Package 'preference'

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Type Package

Title 2-Stage Preference 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

Imports ggplot2, tidyr

Suggests testthat

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`preference-package` *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)

`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)
```

---

fit_preference *Fit the Preference Data Collected from a Two-stage Clinical Trial*

Description

Computes the test statistics and p-values for the preference, selection, and treatment effects for the two-stage randomized trial using collected outcome, random, treatment, and strata values for specified significance level.

Usage

```
fit_preference(outcome, random, treatment, strata, alpha = 0.05)
```

Arguments

outcome	(numeric) individual trial outcomes.
random	(logical) was this individual part of the random arm?
treatment	(character, factor, or integer) which treatment an individual received
strata	(optional integer) which strata the individual belongs to.
alpha	(optional numeric) Level of significance (default=0.05)

Examples

```
# Unstratified
outcome <- c(10, 8, 6, 10, 5, 8, 7, 6, 10, 12, 11, 6, 8, 10, 5, 7, 9, 12, 6,
             8, 9, 10, 7, 8,11)
random <- c(rep(FALSE, 13), rep(TRUE, 12))
treatment <- c(rep(1, 5), rep(2, 8), rep(1, 6), rep(2, 6))
fit_preference(outcome, random, treatment)

# Stratified
# Same data plus strata information.
strata <- c(1,1,2,2,2,1,1,1,1,2,2,2,2,1,1,1,2,2,2,1,1,1,2,2,2)
fit_preference(outcome, random, treatment, strata, alpha=0.1)
```

`fit_preference_summary` *Fit Preference Model from Summary Data*

Description

Computes the test statistics and p-values for the preference, selection, and treatment effects in a two-stage randomized trial using summary data.

Usage

```
fit_preference_summary(x1mean, x1var, m1, x2mean, x2var, m2, y1mean, y1var, n1,
  y2mean, y2var, n2, xi = 1, nstrata = 1, alpha = 0.05)
```

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).
alpha	Type I error rate, used to determine confidence interval level for the effect estimates. Default is 0.05 (i.e. 95% confidence interval)

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

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
fit_preference_summary(x1mean, x2var, m1, x2mean, x2var, m2, y1mean, y1var,
               n1, y2mean, y2var, n2)

# Stratified

x1mean <- c(5, 3)
x1var <- c(1, 1)
m1 <- c(15, 30)
x2mean <- c(7, 7)
x2var <- c(1.1, 3.1)
m2 <- c(35, 40)
y1mean <- c(6, 4)
y1var <- c(1, 2)
n1 <- c(25, 35)
y2mean <- c(8, 12)
y2var <- c(1.2, 1)
n2 <- c(25, 20)
fit_preference_summary(x1mean, x2var, m1, x2mean, x2var, m2, y1mean, y1var,
                       n1, y2mean, y2var, n2, alpha=0.1)
```

imap_stratified_summary

Data from the the IMAP study

Description

The "Improving Management of Abnormal Pap Smears" study 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.

Three data sets are provided with the preference package based on the IMAP study. The first, `imap_summary` provides summary statistics of the entire trial. The second `imap_summary_stratified`, summary statistics of the study per strata. The third `imap` is a resampled version of the individual level data including stratification. Each of these data sets are compatible with the analysis functions `fit_preference_summary`, `fit_preference`, and `preference`, provided in this package. The examples sections in the documentation illustrate their use.

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)

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, k = 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).
k	the ratio of treatment A to treatment B in the random arm (default 1).

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)

---

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, k = 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).
k	the ratio of treatment A to treatment B in the random arm. (default 1, i.e. equal distribution to the two treatments in the random arm)

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)

`preference` *Fit Preference Data Collected from a Two-stage Clinical Trial*

Description

The variables in the formula should reference columns in the data parameter and should have the following characteristics.

- outcome: Numeric values giving the outcome of interest.
- treatment: Character, categorical, or integer values denoting the treatment received by an individual.
- arm: Logical value indicating whether the sample was from the random arm (TRUE) or choice (FALSE).
- strata: An optional integer value denoting which strata individuals belong to.

Usage

```
preference(form, data, alpha = 0.05)
```

Arguments

form	a formula of the form outcome ~ treatment:arm {| strata}. See Details for more explanation.
data	a data.frame containing variables specified in the formula
alpha	(optional numeric) Level of significance (default 0.05)

Examples

```
# Unstratified

outcome <- c(10, 8, 6, 10, 5, 8, 7, 6, 10, 12, 11, 6, 8, 10, 5, 7, 9, 12, 6,
             8, 9, 10, 7, 8, 11)
arm <- c(rep("choice", 13), rep("random", 12))
treatment <- c(rep(1, 5), rep(2, 8), rep(1, 6), rep(2, 6))
d <- data.frame(outcome=outcome, treatment=treatment, arm=arm)
preference(outcome ~ treatment:arm, d)

# Stratified
outcome <- c(10, 8, 6, 10, 5, 8, 7, 6, 10, 12, 11, 6, 8, 10, 5, 7, 9, 12, 6,
             8, 9, 10, 7, 8,11)
random <- c(rep(FALSE, 13), rep(TRUE, 12))
treatment <- c(rep(1, 5), rep(2, 8), rep(1, 6), rep(2, 6))
strata <- c(1,1,2,2,2,1,1,1,1,2,2,2,2,1,1,1,2,2,2,1,1,1,2,2,2)
d <- data.frame(outcome=outcome, treatment=treatment, arm=arm, strata=strata)
preference(outcome ~ treatment:arm|strata, d, alpha=0.1)
```

