# Package: basksim (via r-universe)

October 10, 2024

```
Type Package
Title Simulation-Based Calculation of Basket Trial Operating
     Characteristics
Version 1.0.0
Description Provides a unified syntax for the simulation-based
     comparison of different single-stage basket trial designs with
     a binary endpoint and equal sample sizes in all baskets.
     Methods include the designs by Baumann et al. (2024)
     <doi:10.48550/arXiv.2309.06988>, Fujikawa et al. (2020)
     <doi:10.1002/bimj.201800404>, Berry et al. (2020)
     <doi:10.1177/1740774513497539>, Neuenschwander et al. (2016)
     <doi:10.1002/pst.1730> and Psioda et al. (2021)
     <doi:10.1093/biostatistics/kxz014>. For the latter three
     designs, the functions are mostly wrappers for functions
     provided by the packages 'bhmbasket' and 'bmabasket'.
License GPL (>= 3)
Encoding UTF-8
RoxygenNote 7.2.3
Imports arrangements, bhmbasket, bmabasket, doFuture, extraDistr,
     foreach, HDInterval, progressr
Suggests covr, testthat (>= 3.0.0)
Config/testthat/edition 3
URL https://github.com/lbau7/basksim
BugReports https://github.com/lbau7/basksim/issues
NeedsCompilation no
Author Lukas Baumann [aut, cre]
     (<https://orcid.org/0000-0001-7931-7470>), Lukas Sauer [ctb]
     (0000-0002-1340-9994)
Maintainer Lukas Baumann <br/> <br/> baumann@imbi.uni-heidelberg.de>
Repository CRAN
Date/Publication 2024-04-12 15:20:14 UTC
```

2 Contents

# **Contents**

Index

adjust_lambda	3
adjust_lambda.bhm	3
adjust_lambda.default	5
adjust_lambda.exnex	6
ecd	7
get_data	8
get_details	9
get_details.bhm	9
get_details.bma	10
get_details.cpp	11
get_details.cppglobal	12
get_details.exnex	13
get_details.fujikawa	15
<i>E</i> = <i>3 E</i>	16
get_details.mml	17
get_details.mmlglobal	18
6 -	19
get_results.bhm	20
8	21
get_results.cpp	22
<i>c</i> = 11 <i>c</i>	23
<i>u</i> –	24
<i>E</i> = <i>y</i>	25
get_results.jsdglobal	
<u> </u>	27
6 - 6	28
6 -	29
opt_design	
1-	31
1-	32
setup_cpp	
setup_cppglobal	
setup_exnex	
1-3	36
1-3-6	37
1-	38
setup_mmlglobal	39
toer	40
4	41

adjust\_lambda 3

adjust\_lambda

Adjust Lambda

# Description

Adjust Lambda

# Usage

```
adjust_lambda(design, ...)
```

### **Arguments**

design An object created with one of the setup functions.
... Further arguments.

#### **Details**

The default method for adjust\_lambda uses a combination of uniroot and grid search and calls toer in every iteration. For methods implemented in the bhmbasket package there are separate methods that are computationally more efficient.

# Value

A list containing the greatest estimated value for lambda with prec\_digits decimal places which controls the family wise error rate at level alpha (one-sided) and the estimated family wise error rate for the estimated lambda.

### **Examples**

```
design <- setup_cpp(k = 3, p0 = 0.2)
adjust_lambda(design = design, n = 20, alpha = 0.05,
   design_params = list(tune_a = 1, tune_b = 1), iter = 1000)</pre>
```

adjust\_lambda.bhm

Adjust Lambda for the BHM Design

#### **Description**

Adjust Lambda for the BHM Design

4 adjust\_lambda.bhm

### Usage

```
## S3 method for class 'bhm'
adjust_lambda(
  design,
  n,
  p1 = NULL,
  alpha = 0.05,
  design_params = list(),
  iter = 1000,
  n_mcmc = 10000,
  prec_digits = 3,
  data = NULL,
  ...
)
```

### **Arguments**

design An object created with one of the setup functions.

n The sample size per basket.

p1 Probabilities used for the simulation. If NULL then all probabilities are set to p0.

alpha The one-sided significance level.

design\_params A list of params that is specific to the class of design.

iter The number of iterations in the simulation. Is ignored if data is specified.

n\_mcmc Number of MCMC samples.

prec\_digits Number of decimal places that are considered when adjusting lambda.

data A data matrix with k column with the number of responses for each basket. Has

to be generated with get\_data. If data is used, then iter is ignored.

... Further arguments.

