects

Power Function Calls

- selection_power: study power to detect a given selection effect
- preference_power: study power to detect a given preference effect
- treatment_power: study power to detect a given treatment effect
- overall_power: study power to detect a given set of selection, preference, and treatment effects

Analysis Function Calls

- analyze_raw_data: computes test statistic and p-value for observed selection, preference, and treatment effects using provided raw data
- analyze_summary_data: computes test statistic and p-value for observed selection, preference, and treatment effects using provided summary data (mean, variance, sample size)

Other Function Calls

- treatment_effect_size: computes the treatment effect that can be detected given a specified sample size and power
- optimal_proportion: computes the optimal proportion randomized to choice arm (defined for unstratified design only)
- effects_from_means: computes the treatment, selection, and preference effect sizes provided the study means in each treatment arm

Data Sets

- imap: summary SF36 outcome data for the two-stage randomized IMAP study
- imap_strat: summary SF36 outcome data for the two-stage randomized IMAP study stratified by high vs. low STAI score

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Disclaimer: All statements in this report, including its findings and conclusions, are solely those of the authors and do not necessarily represent the views of the Patient-Centered Outcomes Research Institute (PCORI), its Board of Governors or Methodology Committee.

References

Rucker G (1989). "A two-stage trial design for testing treatment, self-selection and treatment preference effects." *Stat Med*, **8**(4):477-485. (PubMed)

McCaffery et al. (2010) "Psychosocial outcomes of three triage methods for the management of borderline abnormal cervical smears: an open randomised trial." *BMJ*, **340**:b4491. (PubMed)

Walter et. al. (2011). "Optimal allocation of participants for the estimation of selection, preference and treatment effects in the two-stage randomised trial design." *Stat Med*, **31**(13):1307-1322. (PubMed)

McCaffery et al. (2011) "Determining the Impact of Informed Choice: Separating Treatment Effects from the Effects of Choice and Selection in Randomized Trials." *Med Decis Making*, **31**(2):229-236. (PubMed)

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

`preference_power` *Preference Effect Power Calculation*

Description

Calculates the study power to detect the preference effect given a particular sample size in a two-stage randomized clinical trial

Usage

```
preference_power(N, phi, sigma2, delta_pi, delta_nu, alpha = 0.05,
  theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

`N`	overall study sample size.
`phi`	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
`sigma2`	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
`delta_pi`	overall study preference effect.
`delta_nu`	overall study selection effect.
`alpha`	desired type I error rate.

theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
preference_power(N=300, phi=0.6, sigma2=1, delta_pi=1, delta_nu=0.5)
# Stratified
preference_power(N=300, phi=c(0.6,0.5), sigma2=c(1,1), delta_pi=1,
delta_nu=0.5, xi=c(0.5,0.5), nstrata=2)
```

---

preference_sample_size

Preference Effect Sample Size

Description

Calculates the sample size required to detect a given preference effect in a two-stage randomized clinical trial

Usage

```
preference_sample_size(power, phi, sigma2, delta_pi, delta_nu, alpha = 0.05,
  theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

power	desired study power. Should be numeric value between 0 and 1.
phi	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.

delta_pi	overall study preference effect.
delta_nu	overall study selection effect.
alpha	desired type I error rate.
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
preference_sample_size(power=0.8, phi=0.6, sigma2=1, delta_pi=1, delta_nu=0.5)
# Stratified
preference_sample_size(power=0.8, phi=c(0.5, 0.5), sigma2=c(1, 1), delta_pi=1,
 delta_nu=0.5,xi=c(0.3,0.7),nstrata=2)
```

---

selection_power *Selection Effect Power Calculation*

Description

Calculates the study power to detect the selection effect given a particular sample size in a two-stage randomized clinical trial

Usage

```
selection_power(N, phi, sigma2, delta_pi, delta_nu, alpha = 0.05,
  theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

N	overall study sample size.
phi	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
delta_pi	overall study preference effect.
delta_nu	overall study selection effect.
alpha	desired type I error rate.
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
selection_power(N=300, phi=0.6, sigma2=1, delta_pi=1, delta_nu=0.5)
# Stratified
selection_power(N=300, phi=c(0.6,0.5), sigma2=c(1,1), delta_pi=1,
delta_nu=0.5, xi=c(0.5,0.5), nstrata=2)
```

