Package: BayesOrdDesign (via r-universe)

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Type Package

Title Bayesian Group Sequential Design for Ordinal Data

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Description The proposed group-sequential trial design is based on Bayesian methods for ordinal endpoints, including three methods, the proportional-odds-model (PO)-based, non-proportional-odds-model (NPO)-based, and PO/NPO switch-model-based designs, which makes our proposed methods generic to be able to deal with various scenarios. Richard J. Barker, William A. Link (2013) <doi:10.1080/00031305.2013.791644>. Thomas A. Murray, Ying Yuan, Peter F. Thall, Joan H. Elizondo, Wayne L.Hofstetter (2018) <doi:10.1111/biom.12842>. Chengxue Zhong, Haitao Pan, Hongyu Miao (2021) <arXiv:2108.06568>.

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`Bayes_ord` *Bayesian ordinal regression analysis Estimate the correlation coefficients of treatment variable, with and without the proportional odds assumption*

Description

Bayesian ordinal regression based on cumulative likelihood function Estimate the correlation coefficients of treatment variable, with or without the proportional odds assumption

Usage

```
Bayes_ord(formula, data, structure, U)
```

Arguments

`formula`	a formula expression as for regression models, of the form response ~ predictors. The response should be a factor (preferably an ordered factor), which will be interpreted as an ordinal response with levels ordered as in the factor.
`data`	a data frame in which to interpret the variables occurring in the formula.
`structure`	the data structure. i.e., structure = "PO" or structure = "NPO".
`U`	the desirability of each outcome level

Details

This function estimates the coefficients and threshold coefficients. Specifically, the numerical utilities U reflect the desirability of each outcome level. To do this, in our example, we first set U[1] = 100 and U[5] = 0, and then asked physicians to specify numerical values for the intermediate levels, that reflect their desirability relative to the best and worst levels.

Value

Bayes_ord() returns the regression coefficients, including: (1) estimator coefficients (2) thresholds coefficients

Examples

```
### Example One: PO data structure
fm1 = Bayes_ord(response~treatment, example.data, "PO")

### Example Two: NPO data structure
fm2 = Bayes_ord(response~treatment, example.data, "NPO", U = c(100,80,65,25,10,0))
```

`example.data` *Clinical ordinal endpoints and treatments assignment for 200 patient*

Description

A dataset containing the ordinal outcomes and corresponding groups.

Usage

```
example.data
```

Format

A data frame with 200 rows and 2 variables:

response outcome

treatment 0 denotes control, 1 denotes treatment ...

`get_oc_NPO` *Generate operating characteristics for Bayesian two-stage trial design of ordinal endpoints without proportional odds assumption*

Description

Obtain operating characteristics (OC) of the Bayesian two-stage trial design with ordinal endpoints while the proportional odds assumption are violated.

Usage

```
get_oc_NPO(alpha, pro_ctr, U, fixed_ss, ors, nmax, fixed_es, ntrial, method)
```

Arguments

alpha	the desired type I error to be controlled
pro_ctr	distribution of clinical categories for the control group
U	the desirability of each outcome level
fixed_ss	fixed sample size when simulates the OC for various effect size
ors	a user-defined matrix, each row denotes the various scenarios, the number of columns depend on the number of outcome scales.
nmax	the maximum sample size when simulates the OC for different sample size, the increment is 50 and the initial sample size is 50 for each arm each stage.
fixed_es	fixed effect size when simulate the OC for various sample size
ntrial	the number of simulated trials
method	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 400, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 50.

For the parameter estimation section, we have two options, and can be selected using the method argument.Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Specifically, the numerical utilities U reflect the desirability of each outcome level. To do this, in our example, we first set U[1] = 100 and U[5] = 0, and then asked physicians to specify numerical values for the intermediate levels, that reflect their desirability relative to the best and worst levels.

Function provides two types of operating characteristics via simulation. If user specifies the value of ors and fixed_ss, function will calculate the design's power in terms of effect size. If user specifies the value of nmax and fixed_es, function will calculate the design's power in terms of sample size, and nmax is the upper limitation of sample size for the treatment and control groups at each stage, the lower limitation is 50, the default increment of the sequence is 10.

