Package 'BayesDesign'

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Type Package

Title Bayesian Single-Arm Design with Survival Endpoints

Version 0.1.1

Description The proposed event-driven approach for Bayesian two-stage single-arm phase II trial design is a novel clinical trial design and can be regarded as an extension of the Simon's two-stage design with the time-to-event endpoint. This design is motivated by cancer clinical trials with immunotherapy and molecularly targeted therapy, in which time-to-event endpoint is often a desired endpoint.

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NeedsCompilation no

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Repository CRAN

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`optimal_OneStage` *Obtain design settings for one-stage Bayesian Single-Arm Phase II Trial with Time-to-Event Endpoints*

Description

Obtain design parameters, type I error, power and operating characteristics of the Bayesian Single-Arm Phase II Trial Designs with Time-to-Event Endpoints (Wu et al. 2021). The exponential distribution is assumed for the survival time. The gamma prior is used here

Usage

```
optimal_OneStage(alphacutoff, powercutoff, S0,
                 x, ta, tf, a = 2, delta, ntrial,
                 complete = "partial", seed = 8232)
```

Arguments

`alphacutoff`	the desired type I error to be controlled
`powercutoff`	the desired power to be achieved
`S0`	the survival probability at timepoint x
`x`	the survival probability S0 at timepoint x
`ta`	accrual duration
`tf`	follow-up duration
`a`	shape parameter of prior distribution. The default value is a = 2
`delta`	hazard ratio
`ntrial`	the number of simulated trials
`complete`	whether output the full or partial information. The default value is complete = "partial". If want to show full results, it would be complete = "complete"
`seed`	the seed. The default value is seed = 8232

Value

`optimal_OneStage()` depending on the argument "complete", it returns a vector of partial information/complete information which includes:

partial information: (1) m: number of events of the whole design (2) n: number of patients of the whole design (3) k: total observation time of the whole design (4) typeI: type I error of the whole design (5) power: power of the whole design (6) ES1: expected sample size under alternative hypothesis (7) ES0: expected sample size under null hypothesis

full information: (1) eta: cutoff point of "Go" at final stage of analysis (2) zeta: cutoff point of "no-Go" at final stage of analysis (3) m: number of events of the whole design (4) n: number of patients of the whole design (5) k: total observation time of the whole design (6) typeI: type I error of the whole design (7) power: power of the whole design (8) ES1: expected sample size under alternative hypothesis (9) ES0: expected sample size under null hypothesis

Author(s)

Chia-Wei Hsu, Haitao Pan, Jianrong Wu

References

Jianrong Wu, Haitao Pan, Chia-Wei Hsu (2021). "Bayesian Single-Arm Phase II Trial Designs with Time-to-Event Endpoints." Pharmaceutical Statistics. Accepted

Examples

```
### Design 1

# H0 vs. H1: 17% vs. 40% (4-month PFS)
# that is, S0 = 0.17, and hazard ratio, e.g., delta = 0.517
# x = 4

optimal_OneStage(alphacutoff = 0.1, powercutoff = 0.8,
                 S0 = 0.17, x = 4, ta = 6, tf = 6,
                 delta = 0.517, ntrial = 10)

### Design 2

# H0 vs. H1: 17% vs. 30% (4-month PFS)
# that is, S0 = 0.17, and hazard ratio, e.g., delta = 0.679
# x = 4

optimal_OneStage(alphacutoff = 0.1, powercutoff = 0.8,
                 S0 = 0.17, x = 4, ta = 6, tf = 6,
                 delta = 0.679, ntrial = 10)
```

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optimal_TwoStage *Obtain design settings for two-stage Bayesian Single-Arm Phase II Trial with Time-to-Event Endpoints*

Description

Obtain design parameters, type I error, power and operating characteristics of the Bayesian Single-Arm Phase II Trial Designs with Time-to-Event Endpoints (Wu et al. 2021). The exponential distribution is assumed for the survival time. The gamma prior is used here

Usage

```
optimal_TwoStage(alphacutoff, powercutoff, S0, x,
                 ta, tf, a = 2, delta, frac = .5,
                 ntrial, complete = "partial", seed = 8232)
```