---

preference.trial *Create a Preference Trial*

Description

Create a Preference Trial

Usage

```
preference.trial(pref_ss, pref_effect, selection_ss, selection_effect,
  treatment_ss, treatment_effect, sigma2, pref_prop, choice_prop = 0.5,
  stratum_prop = 1, alpha = 0.05, k = 1)
```

Arguments

pref_ss	the sample size of the preference arm.
pref_effect	the effect size of the preference arm (delta_pi).
selection_ss	the sample size of the selection arm.

`selection_effect`
: the effect size of selection arm (delta_nu).

`treatment_ss`
: the sample size of the treatment arm .

`treatment_effect`
: the sample size of the treatment arm (delta_tau)

`sigma2`
: the variance estimate of the outcome of interest. This value 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.

`pref_prop`
: the proportion of patients preferring treatment 1. This value should be between 0 and 1 (phi).

`choice_prop`
: the proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5) (theta).

`stratum_prop`
: 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) (xi).

`alpha`
: the desired type I error rate (default 0.05).

`k`
: the ratio of treatment A to treatment B in the random arm (default 1)..

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 single trial.
preference.trial(pref_ss=100, pref_effect=1, selection_ss=100,
  selection_effect=1, treatment_ss=100, treatment_effect=1,
  sigma2=1, pref_prop=0.6)

# Stratified single trial.
preference.trial(pref_ss=100, pref_effect=1, selection_ss=100,
  selection_effect=1, treatment_ss=100, treatment_effect=1,
  sigma2=list(c(1, 0.8)), pref_prop=list(c(0.6, 0.3)),
  choice_prop=0.5, stratum_prop=list(c(0.3, 0.7)))

# Multiple trials unstratified.
preference.trial(pref_ss=100, pref_effect=seq(0.1, 2, by=0.5),
  selection_ss=100, selection_effect=1, treatment_ss=100,
  treatment_effect=1, sigma2=1, pref_prop=0.6)

# Multiple, stratified trials.
preference.trial(pref_ss=100, pref_effect=seq(0.1, 2, by=0.5),
```

```
selection_ss=100, selection_effect=1, treatment_ss=100,
treatment_effect=1, sigma2=list(c(1, 0.8)), pref_prop=list(c(0.6, 0.3)),
choice_prop=0.5, stratum_prop=list(c(0.3, 0.7)))
```

`pt_from_power` *Design Preference Trials with Power Constraint(s)*

Description

Create a set of preference trials with specified power. The power parameter guarantees that the power will be at least what is specified for each of the three arms.

Usage

```
pt_from_power(power, pref_effect, selection_effect, treatment_effect, sigma2,
  pref_prop, choice_prop = 0.5, stratum_prop = 1, alpha = 0.05, k = 1)
```

Arguments

`power`	the desired power(s) for the trial(s)
`pref_effect`	the effect size of the preference arm (delta_pi).
`selection_effect`	the effect size of selection arm (delta_nu).
`treatment_effect`	the sample size of the treatment arm (delta_tau)
`sigma2`	the variance estimate of the outcome of interest. This value 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.
`pref_prop`	the proportion of patients preferring treatment 1. This value should be between 0 and 1 (phi).
`choice_prop`	the proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5) (theta).
`stratum_prop`	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) (xi).
`alpha`	the desired type I error rate (default 0.05).
`k`	the ratio of treatment A to treatment B in the random arm (default 1)..

Examples

```
# Unstratified trials with power constraints.
pt_from_power(power=seq(.1, 0.8, by=0.1), pref_effect=1, selection_effect=1,
  treatment_effect=1, sigma2=1, pref_prop=0.6)

# Stratified trials with power constraints. Note that the proportion
# of patients in the choice arm (choice prop) is fixed for all strata.
pt_from_power(power=seq(0.1, 0.8, by=0.1), pref_effect=1,
  selection_effect=1, treatment_effect=1,
  sigma2=list(c(1, 0.8)), pref_prop=list(c(0.6, 0.3)),
  choice_prop=0.5, stratum_prop=list(c(0.3, 0.7)))

# or...

pt_from_power(power=seq(0.1, 0.8, by=0.1), pref_effect=1,
  selection_effect=1, treatment_effect=1,
  sigma2=c(1, 0.8), pref_prop=c(0.6, 0.3),
  choice_prop=0.5, stratum_prop=c(0.3, 0.7))
```

---

pt_from_ss *Design Preference Trials with Sample Size Constraint(s)*

Description

Create a set of preference trials where the maximum sample size for an arm is specified.

Usage

