

# Package: BayesPPDSurv (via r-universe)

September 7, 2024

**Title** Bayesian Power Prior Design for Survival Data

**Version** 1.0.3

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**Description** Bayesian power/type I error calculation and model fitting using the power prior and the normalized power prior for proportional hazards models with piecewise constant hazard. The methodology and examples of applying the package are detailed in [doi:10.48550/arXiv.2404.05118](https://doi.org/10.48550/arXiv.2404.05118). The Bayesian clinical trial design methodology is described in Chen et al. (2011) [doi:10.1111/j.1541-0420.2011.01561.x](https://doi.org/10.1111/j.1541-0420.2011.01561.x), and Psioda and Ibrahim (2019) [doi:10.1093/biostatistics/kxy009](https://doi.org/10.1093/biostatistics/kxy009). The proportional hazards model with piecewise constant hazard is detailed in Ibrahim et al. (2001) [doi:10.1007/978-1-4757-3447-8](https://doi.org/10.1007/978-1-4757-3447-8).

**License** GPL (>= 3)

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**LinkingTo** Rcpp, RcppArmadillo, RcppDist

**Suggests** knitr, rmarkdown, testthat (>= 3.0.0)

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**Config/testthat/edition** 3

**Depends** R (>= 2.10)

**NeedsCompilation** yes

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BayesPPDSurv-package	<i>Bayesian sample size determination using the power and normalized power prior for survival data</i>
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### Description

The **BayesPPDSurv** (Bayesian Power Prior Design for Survival Data) package provides two categories of functions: functions for Bayesian power/type I error calculation and functions for model fitting.

### References

- Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.
- Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.
- Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

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approximate.prior.beta	<i>Approximating the normalized power prior for <math>\beta</math> for the proportional hazards model with piecewise constant hazard and random <math>a_0</math></i>
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### Description

Approximation of the normalized power prior for  $\beta$  for the proportional hazards model with piecewise constant hazard and random  $a_0$ . The function returns discrete samples of  $\beta$  from the normalized power prior, and the user can use any mixture of multivariate normal distributions as an approximation for the normalized power prior for  $\beta$ . This function is used to produce `prior.beta.mvn` in the function `power.phm.random.a0`.

**Usage**

```

approximate.prior.beta(
  historical,
  n.intervals,
  change.points = NULL,
  prior.a0.shape1 = rep(1, 10),
  prior.a0.shape2 = rep(1, 10),
  prior.beta.mean = rep(0, 50),
  prior.beta.sd = rep(1000, 50),
  prior.lambda0.hp1 = rep(10^(-5), 50),
  prior.lambda0.hp2 = rep(10^(-5), 50),
  lower.limits = rep(-100, 50),
  upper.limits = rep(100, 50),
  slice.widths = rep(0.1, 50),
  nMC = 10000,
  nBI = 250
)

```

**Arguments**

historical	List of historical dataset(s). Each historical dataset is stored in a list which contains four <i>named</i> elements: time, event, X and S. <ul style="list-style-type: none"> <li>time is a vector of follow up times.</li> <li>event is a vector of status indicators. Normally 0=alive and 1=dead.</li> <li>X is a matrix of covariates. The first column must be the treatment indicator.</li> <li>S is a vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.</li> </ul>
n.intervals	Vector of integers, indicating the number of intervals for the baseline hazards for each stratum. The length of the vector should be equal to the total number of strata.
change.points	List of vectors. Each vector in the list contains the change points for the baseline hazards for each stratum. The length of the list should be equal to the total number of strata. For a given stratum, if there is only one interval, then change.points should be NULL for that stratum. By default, we assign the change points so that the same number of events are observed in all the intervals in the historical data.
prior.a0.shape1	Vector of the first shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
prior.a0.shape2	Vector of the second shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
prior.beta.mean	Vector of means of the normal initial prior on $\beta$ . The default value is zero for all the elements of $\beta$ .

prior.beta.sd	Vector of standard deviations of the normal initial prior on $\beta$ . The default value is $10^3$ for all the elements of $\beta$ .
prior.lambda0.hp1	Vector of first hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
prior.lambda0.hp2	Vector of second hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
lower.limits	Vector of lower limits for $\beta$ to be used by the slice sampler. The length of the vector should be equal to the length of $\beta$ . The default is -100 for all the elements of $\beta$ (may not be appropriate for all situations).
upper.limits	Vector of upper limits for $\beta$ to be used by the slice sampler. The length of the vector should be equal to the length of $\beta$ . The default is 100 for all the elements of $\beta$ (may not be appropriate for all situations).
slice.widths	Vector of initial slice widths for $\beta$ to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 0.1 for all the elements of $\beta$ (may not be appropriate for all situations).
nMC	Number of iterations (excluding burn-in samples) for the slice sampler. The default is 10,000.
nBI	Number of burn-in samples for the slice sampler. The default is 250.