Please note, in our example, argument ntrial = 5 is for the time saving purpose.

Value

get_oc_NPO() returns the operating characteristics of design as a table, including (1) user-defined value, either sample size or effect size (2) corresponding power (3) average sample size

Examples

```
ors = matrix(c(1.5,1.5,1,1,1,1.5,1.5,1.1,1.1,1.1), nrow=2, ncol=5, byrow=TRUE)

get_oc_NPO(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
           U = c(100,80,65,25,10,0), fixed_ss = 200, ors, ntrial = 5,
```

```
            method = "Frequentist")

set.seed(123)
get_oc_NPO(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
           U = c(100,80,65,25,10,0), nmax = 100, fixed_es = c(1.5,1.3,1,1,1),
           ntrial = 5, method = "Frequentist")
```

`get_oc_PO` *Generate operating characteristics for Bayesian two-stage trial design of ordinal endpoints with proportional odds assumption*

Description

Obtain operating characteristics (OC) of the Bayesian two-stage trial design of ordinal endpoints with proportional odds assumption.

Usage

```
get_oc_PO(alpha, pro_ctr, nmax, fixed_es, ormax, fixed_ss, ntrial, method)
```

Arguments

`alpha`	the desirable type I error rate to be controlled
`pro_ctr`	distribution of clinical categories for the control group
`nmax`	the maximum sample size for operating characteristics
`fixed_es`	fixed effect size when simulate the OC for various sample size
`ormax`	the maximum effect size for OC
`fixed_ss`	fixed sample size when simulate the OC for various effect size
`ntrial`	the number of simulated trials
`method`	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 200, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 10.

For the parameter estimation section, we have two options, and can be selected using the method argument.Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Two types of operating characteristics can be implemented through this function.

Please note, in our example, argument ntrial = 5 is for the time saving purpose.

Value

get_oc_PO() returns the operating characteristics of design as a table, including: (1) user-defined value, either sample size or effect size (2) corresponding power (3) average sample size

Examples

```
get_oc_PO(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
          ormax = 1.5, fixed_ss = 150,
          ntrial = 5, method = "Frequentist")


get_oc_PO(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
          nmax = 200, fixed_es = 1.5,
          ntrial = 5, method = "Frequentist")
```

---

get_oc_Switch *Generate operating characteristics for Bayesian two-stage trial design of ordinal endpoints without proportional odds assumption.*

Description

Obtain operating characteristics (OC) of the Bayesian two-stage trial design with ordinal endpoints while the proportional odds assumption are violated.

Usage

```
get_oc_Switch(
  alpha,
  pro_ctr,
  U,
  ors,
  n_range,
  fixed_es,
  n_po,
  n_npo,
  ntrial,
  method
)
```

Arguments

alpha	the desired type I error to be controlled.
pro_ctr	distribution of clinical categories for the control group.
U	the desirability of each outcome level.
ors	a user-defined matrix, each row denotes the various scenarios, the number of columns depend on the number of outcome scales.

n_range	the additional sample size for each arm each stage after n_po, n_npo.
fixed_es	fixed effect size when simulate the OC for various sample size.
n_po	sample size for the treatment and control groups, at each stage based on PO model.
n_npo	sample size for the treatment and control groups, at each stage based on NPO model.
ntrial	the number of simulated trials.
method	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach.

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 200, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 10.

For the parameter estimation section, we have two options, and can be selected using the method argument. Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Specifically, the numerical utilities U reflect the desirability of each outcome level. To do this, in our example, we first set U[1] = 100 and U[5] = 0, and then asked physicians to specify numerical values for the intermediate levels, that reflect their desirability relative to the best and worst levels.

Function provides two types of operating characteristics via simulation. If user specifies the value of ors and fixed_ss, function will calculate the design's power in terms of effect size. If user specifies the value of n_range and fixed_es, function will calculate the design's power in terms of sample size, and n_range is the upper limitation of sample size for the treatment and control groups at each stage, the lower limitation is 50, the default increment of the sequence is 10.

Arguments n_po and n_npo are the estimated sample size for the treatment and control groups at each stage based on PO model and NPO model respectively. Users can obtained them through function ss_po and ss_npo.