# Value

A list containing the greatest estimated value for lambda with prec\_digits decimal places which controls the family wise error rate at level alpha (one-sided) and the estimated family wise error rate for the estimated lambda.

```
design <- setup_bhm(k = 3, p0 = 0.2, p_target = 0.5)
adjust_lambda(design = design, n = 15,
  design_params = list(tau_scale = 1), iter = 100, n_mcmc = 5000)</pre>
```

adjust\_lambda.default 5

```
adjust_lambda.default Adjust Lambda
```

### **Description**

Adjust Lambda

# Usage

```
## Default S3 method:
adjust_lambda(
  design,
  n,
  p1 = NULL,
  alpha = 0.05,
  design_params = list(),
  iter = 1000,
  prec_digits = 3,
  data = NULL,
  ...
)
```

# **Arguments**

design	An object created with one of the setup functions.
n	The sample size per basket.
p1	Probabilities under the alternative hypothesis. If NULL then the type 1 error rate under the global null hypothesis is calculated.
alpha	The one-sided significance level.
design_params	A list of params that is specific to the class of design.
iter	The number of iterations in the simulation. Is ignored if data is specified.
prec_digits	Number of decimal places that are considered when adjusting lambda.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

# **Details**

It is recommended to use data and then use the same simulated data set for all further calculations. If data = NULL then new data is generated in each step of the algorithm, so lambda doesn't necessarily protect the family wise error rate for different simulated data due to Monte Carlo simulation error.

#### Value

A list containing the greatest estimated value for lambda with prec\_digits decimal places which controls the family wise error rate at level alpha (one-sided) and the estimated family wise error rate for the estimated lambda.

# **Examples**

```
# Example for a basket trial with Fujikawa's Design
design <- setup_fujikawa(k = 3, p0 = 0.2)
adjust_lambda(design = design, n = 20, alpha = 0.05,
   design_params = list(epsilon = 2, tau = 0), iter = 1000)</pre>
```

adjust\_lambda.exnex

Adjust Lambda for the EXNEX Design

# Description

Adjust Lambda for the EXNEX Design

### Usage

```
## S3 method for class 'exnex'
adjust_lambda(
  design,
  n,
  p1 = NULL,
  alpha = 0.05,
  design_params = list(),
  iter = 1000,
  n_mcmc = 10000,
  prec_digits = 3,
  data = NULL,
  ...
)
```

### **Arguments**

design An object created with one of the setup functions.

n The sample size per basket.

p1 Probabilities used for the simulation. If NULL then all probabilities are set to p0.

alpha The one-sided significance level.

design\_params A list of params that is specific to the class of design.

iter The number of iterations in the simulation. Is ignored if data is specified.

n\_mcmc Number of MCMC samples.

prec\_digits Number of decimal places that are considered when adjusting lambda.

7 ecd

A data matrix with k column with the number of responses for each basket. Has data

to be generated with get\_data. If data is used, then iter is ignored.

Further arguments.

#### Value

A list containing the greatest estimated value for lambda with prec\_digits decimal places which controls the family wise error rate at level alpha (one-sided) and the estimated family wise error rate for the estimated lambda.

# **Examples**

```
design <- setup_exnex(k = 3, p0 = 0.2)
adjust_lambda(design = design, n = 15,
 design_params = list(tau_scale = 1, w = 0.5), iter = 100, n_mcmc = 5000)
```

ecd

Calculate the Expected Number of Correct Decisions for a Basket Trial Design

#### **Description**

Calculate the Expected Number of Correct Decisions for a Basket Trial Design

#### Usage

```
ecd(
  design,
  n,
  p1,
  lambda,
  design_params = list(),
  iter = 1000,
  data = NULL,
)
```

# **Arguments**

design An object created with one of the setup functions.

The sample size per basket.

Probabilities used for the simulation. If NULL then all probabilities are set to p0. р1

lambda The posterior probability threshold.

design\_params A list of params that is specific to the class of design.

iter The number of iterations in the simulation. Is ignored if data is specified. data

A data matrix with k column with the number of responses for each basket. Has

to be generated with get\_data. If data is used, then iter is ignored.

Further arguments.

8 get\_data

# Value

A numeric value.

# **Examples**

```
# Example for a basket trial with Fujikawa's Design design <- setup_fujikawa(k = 3, p0 = 0.2) ecd(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, design_params = list(epsilon = 2, tau = 0), iter = 1000)
```

get\_data

Simulate Data Based on a Binomial Distribution

# **Description**

Simulate Data Based on a Binomial Distribution

### Usage

```
get_data(k, n, p, iter, type = c("matrix", "bhmbasket"))
```

# Arguments

k	The number of baskets.
n	The sample size per basket.
р	Probabilities used to simulate the data
iter	The number of iterations in the simulation. Is ignored if data is specified.
type	Type of output. Use bhmbasket for the BHM and EXNED design and matrix for everything else.

# **Details**

For type = "bhmbasket" this is simply a wraper for bhmbasket::simulateScenarios.

### Value

If type = "matrix" then a matrix is returned, if type = "bhmbasket" then an element with class scenario\_list.

```
get_data(k = 3, n = 20, p = c(0.2, 0.2, 0.5), iter = 1000)
```

get\_details 9

get\_details

Get Details of a Basket Trial Simulation

#### **Description**

Get Details of a Basket Trial Simulation

#### Usage

```
get_details(design, ...)
```

# **Arguments**

design An object created with one of the setup functions.
... Further arguments.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors of all baskets and the family-wise error rate. For some methods the mean limits of HDI intervals are also returned.