### Details

This function is used to produce `prior.beta.mvn` in the function `power.phm.random.a0`. It approximates the normalized power prior for  $\beta$  when  $a_0$  is modeled as random. The function returns discrete samples of  $\beta$  from the normalized power prior, and the user can use any mixture of multivariate normal distributions as an approximation for the normalized power prior for  $\beta$ .

Baseline hazard parameters for the current and historical data are NOT shared. The baseline hazards of the historical data are denoted by  $\lambda_0$ . We assume Gamma priors for  $\lambda_0$  and independent normal initial priors for  $\beta$ .

Posterior samples are obtained through slice sampling. The default lower limits are -100 for  $\beta$ . The default upper limits for the parameters are 100. The default slice widths for the parameters are 0.1. The defaults may not be appropriate for all situations, and the user can specify the appropriate limits and slice width for each parameter.

### Value

Samples of  $\beta$  (approximating the normalized power prior) are returned.

### References

- Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.
- Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.

Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

### See Also

[phm.random.a0](#) and [power.phm.random.a0](#)

### Examples

```
# Simulate two historical datasets
n <- 100
P <- 4
time1 <- round(rexp(n, rate=0.5),1)
event1 <- rep(1,n)
X1 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S1 <- c(rep(1,n/2),rep(2,n/2))
time2 <- round(rexp(n, rate=0.7),1)
event2 <- rep(1,n)
X2 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S2 <- c(rep(1,n/2),rep(2,n/2))
historical <- list(list(time=time1, event=event1, X=X1, S=S1),
                  list(time=time2, event=event2, X=X2, S=S2))

# We choose three intervals for the first stratum and two intervals for the second stratum
n.intervals <- c(3,2)
change.points <- list(c(1,2), 2)

# Get samples from the approximate normalized power prior for beta
nMC <- 100 # nMC should be larger in practice
nBI <- 50
prior.beta <- approximate.prior.beta(historical, n.intervals, change.points=change.points,
                                     prior.a0.shape1=c(1,1), prior.a0.shape2=c(1,1),
                                     nMC=nMC, nBI=nBI)

prior_beta_mu=colMeans(prior.beta)
prior_beta_sigma=cov(prior.beta)

# Approximate the discrete samples with a single multivariate normal with weight one.
# The user can use any mixture of multivariate normal distributions as an
# approximation for the normalized power prior for beta.
prior.beta.mvn <- list(list(prior_beta_mu, prior_beta_sigma, 1))
# prior.beta.mvn is a parameter for phm.random.a0() and power.phm.random.a0()
```

**Description**

A dataset for the E1684 clinical trial (1996) and the E1690 clinical trial (2000) conducted to assess the utility of interferon alfa-2b (INF) as an adjuvant therapy following surgery for deep primary or regionally metastatic melanoma. This dataset contains subjects in disease stage four only, i.e., regional lymph node recurrence at any interval after appropriate surgery for primary melanoma of any depth.

**Usage**

melanoma

**Format**

A data frame with 381 rows and 5 variables:

**study** study number (1684 or 1690)

**failtime** time to relapse in years

**rfscens** censoring indicator for time to relapse (0 = did not relapse, 1 = relapsed)

**trt** treatment (1 if IFN and 0 if Observation)

**stratum** stratum index (1 if number of positive nodes  $\leq 2$  and 2 if number of positive nodes  $\geq 3$ )

**Source**

Kirkwood, J. M., J. G. Ibrahim, V. K. Sondak, J. Richards, L. E. Flaherty, M. S. Ernstoff, T. J. Smith, U. Rao, M. Steele, and R. H. Blum (2000, Jun). High- and low-dose interferon alfa-2b in high-risk melanoma: first analysis of intergroup trial e1690/s9111/c9190. *Journal of Clinical Oncology* 18 (12), 2444–2458.