```
pt_from_ss(ss, pref_effect, selection_effect, treatment_effect, sigma2,
  pref_prop, choice_prop = 0.5, stratum_prop = 1, alpha = 0.05, k = 1)
```

Arguments

ss	the maximum size of any of the three arms.
pref_effect	the effect size of the preference arm (delta_pi).
selection_effect	the effect size of selection arm (delta_nu).
treatment_effect	the sample size of the treatment arm (delta_tau)
sigma2	the variance estimate of the outcome of interest. This value 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.
pref_prop	the proportion of patients preferring treatment 1. This value should be between 0 and 1 (phi).

choice_prop	the proportion of patients assigned to choice arm in the initial randomization. Should be numeric value between 0 and 1 (default=0.5) (theta).
stratum_prop	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) (xi).
alpha	the desired type I error rate (default 0.05)..
k	the ratio of treatment A to treatment B in the random arm (default 1).

Examples

```
# Unstratified trials with power constraints.
pt_from_ss(ss=seq(100, 1000, by=100), pref_effect=1,
  selection_effect=1, treatment_effect=1, sigma2=1, pref_prop=0.6)

# Stratified trials with power constraints. Note that the proportion
# of patients in the choice arm (choice prop) is fixed for all strata.
pt_from_ss(ss=seq(100, 1000, by=100), pref_effect=1,
  selection_effect=1, treatment_effect=1,
  sigma2=list(c(1, 0.8)), pref_prop=list(c(0.6, 0.3)),
  choice_prop=0.5, stratum_prop=list(c(0.3, 0.7)))

# or...

pt_from_ss(ss=seq(100, 1000, by=100), pref_effect=1,
  selection_effect=1, treatment_effect=1,
  sigma2=c(1, 0.8), pref_prop=c(0.6, 0.3),
  choice_prop=0.5, stratum_prop=c(0.3, 0.7))
```

---

pt_plot *Plot the effect sizes of a preference trial*

Description

The pt_plot() function visualizes the change in the preference effect, the selection effect, or both as a function of the total sample size of the trial. If the preference effect varies but the selection effect does not, then it plots the preference effect by the total sample size. Similarly if the selection effect varies but not the preference effect then selection effect vs total sample size is shown. When both preference and selection effect vary then the selection effect is shown conditioned on the given preference effects.

It is assumed that the set of trial provided as a parameter are related and are comparable. For example, the function does not check to if the strata are the same for all trials. If some other visualization is required then the user is reminded that a preference.trial object is a data frame and can be visualized in the usual way.

Usage

```
pt_plot(pt)
```

Arguments

pt	an object of class preference.trial.

Examples

```
# Plot trials with fixed power and varying preference effect.
trials <- pt_from_power(power = 0.8, pref_effect = seq(0.5, 2, by = 0.1),
                        selection_effect = 1, treatment_effect = 1,
                        sigma2 = 1, pref_prop = 0.6)
pt_plot(trials)

# Plot trials with fixed power and varying selection effect.
trials <- pt_from_power(power = 0.8, pref_effect = 1,
                        selection_effect = seq(0.5, 2, by = 0.1),
                        treatment_effect = 1, sigma2 = 1, pref_prop = 0.6)
pt_plot(trials)

# Plot trials with fixed power and varying preference and
# selection effects.

# the selection effects of interest
selection_effects <- rep(seq(0.5, 2, by = 0.1), 4)

# the preference effects to condition on
pref_effects <- rep(seq(0.4, 1, by = 0.2),
                    each = length(selection_effects)/4)

trials <- pt_from_power(power = 0.8, pref_effect = pref_effects,
                        selection_effect = selection_effects,
                        treatment_effect = 1, sigma2 = 1, pref_prop = 0.6)
pt_plot(trials)
```

---

sample_size *Preference trial parameter accessors*

Description

Accessor function have been created to get the sample size (sample_size), power (power), effect size (effect_size), arm proportion (proportion), significance (significance), and trial variance estimates (sigma2) for a set of preference trials.

Note that these methods are preferred over accessing the underlying data frame directly since the structure is slightly non-standard (some columns are lists) and some values, like power, are not stored directly.

Usage

```
sample_size(x)

## S3 method for class 'preference.trial'
sample_size(x)

power(x)

## S3 method for class 'preference.trial'
power(x)

effect_size(x)

## S3 method for class 'preference.trial'
effect_size(x)

proportion(x)

## S3 method for class 'preference.trial'
proportion(x)

significance(x)

## S3 method for class 'preference.trial'
significance(x)

sigma2(x)

## S3 method for class 'preference.trial'
sigma2(x)
```

Arguments

x	the set of preference trials.

Examples

```
# Create a set of trials with a sequence of preference effects.
trials <- preference.trial(pref_ss=100, pref_effect=seq(0.1, 2, by=0.5),
                           selection_ss=100, selection_effect=1,
                           treatment_ss=100, treatment_effect=1, sigma2=1,
                           pref_prop=0.6)

# the sample sizes
sample_size(trials)

# the powers
power(trials)
```

```
# the effect sizes
effect_size(trials)

# the arm proportions
proportion(trials)

# the significance
significance(trials)

# the variance estimates
sigma2(trials)
```

`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)
```

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