# Examples

```
# Example for a basket trial with Fujikawa's Design
design <- setup_fujikawa(k = 3, p0 = 0.2)
get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
    epsilon = 2, tau = 0, iter = 100)</pre>
```

get\_details.bhm

Get Details of a BHM Basket Trial Simulation

# Description

Get Details of a BHM Basket Trial Simulation

```
## S3 method for class 'bhm'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  tau_scale,
  iter = 1000,
```

10 get\_details.bma

```
n_mcmc = 10000,
data = NULL,
...
)
```

#### **Arguments**

design An object of class bhm. The sample size per basket. Probabilities used for the simulation. If NULL then all probabilities are set to p0. р1 lambda The posterior probability threshold. level Level of the credibility intervals. tau\_scale Standard deviation of the half normal prior distribution for the variance of the iter The number of iterations in the simulation. Is ignored if data is specified. Number of MCMC samples. n\_mcmc data An object of class scenario\_list as returned by the function bhmbasket::simulateScenarios.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

### **Examples**

```
design <- setup_bhm(k = 3, p0 = 0.2, p_target = 0.5) get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, tau_scale = 1, iter = 100)
```

Further arguments.

get\_details.bma Get Details of a BMA Basket Trial Simulation

#### **Description**

Get Details of a BMA Basket Trial Simulation

```
## S3 method for class 'bma'
get_details(design, n, p1 = NULL, lambda, pmp0, iter = 1000, data = NULL, ...)
```

get\_details.cpp 11

### **Arguments**

design	An object of class bma.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
pmp0	Power parameter that is used to compute prior probabilities. See bma for details.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

### Value

A list containing the rejection probabilites, posterior means, and mean squared errors for all baskets as well as the family-wise error rate.

# **Examples**

```
design <- setup_bma(k = 3, p0 = 0.2) get_details(design = design, n = 20, p1 = 0.5, lambda = 0.95, pmp0 = 1, iter = 100)
```

get\_details.cpp

Get Details of a Basket Trial Simulation with the Calibrated Power Prior Design

# Description

Get Details of a Basket Trial Simulation with the Calibrated Power Prior Design

```
## S3 method for class 'cpp'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  tune_a,
  tune_b,
  iter = 1000,
  data = NULL,
  ...
)
```

### **Arguments**

design	An object of class cpp.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
tune_a	First tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
tune_b	Second tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

#### **Examples**

```
design <- setup_cpp(k = 3, p0 = 0.2) get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, tune_a = 1, tune_b = 1, iter = 100)
```

# **Description**

Get Details of a Basket Trial Simulation with the Global Calibrated Power Prior Design

```
## S3 method for class 'cppglobal'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  tune_a,
```

get\_details.exnex 13

```
tune_b,
  epsilon,
  iter = 1000,
  data = NULL,
   ...
)
```

# Arguments

design	An object of class cppgen.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to $p0$ .
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
tune_a	First tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
tune_b	Second tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
epsilon	Tuning parameter that determines the amount of borrowing based on overall heterogeneity.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with $get\_data$ . If data is used, then iter is ignored.
	Further arguments.

# Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

# **Examples**

```
design <- setup_cppglobal(k = 3, p0 = 0.2)
get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
tune_a = 1, tune_b = 1, epsilon = 2, iter = 100)
```

get\_details.exnex

Get Details of a Basket Trial Simulation with the EXNEX Design

# Description

Get Details of a Basket Trial Simulation with the EXNEX Design

14 get\_details.exnex

### Usage

```
## S3 method for class 'exnex'
get_details(
   design,
   n,
   p1 = NULL,
   lambda,
   level = 0.95,
   tau_scale,
   w,
   iter = 1000,
   n_mcmc = 10000,
   data = NULL,
   ...
)
```

An object of class exnex.

#### **Arguments**

design

n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
tau_scale	Standard deviation of the half normal prior exchangeability distribution for the variance of the thetas.
W	Fixed prior weight for the exchangeability part of the model.
iter	The number of iterations in the simulation. Is ignored if data is specified.
n_mcmc	Number of MCMC samples.
data	$An \ object \ of \ class \ scenario\_list \ as \ returned \ by \ the \ function \ bhmbasket:: simulate Scenarios.$
	Further arguments.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

```
design <- setup_exnex(k = 3, p0 = 0.2) get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, tau_scale = 1, w = 0.5, iter = 100)
```

get\_details.fujikawa 15

get\_details.fujikawa Get Details of a Basket Trial Simulation with Fujikawa's Design

**Description** 

Get Details of a Basket Trial Simulation with Fujikawa's Design

# Usage

```
## S3 method for class 'fujikawa'
get_details(
  design,
    n,
    p1 = NULL,
    lambda,
    level = 0.95,
    epsilon,
    tau,
    logbase = 2,
    iter = 1000,
    data = NULL,
    ...
)
```

# **Arguments**

design	An object of class fujikawa.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
epsilon	Tuning parameter that determines the amount of borrowing. See setup_fujikawa).
tau	Tuning parameter that determines how similar the baskets have to be that information is shared. See <pre>setup_fujikawa</pre> ).
logbase	Tuning parameter. The base of the logarithm that is used to calculate the Jensen-Shannon divergence.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

# Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate and the experiment-wise power.

get\_details.jsdglobal

# **Examples**

16

```
design <- setup_fujikawa(k = 3, p0 = 0.2) get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, epsilon = 2, tau = 0, iter = 100)
```

 $\begin{array}{ll} \texttt{get\_details.jsdglobal} & \textit{Get Details of a Basket Trial Simulation with the Power Prior Design} \\ & \textit{Based on Global JSD Weights} \end{array}$ 