Arguments

alphacutoff	the desired type I error to be controlled
powercutoff	the desired power to be achieved
S0	the survival probability at timepoint x
x	the survival probability S0 at timepoint x
ta	accrual duration
tf	follow-up duration
a	shape parameter of prior distribution. The default value is a = 2
delta	hazard ratio
frac	a information fraction for interim analysis. The fefault value is frac = 0.5
ntrial	the number of simulated trials
complete	whether output the full or partial information. The default value is complete = "partial". If want to show full results, it would be complete = "complete"
seed	the seed. The default value is seed = 8232

Value

`optimal()` depending on the argument "complete", it returns a vector of partial information/complete information which includes:

partial information: (1) m1: number of events at stage 1 (2) n1: number of patients at stage 1 (3) k1: total observation time at stage 1 (4) m: number of events of the whole design (5) n: number of patients of the whole design (6) k: total observation time of the whole design (7) typeI: type I error of the whole design (8) power: power of the whole design (9) PET1: early stopping probabilites under alternative hypothesis (10) ES1: expected sample size under alternative hypothesis (11) PET0: early stopping probabilites under null hypothesis (12) ES0: expected sample size under null hypothesis

full information: (1) eta: cutoff point of "Go" at final stage of analysis (2) xi: cutoff point of "no-Go" at final stage of analysis (3) m1: number of events at stage 1 (4) n1: number of patients at stage 1 (5) k1: total observation time at stage 1 (6) m: number of events of the whole design (7) n: number of patients of the whole design (8) k: total observation time of the whole design (9) typeI: type I error of the whole design (10) power: power of the whole design (11) PET1: early stopping probabilites under alternative hypothesis (12) ES1: expected sample size under alternative hypothesis (13) PET0: early stopping probabilites under null hypothesis (14) ES0: expected sample size under null hypothesis

Author(s)

Chia-Wei Hsu, Haitao Pan, Jianrong Wu

References

Jianrong Wu, Haitao Pan, Chia-Wei Hsu (2021). "Bayesian Single-Arm Phase II Trial Designs with Time-to-Event Endpoints." Pharmaceutical Statistics. Accepted

Examples

```
### Design 1

# H0 vs. H1: 17% vs. 40% (4-month PFS)
# that is, S0 = 0.17, and hazard ratio, e.g., delta = 0.517
# x = 4

optimal_TwoStage(alphacutoff = 0.1, powercutoff = 0.8, S0 = 0.17,
                 x = 4, ta = 6, tf = 6, delta = 0.517, ntrial = 10)

### Design 2

# H0 vs. H1: 17% vs. 30% (4-month PFS)
# that is, S0 = 0.17, and hazard ratio, e.g., delta = 0.679
# x = 4

optimal_TwoStage(alphacutoff = 0.1, powercutoff = 0.8, S0 = 0.17,
                 x = 4, ta = 6, tf = 6, delta = 0.679, ntrial = 10)
```

---

tot_time *Sum up transformed observation time for each patient*

Description

Sum up transformed observation time for each patient to get U in order to determine the trial: (1) goes to second stage (2) stops for futility (3) declares the treatment is promising and warrants for further study in a large scale phase III trial (4) declares the treatment is unpromising and is not worth for further study.

Usage

```
tot_time(obs_time, S0, x)
```

Arguments

obs_time	a vector. Each element represents an observation time of the patient
S0	the survival probability at timepoint x
x	the survival probability S0 at timepoint x

Value

the function returns the total transformed observation time for all patients

Author(s)

Chia-Wei Hsu, Haitao Pan, Jianrong Wu

References

Jianrong Wu, Haitao Pan, Chia-Wei Hsu (2021). "Bayesian Single-Arm Phase II Trial Designs with Time-to-Event Endpoints." Pharmaceutical Statistics. Accepted

Examples

```
obs_time <- c(3.003, 11.987, 4.306, 2.561, 1.575, 0.329, 1.940,
              0.869, 7.481, 1.861, 7.279, 0.007, 6.485, 1.981,
              4.257, 0.967, 2.619, 0.040, 0.426, 4.628)
S0 <- 0.17
x <- 4
tot_time(obs_time = obs_time, S0 = S0, x = x)
```

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