Value

get_oc_NPO() returns the operating characteristics of design as a table, including (1) user-defined value, either sample size or effect size (2) corresponding power (3) average sample size

Examples

```
get_oc_Switch(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
              U = c(100,80,65,25,10,0), n_range = 10, fixed_es = c(1.5,1.5,1,1,1),
              n_po = 475,n_npo = 75, ntrial = 5, method = "Frequentist")


or2 = matrix(rep(seq(1,1.3, by=0.1), times=1, each=3),ncol = 3,byrow = TRUE)
```

```
or1 = matrix(rep(1.5, dim(or2)[1]*2), ncol = 2, byrow = TRUE)
ors = cbind(or1, or2)

get_oc_Switch(alpha = 0.05, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
              U = c(100,80,65,25,10,0), ors, n_po = 475, n_npo = 75,
              ntrial = 5, method = "Frequentist")
```

`rjmcmc_func` *Perform reversible-jump MCMC post-process to select appropriate model between proportional odds (PO) model and non-proportional odds (NPO) model*

Description

Performs Bayesian multi-model inference, estimating posterior model probabilities for 2 candidate models.

Usage

```
rjmcmc_func(g1, ginv1, g2, ginv2, or_alt, sd, pro_ctr, n, U)
```

Arguments

`g1`	specify the bi-jections from the universal parameter psi to PO model parameter set
`ginv1`	specify the bi-jections from the PO model parameter set to psi. It is the inverse transformation of g1.
`g2`	specify the bi-jections from the universal parameter psi to NPO model parameter set
`ginv2`	specify the bi-jections from the NPO model parameter set to psi. It is the inverse transformation of g2.
`or_alt`	effect size to be detected (under H_1) in terms of odds ratio
`sd`	the standard error
`pro_ctr`	distribution of clinical categories for the control group
`n`	sample size for each group and each interim look
`U`	the desirability of each outcome level

Value

rjmcmc_func() returns the selection probabilities for PO and NPO model

Examples

```
g1 = function(psi){
  w = sum(psi[6:10])/5
  theta = c(psi[1], psi[2], psi[3], psi[4], psi[5],
            w, w-psi[7], w-psi[8], w-psi[9], w-psi[10])
  return(theta)
}

ginv1 = function(theta){

  w = sum(theta[6:10])
  psi = c(theta[1], theta[2], theta[3], theta[4], theta[5],
          w, theta[6]-theta[7], theta[6]-theta[8],
          theta[6]-theta[9], theta[6]-theta[10])
  return(psi)
}


g2 = function(psi){
  theta = psi
  return(theta)
}
ginv2 = function(theta){
  psi = theta
  return(psi)
}

out = rjmcmc_func(g1, ginv1, g2, ginv2, or_alt = c(1.4,1.4,1.4,1.4,1.4), sd = 0.2,
                  pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
                  n = 100, U = c(100,80,65,25,10,0))
```

---

ss_npo *Determine the sample size for Bayesian two-stage trial design of ordinal endpoints without proportional odds assumption*

Description

Obtain estimated sample size based on user-specified type I error, power and effect size defined by the odds ratio between the treatment and control groups, without the proportional odds (PO) assumption.

Usage

```
ss_npo(nmax, or_alt, pro_ctr, U, alpha, power, ntrial, method)
```

Arguments

nmax	the maximum sample size for searching to get the desirable power
or_alt	effect size to be detected (under H_1) in terms of odds ratio
pro_ctr	distribution of clinical categories for the control group
U	the desirability of each outcome level
alpha	the desirable type I error rate to be controlled
power	the desirable power to be achieved
ntrial	the number of simulated trials
method	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 200, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 50.

For the parameter estimation section, we have two options, and can be selected using the method argument.Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Specifically, the numerical utilities U reflect the desirability of each outcome level. To do this, in our example, we first set U[1] = 100 and U[5] = 0, and then asked physicians to specify numerical values for the intermediate levels, that reflect their desirability relative to the best and worst levels.

Please note, in our example, argument ntrial = 5 is for the time saving purpose.

Value

ss_npo() returns recommended sample size for each of two groups for the interim and final stages, by assuming 1:1 equal randomization for the two groups at each stage; and corresponding power.