# **Description**

Get Details of a Basket Trial Simulation with the Power Prior Design Based on Global JSD Weights

# Usage

```
## S3 method for class 'jsdglobal'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  eps_pair,
  tau = 0,
  eps_all,
  logbase = 2,
  iter = 1000,
  data = NULL,
  ...
)
```

design	An object of class jsdgen.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to $p0$ .
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
eps_pair	Tuning parameter that determines the amount of borrowing based on pairwise similarity.
tau	Tuning parameter that determines how similar the baskets have to be that information is shared.
eps_all	Tuning parameter that determines the amount of borrowing based on overall heterogeneity.

get\_details.mml

logbase	Tuning parameter. The base of the logarithm that is used to calculate the Jensen-Shannon divergence.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

# **Examples**

```
design <- setup_jsdglobal(k = 3, p0 = 0.2)
get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
   eps_pair = 2, eps_all = 2, iter = 100)</pre>
```

get\_details.mml

Get Details of a Basket Trial Simulation with the MML Design

### **Description**

Get Details of a Basket Trial Simulation with the MML Design

### Usage

```
## S3 method for class 'mml'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  iter = 1000,
  data = NULL,
  ...
)
```

design	An object of class cpp.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
level	Level of the credibility intervals.

The number of iterations in the simulation. Is ignored if data is specified.
 A data matrix with k column with the number of responses for each basket. Has to be generated with get\_data. If data is used, then iter is ignored.
 Further arguments.

#### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

### **Examples**

```
design <- setup_mml(k = 3, p0 = 0.2)
get_details(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
tune_a = 1, tune_b = 1, iter = 100)
```

get\_details.mmlglobal Get Details of a Basket Trial Simulation with the Global MML Design

# **Description**

Get Details of a Basket Trial Simulation with the Global MML Design

### Usage

```
## $3 method for class 'mmlglobal'
get_details(
  design,
  n,
  p1 = NULL,
  lambda,
  level = 0.95,
  iter = 1000,
  data = NULL,
  ...
)
```

design	An object of class mmlglobal.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
level	Level of the credibility intervals.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

get\_results 19

### Value

A list containing the rejection probabilites, posterior means, mean squared errors and mean limits of HDI intervals for all baskets as well as the family-wise error rate.

### **Examples**

```
design <- setup_mmlglobal(k = 3, p0 = 0.2)
get_details(design = design, n = 20, p1 = 0.5, lambda = 0.95, iter = 100)
```

get\_results

Get Results for Simulation of Basket Trial Designs

# Description

Get Results for Simulation of Basket Trial Designs

#### Usage

```
get_results(design, ...)
```

# Arguments

design An object created with one of the setup functions.

... Further arguments.

# Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

```
# Example for a basket trial with Fujikawa's Design
design <- setup_fujikawa(k = 3, p0 = 0.2)
get_results(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
    epsilon = 2, tau = 0, iter = 100)</pre>
```

20 get\_results.bhm

get\_results.bhm

Get Results for Simulation of a Basket Trial with the BHM Design

### **Description**

Get Results for Simulation of a Basket Trial with the BHM Design

# Usage

```
## S3 method for class 'bhm'
get_results(
  design,
  n,
  p1 = NULL,
  lambda,
  tau_scale,
  iter = 1000,
  n_mcmc = 10000,
  data = NULL,
   ...
)
```

# Arguments

design	An object of class bhm.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
tau_scale	Standard deviation of the half normal prior distribution for the variance of the thetas.
iter	The number of iterations in the simulation. Is ignored if data is specified.
n_mcmc	Number of MCMC samples.
data	$An \ object \ of \ class \ scenario\_list \ as \ returned \ by \ the \ function \ bhmbasket:: simulate Scenarios.$
	Further arguments.

#### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

```
design <- setup_bhm(k = 3, p0 = 0.2, p_target = 0.5)
get_results(design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
  tau_scale = 1, iter = 100)</pre>
```

get\_results.bma 21

		٦.	
σ <sub>P</sub> t	resi	11†5	nma

Get Results for Simulation of a Basket Trial with the BMA Design

# Description

Get Results for Simulation of a Basket Trial with the BMA Design

# Usage

```
## S3 method for class 'bma'
get_results(design, n, p1 = NULL, lambda, pmp0, iter = 1000, data = NULL, ...)
```

# **Arguments**

design	An object of class bma.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
pmp0	Power parameter that is used to compute prior probabilities. See bma for details.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

# Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

```
design <- setup_bma(k = 3, p0 = 0.2) get_results(design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, pmp0 = 1, iter = 100)
```

22 get\_results.cpp

0 - 11	et Results for Simulation of a Basket Trial with a Calibrated Power rior Design
--------	---

# Description

Get Results for Simulation of a Basket Trial with a Calibrated Power Prior Design

# Usage

```
## S3 method for class 'cpp'
get_results(
  design,
  n,
  p1 = NULL,
  lambda,
  tune_a,
  tune_b,
  iter = 1000,
  data = NULL,
  ...
)
```

# Arguments

design	An object of class cpp.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to $p0$ .
lambda	The posterior probability threshold.
tune_a	First tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
tune_b	Second tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with $\texttt{get\_data}$ . If data is used, then iter is ignored.
	Further arguments.

### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

get\_results.cppglobal 23

# **Examples**

```
design <- setup_cpp(k = 3, p0 = 0.2)
get_results(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
  tune_a = 1, tune_b = 1, iter = 100)</pre>
```

# Description

Get Results for Simulation of a Basket Trial with a Global Calibrated Power Prior Design

# Usage

```
## S3 method for class 'cppglobal'
get_results(
  design,
  n,
  p1 = NULL,
  lambda,
  tune_a,
  tune_b,
  epsilon,
  iter = 1000,
  data = NULL,
  ...
)
```

design	An object of class cppgen.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to $p0$ .
lambda	The posterior probability threshold.
tune_a	First tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
tune_b	Second tuning parameter that determines the amount of borrowing based on pairwise similarity between baskets.
epsilon	Tuning parameter that determines the amount of borrowing based on overall heterogeneity.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

24 get\_results.exnex

#### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

#### **Examples**

```
design <- setup_cppglobal(k = 3, p0 = 0.2) get_results(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, tune_a = 1, tune_b = 1, epsilon = 2, iter = 100)
```

get\_results.exnex

Get Results for Simulation of a Basket Trial with the EXNEX Design

# **Description**

Get Results for Simulation of a Basket Trial with the EXNEX Design

#### Usage

```
## S3 method for class 'exnex'
get_results(
  design,
  n,
  p1 = NULL,
  lambda,
  tau_scale,
  w,
  iter = 1000,
  n_mcmc = 10000,
  data = NULL,
  ...
)
```

### **Arguments**

design An object of class exnex. n The sample size per basket. Probabilities used for the simulation. If NULL then all probabilities are set to p0. p1 lambda The posterior probability threshold. tau\_scale Standard deviation of the half normal prior exchangeability distribution for the variance of the thetas. W Fixed prior weight for the exchangeability part of the model. The number of iterations in the simulation. Is ignored if data is specified. iter n\_mcmc Number of MCMC samples. data An object of class scenario\_list as returned by the function bhmbasket::simulateScenarios. Further arguments.

get\_results.fujikawa 25

### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

# **Examples**

```
design <- setup_exnex(k = 3, p0 = 0.2) get_results(design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, tau_scale = 1, w = 0.5, iter = 100)
```

get\_results.fujikawa Get Results for Simulation of a Basket Trial with Fujikawa's Design

# Description

Get Results for Simulation of a Basket Trial with Fujikawa's Design

# Usage

```
## $3 method for class 'fujikawa'
get_results(
  design,
    n,
    p1 = NULL,
    lambda,
    epsilon,
    tau,
    logbase = 2,
    iter = 1000,
    data = NULL,
    ...
)
```

design	An object of class fujikawa.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
epsilon	Tuning parameter that determines the amount of borrowing. See setup_fujikawa).
tau	Tuning parameter that determines how similar the baskets have to be that information is shared. See setup_fujikawa).
logbase	Tuning parameter. The base of the logarithm that is used to calculate the Jensen-Shannon divergence.
iter	The number of iterations in the simulation. Is ignored if data is specified.

26 get\_results.jsdglobal

A data matrix with k column with the number of responses for each basket. Has to be generated with get\_data. If data is used, then iter is ignored.

... Further arguments.

#### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

### **Examples**

```
design <- setup_fujikawa(k = 3, p0 = 0.2)
get_results(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
epsilon = 2, tau = 0, iter = 100)
```

get\_results.jsdglobal Get Results for Simulation of a Basket Trial with the Power Prior Design Based on Global JSD Weights

### Description

Get Results for Simulation of a Basket Trial with the Power Prior Design Based on Global JSD Weights

# Usage

```
## S3 method for class 'jsdglobal'
get_results(
  design,
  n,
  p1 = NULL,
  lambda,
  eps_pair,
  tau = 0,
  eps_all,
  logbase = 2,
  iter = 1000,
  data = NULL,
  ...
)
```

### Arguments

design An object of class jsdgen.

n The sample size per basket.

p1 Probabilities used for the simulation. If NULL then all probabilities are set to p0.

1 The posterior probability threshold.

get\_results.mml 27

eps_pair	Tuning parameter that determines the amount of borrowing based on pairwise similarity.
tau	Tuning parameter that determines how similar the baskets have to be that information is shared.
eps_all	Tuning parameter that determines the amount of borrowing based on overall heterogeneity.
logbase	Tuning parameter. The base of the logarithm that is used to calculate the Jensen-Shannon divergence.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

#### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

# **Examples**

```
design <- setup_jsdglobal(k = 3, p0 = 0.2)
get_results(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
   eps_pair = 2, eps_all = 2, iter = 100)</pre>
```

get\_results.mml

Get Results for Simulation of a Basket Trial with the MML Design

### **Description**

Get Results for Simulation of a Basket Trial with the MML Design

# Usage

```
## S3 method for class 'mml'
get_results(design, n, p1 = NULL, lambda, iter = 1000, data = NULL, ...)
```

design	An object of class mml.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

#### Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

# **Examples**

```
design <- setup_mml(k = 3, p0 = 0.2) get_results(design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, iter = 100)
```

### **Description**

Get Results for Simulation of a Basket Trial with the Global MML Design

#### Usage

```
## S3 method for class 'mmlglobal'
get_results(design, n, p1 = NULL, lambda, iter = 1000, data = NULL, ...)
```

#### **Arguments**

design	An object of class mmlglobal.
n	The sample size per basket.
p1	Probabilities used for the simulation. If NULL then all probabilities are set to p0.
lambda	The posterior probability threshold.
iter	The number of iterations in the simulation. Is ignored if data is specified.
data	A data matrix with k column with the number of responses for each basket. Has to be generated with get_data. If data is used, then iter is ignored.
	Further arguments.