---

selection_sample_size *Selection Effect Sample Size*

Description

Calculates the sample size required to detect a given selection effect in a two-stage randomized clinical trial

Usage

```
selection_sample_size(power, phi, sigma2, delta_pi, delta_nu, alpha = 0.05,
  theta = 0.5, xi = 1, nstrata = 1)
```

Arguments

power	desired study power. Should be numeric value between 0 and 1.
phi	the proportion of patients preferring treatment 1. Should be numeric value between 0 and 1. If study is stratified, should be vector with length equal to the number of strata in the study.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
delta_pi	overall study preference effect.
delta_nu	overall study selection effect.
alpha	desired type I error rate.
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
selection_sample_size(power=0.8, phi=0.6, sigma2=1, delta_pi=1, delta_nu=0.5)
# Stratified
selection_sample_size(power=0.8, phi=c(0.5, 0.5), sigma2=c(1,1), delta_pi=1,
 delta_nu=0.5,xi=c(0.3,0.7),nstrata=2)
```

treatment_effect_size *Treatment Effect Back Calculation*

Description

Calculates the treatment effect that can be detected given a desired study power and overall study sample size for the two-stage randomized design

Usage

```
treatment_effect_size(N, power, sigma2, alpha = 0.05, theta = 0.5, xi = 1,
  nstrata = 1)
```

Arguments

N	overall study sample size.
power	desired study power. Should be numeric value between 0 and 1.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
alpha	desired type I error rate.
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

Examples

```
treatment_effect_size(N=300,power=0.9,sigma2=c(1,0.8), xi=c(0.3,0.7),
nstrata=2)
```

treatment_power *Treatment Effect Power Calculation*

Description

Calculates the study power to detect the treatment effect given a particular sample size in a two-stage randomized clinical trial

Usage

```
treatment_power(N, sigma2, delta_tau, alpha = 0.05, theta = 0.5, xi = 1,
  nstrata = 1)
```

Arguments

N	overall study sample size.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
delta_tau	overall study treatment effect.
alpha	desired type I error rate..
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
treatment_power(N=300, sigma2=1, delta_tau=0.5)
# Stratified
treatment_power(N=300, sigma2=c(1,1), delta_tau=0.5, xi=c(0.5,0.5), nstrata=2)
```

---

treatment_sample_size *Treatment Effect Sample Size*

Description

Calculates the sample size required to detect a given treatment effect in a two-stage randomized clinical trial

Usage

```
treatment_sample_size(power, sigma2, delta_tau, alpha = 0.05, theta = 0.5,
  xi = 1, nstrata = 1)
```

Arguments

power	desired study power. Should be numeric value between 0 and 1.
sigma2	variance estimate. Should be positive numeric values. If study is stratified, should be vector of within-stratum variances with length equal to the number of strata in the study.
delta_tau	overall study treatment effect.
alpha	desired type I error rate.
theta	proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5).
xi	a numeric vector of the proportion of patients in each stratum. Length of vector should equal the number of strata in the study and sum of vector should be 1. All vector elements should be numeric values between 0 and 1. Default is 1 (i.e. unstratified design).
nstrata	number of strata. Default is 1 (i.e. unstratified design).

References

Turner RM, et al. (2014). "Sample Size and Power When Designing a Randomized Trial for the Estimation of Treatment, Selection, and Preference Effects." *Medical Decision Making*, **34**:711-719. (PubMed)

Cameron B, Esserman D (2016). "Sample Size and Power for a Stratified Doubly Randomized Preference Design." *Stat Methods Med Res*. (PubMed)

Examples

```
# Unstratified
treatment_sample_size(power=0.8, sigma2=1, delta_tau=1.5)
# Stratified
treatment_sample_size(power=0.8, sigma2=c(1, 1), delta_tau=1.5,
                      xi=c(0.3,0.7),
nstrata=2)
```

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