Examples

```
set.seed(123)
ss_npo(nmax = 200, or_alt = c(1.6,1.5,1.5,1.4,1.4),
       pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13), U = c(100,80,65,25,10,0),
       alpha = 0.05, power = 0.8, ntrial = 5, method = "Frequentist")
```

ss_po *Determine the sample size for Bayesian two-stage trial design of ordinal endpoints with proportional odds assumption*

Description

Obtain estimated sample size based on user-specified type I error, power and effect size defined by the odds ratio between the treatment and control groups, under the proportional odds (PO) assumption.

Usage

```
ss_po(or_alt, pro_ctr, alpha, power, nmax, ntrial, method)
```

Arguments

or_alt	effect size to be detected (under H_1) in terms of odds ratio
pro_ctr	distribution of clinical categories for the control group
alpha	the desirable type I error rate to be controlled
power	the desirable power to be achieved
nmax	the maximum sample size for searching to get the desirable power
ntrial	the number of simulated trials
method	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 200, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 50.

For the parameter estimation section, we have two options, and can be selected using the method argument.Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Please note, in our example, argument ntrial = 5 is for the time saving purpose.

Value

ss_po() returns recommended sample size for each of two groups for the interim and final stages, by assuming 1:1 equal randomization for the two groups at each stage; and the corresponding power.

Examples

```
ss_po(or_alt = 1.5, pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13), alpha = 0.05,
      power = 0.8, nmax = 100, ntrial = 5, method ="Frequentist")
```

`ss_switch` *Determine the sample size for Bayesian two-stage trial design for ordinal endpoints based on switch model*

Description

When there lacks of sufficient information to determine which of these two models (PO or NPO) is more appropriate, PO/NPO switch model-based design is utilized to obtain estimated sample size based on user specified type I error, power and expected effect.

Usage

```
ss_switch(
  alpha,
  power,
  n_po,
  n_npo,
  or_alt,
  pro_ctr,
  U,
  ntrial,
  method,
  n_range
)
```

Arguments

`alpha`	the desirable type I error rate to be controlled
`power`	the desirable power to be achieved
`n_po`	sample size for the treatment and control groups, at each stage based on PO model
`n_npo`	sample size for the treatment and control groups, at each stage based on NPO model
`or_alt`	expected treatment efficacy effect size to be detected (under H_1) in terms of odds ratio
`pro_ctr`	distribution of clinical categories for the control group
`U`	the desirability of each outcome level
`ntrial`	the number of simulated trials
`method`	whether the statistical test for interim/final analysis is Bayesian or Frequentist. method = "Frequentist" for Frequentist approach; method = "Bayesian" for Bayesian approach
`n_range`	the additional sample size for each arm each stage after n_po, n_npo.

Details

Grid search of sample size is used for guarantee a desirable type I error rate. The upper limitation is 200, and lower limitation default is sample size 50 for the control and treatment groups at each stage. Default increment of the sequence is 10.

For the parameter estimation section, we have two options, and can be selected using the method argument.Two following options are available: (i) method = "Frequentist", (ii) method = "Bayesian". If method = "Frequentist", parameters are estimated via package ordinal, which is based on frequentist method, while method = "Bayesian", parameters are estimated through Bayesian model.

Specifically, the numerical utilities U reflect the desirability of each outcome level. To do this, in our example, we first set U[1] = 100 and U[5] = 0, and then asked physicians to specify numerical values for the intermediate levels, that reflect their desirability relative to the best and worst levels.

Arguments n_po and n_npo are the estimated sample size for the treatment and control groups at each stage based on PO model and NPO model respectively. Users can obtained them through function ss_po and ss_npo.

Value

ss_switch() returns recommended sample size for each group at every interim look, with assumption that the sample size in the control arm of the study is same as in the treatment arm, and the sample size at each interim look is same.

Examples

```
ss_switch(alpha = 0.05, power=0.8, n_po = 475, n_npo = 75, n_range = 10,
          or_alt = c(1.5,1.5,1.5,1.5,1.5), pro_ctr = c(0.58,0.05,0.17,0.03,0.04,0.13),
          U = c(100,80,65,25,10,0), ntrial = 5, method = "Frequentist")
```

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