# Value

A matrix of results with iter rows. A 0 means, that the null hypothesis that the response probability exceeds p0 was not rejected, a 1 means, that the null hypothesis was rejected.

```
design <- setup_mmlglobal(k = 3, p0 = 0.2) get_results(design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95, iter = 100)
```

get\_scenarios 29

get\_scenarios

Create a Scenario Matrix

# **Description**

Creates a default scenario matrix.

### Usage

```
get_scenarios(design, p1)
```

# **Arguments**

design An object created with one of the setup functions.

p1 Probability under the alternative hypothesis.

#### **Details**

get\_scenarios creates a default scenario matrix that can be used for  $opt_design$ . The function creates k + 1 scenarios, from a global null to a global alternative scenario.

### Value

A matrix with k rows and k + 1 columns.

# **Examples**

```
design <- setup_fujikawa(k = 3, p0 = 0.2)
get_scenarios(design = design, p1 = 0.5)
```

 ${\tt opt\_design}$ 

Optimize a Basket Trial Design

### **Description**

Optimize a Basket Trial Design

```
opt_design(
  design,
  n,
  alpha,
  design_params = list(),
  scenarios,
  prec_digits,
```

30 opt\_design

```
iter = 1000,
data = NULL,
...
)
```

#### Arguments

design An object created with one of the setup functions.

n The sample size per basket.

alpha The one-sided significance level.

design\_params A list of params that is specific to the class of design.

scenarios A matrix of scenarios.

prec\_digits Number of decimal places that are considered when adjusting lambda.

iter The number of iterations in the simulation. Is ignored if data is specified.

data A list of data matrices generated with get\_data. The list elements have to

correspond to the columnn of scenarios.

... Further arguments.

#### Value

A matrix with the expected number of correct decisions.

```
design <- setup_fujikawa(k = 3, p0 = 0.2)
scenarios <- get_scenarios(design, p1 = 0.5)

# Without simulated data
opt_design(design, n = 20, alpha = 0.05, design_params =
    list(epsilon = c(1, 2), tau = c(0, 0.5)), scenarios = scenarios,
    prec_digits = 3)

# With simulated data
scenario_list <- as.list(data.frame(scenarios))
data_list <- lapply(scenario_list,
    function(x) get_data(k = 3, n = 20, p = x, iter = 1000))
opt_design(design, n = 20, alpha = 0.05, design_params =
    list(epsilon = c(1, 2), tau = c(0, 0.5)), scenarios = scenarios,
    prec_digits = 3, data = data_list)</pre>
```

setup\_bhm 31

setup_bhm	Setup BHM Design Object	
oc cap_brilli	Settip BIIII Besign Coject	

#### **Description**

Setup BHM Design Object

### Usage

```
setup_bhm(k, p0, p_target, mu_mean = NULL, mu_sd = 100)
```

# **Arguments**

k The number of baskets.

p0 A common probability under the null hypothesis.

p\_target The response rate of interest. See details.

mu\_mean Mean of the normal prior distribution for the mean of the thetas. See details.

mu\_sd Standard deviation of the normal prior distribution for the mean of the thetas.

#### **Details**

The class bhm implements the Bayesian Hierarchical Model proposed by Berry et al. (2013). Methods for this class are mostly wrappers for functions from the package bhmbasket.

In the BHM the thetas of all baskets are modeled, where theta\_i =  $logit(p_i)$  -  $logit(p_target)$ . These thetas are assumed to come from a normal distribution with mean mu\_mean and standard deviation mu\_sd. If mu\_mean = NULL then mu\_mean is determined as logit(p0) -  $logit(p_target)$ , hence the mean of the normal distribution corresponds to the null hypothesis.

#### Value

An S3 object of class bhm

#### References

Berry, S. M., Broglio, K. R., Groshen, S., & Berry, D. A. (2013). Bayesian hierarchical modeling of patient subpopulations: efficient designs of phase II oncology clinical trials. Clinical Trials, 10(5), 720-734.

```
design_bhm <- setup_bhm(k = 3, p0 = 0.2, p_target = 0.5)
```

32 setup\_bma

setup_	bma	

Setup bma Design Object

### **Description**

Creates an object of class bma.

# Usage

```
setup_bma(k, p0, shape1 = 1, shape2 = 1)
```

# Arguments

k	The number of baskets

p0 A common probability under the null hypothesis.

shape1 First common shape parameter of the beta prior.

shape2 Second common shape parameter of the beta prior.

### **Details**

The class bma implements the Bayesian Model Averaging design by Pisoda et al. (2021). Functions for this class are mostly wrappers for functions of the bmabasket package.

