Package ‘preference’

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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 <doi:10.18637/jss.v094.c02>.
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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 preference computes the test statistic and p-value for each effect given provided raw study data. The function fit_preference_summary uses provided summary data (mean, variance,
and sample size) of each study group to compute the test statistic and p-value of each effect. The test statistics can be accessed from the models using the `summary()` function.

Preference Trial Function Calls:

- preference.trial: construct a `preference.trial` based on effect and sample sizes.
- pt_from_power: construct a `preference.trial` based on power and effect size.
- pt_from_ss: construct a `preference.trial` based on sample size

Analysis Function Calls

- preference and fit_preference: computes test statistic and p-value for observed # selection, preference, and treatment effects using provided raw data
- fit_preference_summary: 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


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**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.
fit_preference

References


Examples

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

---

**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, arm, treatment, strata, alpha = 0.05)

**Arguments**

- `outcome` (numeric) individual trial outcomes.
- `arm` (character or factor) a vector of "choice" and "random" character or factor values indicating the arm of the sample?
- `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, 11, 6, 8, 10, 5, 7, 9, 12, 6, 8, 9, 10, 7, 8,11)
arm <- c(rep("choice", 10), rep("random", 12))
treatment <- c(rep(1, 5), rep(2, 8), rep(1, 6), rep(2, 6))
fit_preference(outcome, arm, treatment)

# Stratified
# Same data plus strata information.
strata <- c(1,1,2,2,1,1,1,2,2,2,1,1,1,2,2,2,1,1,1,2,2,2)
fit_preference(outcome, arm, 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

```r
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


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 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


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


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)
Description

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

Usage

```r
overall_power_binom(
  N,
  phi,
  p11,
  p22,
  p1,
  p2,
  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.
- **p11**: response proportion of patients choosing to receive treatment 1 in the choice arm. 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.
- **p22**: response proportion of patients choosing to receive treatment 2 in the choice arm. 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.
- **p1**: response proportion of patients randomized to receive treatment 1 in the random arm. 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.
- **p2**: response proportion of patients randomized to receive treatment 2 in the random arm. 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.
- **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).
overall_sample_size_bin

Description
Calculates the sample size required to detect a given set of effects in a two-stage randomize clinical trial with a binary outcome. Returns the sample size for each of the three effects: preference, selection and treatment.

Usage

overall_sample_size_bin(
  power,
  phi,
  p11,
  p22,
  p1,
  p2,
  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.
p11 response proportion of patients choosing to receive treatment 1 in the choice arm. 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.
p22 response proportion of patients choosing to receive treatment 2 in the choice arm. 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.

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).
overall_sample_size_pois

**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**

```r
overall_sample_size_pois(
  power,
  phi,
  lambda11,
  lambda22,
  lambda1,
  lambda2,
  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.
- **lambda11**: response mean of patients choosing to receive treatment 1 in the choice arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.
- **lambda22**: response mean of patients choosing to receive treatment 2 in the choice arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.
- **lambda1**: response mean of patients randomized to receive treatment 1 in the random arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.
- **lambda2**: response mean of patients randomized to receive treatment 2 in the random arm. Should be numeric value larger than 0. If study is stratified, should be vector 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

```r
# Unstratified
overall_sample_size_pois(power = 0.8, phi = 0.6, lambda11 = 0.6, lambda22 = 0.7, lambda1 = 0.4, lambda2 = 0.6)

# Stratified
overall_sample_size_pois(power = 0.8, phi = c(0.6, 0.5), lambda11 = c(0.6, 0.7), lambda22 = c(0.7,0.7), lambda1 = c(0.4, 0.6), lambda2 = c(0.6, 0.6), xi = c(0.5, 0.5), nstrata = 2)
```

---

**plot.preference.trial**  
*Plot the effect sizes of a preference trial*
Description

The 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

```r
## S3 method for class 'preference.trial'
plot(x, ...)
```

Arguments

- `x`: an object of class preference.trial.
- `...`: any other parameters (this is currently not used).

Examples

```r
# 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)
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)
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)
```
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.
- `random`: 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

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

Arguments

- `form`: a formula of the form `outcome ~ treatment:arm [1 strata].`
- `data`: a data.frame containing variables specified in the formula. It should be noted that the arm values must be either "choice" or "random".
- `alpha` (optional numeric) Level of significance (default 0.05)

Examples

```r
# 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
random <- c(rep(FALSE, 13), rep(TRUE, 12))
treatment <- c(rep(1, 5), rep(2, 8), rep(1, 6), rep(2, 6))
strata <- c(1,1,2,2,2,1,1,1,2,2,2,1,1,2,2,1,1,2,2,2)
d <- data.frame(outcome=outcome, treatment=treatment, arm=arm,
                strata=strata)
preference(outcome ~ treatment:arm|strata, d, alpha=0.1)
```
Create a Preference Trial

**Description**
Create a Preference Trial

**Usage**
```r
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


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

```r
pt_from_power(
  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 = 0.5,  # 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 = 1,  # 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 = 0.05,  # the desired type I error rate (default 0.05).
  k = 1  # the ratio of treatment A to treatment B in the random arm (default 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

```r
# 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=1, pref_prop=0.6, choice_prop=0.5)
```
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

```r
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))

---

**pwr_overall_pois**  
**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**

```r
pwr_overall_pois(
    N,
    phi,
    lambda11,
    lambda22,
    lambda1,
    lambda2,
)```
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.

lambda11 response mean of patients choosing to receive treatment 1 in the choice arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.

lambda22 response mean of patients choosing to receive treatment 2 in the choice arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.

lambda1 response mean of patients randomized to receive treatment 1 in the random arm. Should be numeric value larger than 0. If study is stratified, should be vector with length equal to the number of strata in the study.

lambda2 response mean of patients randomized to receive treatment 2 in the random arm. Should be numeric value larger than 0. If study is stratified, should be vector 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

# Unstratified
pwr_overall_pois(N=400, phi=0.5, lambda11=0.8, lambda22=0.5, lambda1=0.5, lambda2=0.4)

# Stratified
pwr_overall_pois(N=400, phi=c(0.5,0.5), lambda11=c(0.7,0.8), lambda22=c(0.4,0.4),
lambda1=c(0.5,0.4), lambda2=c(0.2,0.3), xi=c(0.3,0.7), nstrata=2)
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

```r
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

```r
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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