# Value

An S3 object of class bma

#### References

Psioda, M. A., Xu, J., Jiang, Q. I., Ke, C., Yang, Z., & Ibrahim, J. G. (2021). Bayesian adaptive basket trial design using model averaging. Biostatistics, 22(1), 19-34.

```
design_bma \leftarrow setup_bma(k = 3, p0 = 0.2)
```

setup\_cpp 33

setup_cpp	Setup Calibrated Power Prior Design Object

### **Description**

Setup Calibrated Power Prior Design Object

#### Usage

```
setup\_cpp(k, p0, shape1 = 1, shape2 = 1)
```

# Arguments

k	The number of baskets.
p0	A common probability under the null hypothesis.
shape1	First common shape parameter of the beta prior.
shape2	Second common shape parameter of the beta prior.

### **Details**

The class cpp implements a version of the power prior design, in which the amount of information that is shared between baskets is determined by the Kolmogorov-Smirnov test statistic between baskets (which is equivalent to the absolut difference in response rates).

### Value

An S3 object of class cpp

#### References

Baumann, L., Sauer, L., & Kieser, M. (2024). A basket trial design based on power priors. arXiv:2309.06988.

```
design_cpp \leftarrow setup_cpp(k = 3, p0 = 0.2)
```

34 setup\_cppglobal

Setup Global Calibrated Power Prior Design Object

### **Description**

Setup Global Calibrated Power Prior Design Object

### Usage

```
setup\_cppglobal(k, p0, shape1 = 1, shape2 = 1)
```

# **Arguments**

p0 A common probability under the null hypothesis.

shape1 First common shape parameter of the beta prior.

shape2 Second common shape parameter of the beta prior.

#### **Details**

The class cppglobal implements a version of the power prior design, in which the amount of information that is shared between baskets is determined by the Kolmogorov-Smirnov test statistic between basekts and a function based on response rate differences that quantifies the overall heterogeneity.

# Value

An S3 object of class cppglobal

# References

Baumann, L., Sauer, L., & Kieser, M. (2024). A basket trial design based on power priors. arXiv:2309.06988.

```
design_cppglobal <- setup_cppglobal(k = 3, p0 = 0.2)</pre>
```

setup\_exnex 35

setup\_exnex

Setup EXNEX Design Object

### Description

Setup EXNEX Design Object

#### Usage

```
setup_exnex(
   k,
   p0,
   basket_mean = NULL,
   basket_sd = 100,
   mu_mean = NULL,
   mu_sd = 100
)
```

# **Arguments**

k The number of baskets.

p0 A common probability under the null hypothesis.

basket\_mean Mean of the normal prior distribution of the individual thetas (NEX part). See

details.

basket\_sd Standard deviation of the normal prior distribution of the individual thetas (NEX

part).

mu\_mean Mean of the normal prior exchangeability distribution for the mean of the thetas

(EX part). See details.

mu\_sd Standard deviation of the normal prior exchangeability distribution for the mean

of the thetas (EX part).

#### **Details**

The class exnex implements the EXNEX model proposed by Neuenschwander et al. (2016). Methods for this class are mostly wrappers for functions from the package bhmbasket.

In the EXNEX model the thetas of all baskets are modeled as a mixture of individual models and a Bayesian Hierarchical Model with a fixed mixture weight w. If mu\_mean and basket\_mean are NULL then they are set to logit(p0). Note that Neuenschwander et al. (2016) use different prior means and standard deviations. The default values here are used for better comparison with the BHM model (see setup\_bhm).

#### Value

An S3 object of class exnex

36 setup\_fujikawa

#### References

Neuenschwander, B., Wandel, S., Roychoudhury, S., & Bailey, S. (2016). Robust exchangeability designs for early phase clinical trials with multiple strata. Pharmaceutical statistics, 15(2), 123-134.

# **Examples**

```
design_exnex < - setup_exnex(k = 3, p0 = 0.2)
```

setup\_fujikawa

Setup Fujikawa Design Object

# Description

Setup Fujikawa Design Object

### Usage

```
setup_fujikawa(k, p0, shape1 = 1, shape2 = 1)
```

### **Arguments**

k The number of baskets.

p0 A common probability under the null hypothesis.
shape1 First common shape parameter of the beta prior.
shape2 Second common shape parameter of the beta prior.

# **Details**

The class fujikawa implements a design by Fujikawa et al. (2020) in which information is shared based on the pairwise similarity between baskets which is quantified using the Jensen-Shannon divergence between the individual posterior distributions between baskets.

#### Value

An S3 object of class fujikawa

#### References

Fujikawa, K., Teramukai, S., Yokota, I., & Daimon, T. (2020). A Bayesian basket trial design that borrows information across strata based on the similarity between the posterior distributions of the response probability. Biometrical Journal, 62(2), 330-338.

```
design_fujikawa <- setup_fujikawa(k = 3, p0 = 0.2)</pre>
```

setup\_jsdglobal 37

setup_jsdglobal	Setup Global JSD Design Object

### **Description**

Setup Global JSD Design Object

#### Usage

```
setup_jsdglobal(k, p0, shape1 = 1, shape2 = 1)
```

# Arguments

k	The number of baskets.
p0	A common probability under the null hypothesis.
shape1	First common shape parameter of the beta prior.
shape2	Second common shape parameter of the beta prior.

### **Details**

The class jsdglobal implements a version of the power prior design, in which information is shared based on pairwise similarity and overall heterogeneity between baskets. Both pairwise similarity and overall heterogeneity are assessed using the Jensen-Shannon divergence.

### Value

An S3 object of class jsdglobal

#### References

Baumann, L., Sauer, L., & Kieser, M. (2024). A basket trial design based on power priors. arXiv:2309.06988.

```
design_jsdglobal \leftarrow setup_jsdglobal(k = 3, p0 = 0.2)
```

38 setup\_mml

			-
setu	n	mm	١I

Setup mml Design Object

### Description

Creates an object of class mml.

#### Usage

```
setup_mml(k, p0, shape1 = 1, shape2 = 1)
```

# **Arguments**

L.	The number of baskets.
K	The number of paskers

p0 A common probability under the null hypothesis.
shape1 First common shape parameter of the beta prior.
shape2 Second common shape parameter of the beta prior.

#### **Details**

The class mml implements a modified version of the empirical Bayes method by Gravestock & Held (2017) which was proposed for borrowing strength from an external study. In their approach, the sharing weight is found as the maximum of the marginal likelihood of the weight, given the external data set. This leads, however, to non-symmetric weights when applied to sharing in basket trials, i.e. Basket i would not share the information from Basket j as the other way round. Therefore, a symmetrised version is used, where the mean of the two weights resulting from sharing in both directions is used.

#### Value

An S3 object of class mml

### References

Gravestock, I., & Held, L. (2017). Adaptive power priors with empirical Bayes for clinical trials. Pharmaceutical statistics, 16(5), 349-360.

```
design_mml \leftarrow setup_mml(k = 3, p0 = 0.2)
```

setup\_mmlglobal 39

setup_mmlglobal	Setup mmlglobal Design Object

# Description

Creates an object of class mmlglobal.

### Usage

```
setup_mmlglobal(k, p0, shape1 = 1, shape2 = 1)
```

### **Arguments**

k	The number of baskets.
p0	A common probability under the null hypothesis.
shape1	First common shape parameter of the beta prior.

# Details

shape2

The class mmlglobal implements an empirical Bayes method by Gravestock & Held (2019) which was proposed for borrowing strength from multiple external studies.

Second common shape parameter of the beta prior.

### Value

An S3 object of class mmlglobal

### References

Gravestock, I., & Held, L. (2019). Power priors based on multiple historical studies for binary outcomes. Biometrical Journal, 61(5), 1201-1218.

Baumann, L., Sauer, L., & Kieser, M. (2024). A basket trial design based on power priors. arXiv:2309.06988.

```
design_mmlglobal <- setup_mmlglobal(k = 3, p0 = 0.2)
```

40 toer

toer

Calculate the Type 1 Error Rate for a Basket Trial Design

#### Description

Calculate the Type 1 Error Rate for a Basket Trial Design

### Usage

```
toer(
  design,
  n,
  p1 = NULL,
  lambda,
  design_params = list(),
  iter = 1000,
  data = NULL,
  ...
)
```

#### **Arguments**

design An object created with one of the setup functions.

n The sample size per basket.

p1 Probabilities under the alternative hypothesis. If NULL then the type 1 error rate

under the global null hypothesis is calculated.

lambda The posterior probability threshold.

design\_params A list of params that is specific to the class of design.

iter The number of iterations in the simulation. Is ignored if data is specified.

data A data matrix with k column with the number of responses for each basket. Has

to be generated with get\_data. If data is used, then iter is ignored.

... Further arguments.

### Value

A numeric value.

```
# Example for a basket trial with Fujikawa's Design
design <- setup_fujikawa(k = 3, p0 = 0.2)
toer(design = design, n = 20, p1 = c(0.2, 0.5, 0.5), lambda = 0.95,
  design_params = list(epsilon = 2, tau = 0), iter = 1000)</pre>
```

# **Index**

```
adjust_lambda, 3
                                                setup_mml, 38
adjust_lambda.bhm, 3
                                                setup_mmlglobal, 39
adjust_lambda.default, 5
                                                toer, 3, 40
adjust_lambda.exnex, 6
                                                uniroot, 3
bma, 11, 21
ecd, 7
get_data, 8
get_details, 9
get_details.bhm, 9
get_details.bma, 10
get_details.cpp, 11
get_details.cppglobal, 12
get_details.exnex, 13
get_details.fujikawa, 15
get_details.jsdglobal, 16
get_details.mml, 17
get_details.mmlglobal, 18
get_results, 19
get\_results.bhm, 20
get\_results.bma, 21
get_results.cpp, 22
get_results.cppglobal, 23
get_results.exnex, 24
get_results.fujikawa, 25
get_results.jsdglobal, 26
get\_results.mml, 27
get_results.mmlglobal, 28
get_scenarios, 29
opt_design, 29, 29
setup_bhm, 31, 35
setup_bma, 32
setup_cpp, 33
setup_cppglobal, 34
setup_exnex, 35
setup_fujikawa, 15, 25, 36
setup_jsdglobal, 37
```