Kirkwood, J. M., M. H. Strawderman, M. S. Ernstoff, T. J. Smith, E. C. Borden, and R. H. Blum (1996). Interferon alfa-2b adjuvant therapy of high-risk resected cutaneous melanoma: the eastern cooperative oncology group trial est 1684. *Journal of Clinical Oncology* 14, 7–17.

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p<sub>hm.fixed.a0</sub>

*Model fitting for the proportional hazards model with piecewise constant hazard and fixed  $a_0$*

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**Description**

Model fitting using power priors for the proportional hazards model with piecewise constant hazard and fixed  $a_0$

**Usage**

```

phm.fixed.a0(
  time = NULL,
  event = NULL,
  X = NULL,
  S = NULL,
  historical,
  a0,
  n.intervals,
  change.points = NULL,
  shared.blh = FALSE,
  prior.beta = "Normal",
  prior.beta.mean = rep(0, 50),
  prior.beta.sd = rep(1000, 50),
  prior.lambda = "Gamma",
  prior.lambda.hp1 = rep(10-5, 50),
  prior.lambda.hp2 = rep(10-5, 50),
  prior.lambda0.hp1 = rep(10-5, 50),
  prior.lambda0.hp2 = rep(10-5, 50),
  lower.limits = NULL,
  upper.limits = rep(100, 50),
  slice.widths = rep(0.1, 50),
  current.data = TRUE,
  nMC = 10000,
  nBI = 250
)

```

**Arguments**

time	Vector of follow up times.
event	Vector of status indicators. Normally 0=alive and 1=dead.
X	Matrix of covariates. The first column must be the treatment indicator.
S	Vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.
historical	List of historical dataset(s). Each historical dataset is stored in a list which contains four <i>named</i> elements: time, event, X and S. <ul style="list-style-type: none"> <li>• time is a vector of follow up times.</li> <li>• event is a vector of status indicators. Normally 0=alive and 1=dead.</li> <li>• X is a matrix of covariates. The first column must be the treatment indicator.</li> <li>• S is a vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.</li> </ul>
a0	Vector containing numbers between 0 and 1 indicating the discounting parameter value for each historical dataset. The length of the vector should be equal to the length of historical.

<code>n.intervals</code>	Vector of integers, indicating the number of intervals for the baseline hazards for each stratum. The length of the vector should be equal to the total number of strata.
<code>change.points</code>	List of vectors. Each vector in the list contains the change points for the baseline hazards for each stratum. The length of the list should be equal to the total number of strata. For a given stratum, if there is only one interval, then <code>change.points</code> should be NULL for that stratum. By default, we assign the change points so that the same number of events are observed in all the intervals in the pooled current and historical data.
<code>shared.blh</code>	Logical value indicating whether baseline hazard parameters are shared between the current and historical data. If TRUE, baseline hazard parameters are shared. The default value is FALSE.
<code>prior.beta</code>	Prior used for $\beta$ . The choices are "Uniform" and "Normal". If <code>prior.beta</code> is "Uniform", the uniform improper prior is used. If <code>prior.beta</code> is "Normal", independent normal priors are used for each element of $\beta$ . The default choice is "Normal".
<code>prior.beta.mean</code>	(Only applies if <code>prior.beta</code> is "Normal") vector of means of the normal prior on $\beta$ . The default value is zero for all the elements of $\beta$ .
<code>prior.beta.sd</code>	(Only applies if <code>prior.beta</code> is "Normal") vector of standard deviations of the normal prior on $\beta$ . The default value is $10^3$ for all the elements of $\beta$ .
<code>prior.lambda</code>	Prior used for $\lambda$ . The choices are "Gamma", "Log-normal" and "Improper". The default choice is "Gamma". If <code>prior.lambda</code> is "Gamma", then the prior on the first element of $\lambda$ is $\text{Gamma}(\text{shape}=\text{prior.lambda.hp1}[1], \text{rate}=\text{prior.lambda.hp2}[1])$ . If <code>prior.lambda</code> is "Log-normal", then the prior on the first element of $\lambda$ is $\text{Log-normal}(\text{mean}=\text{prior.lambda.hp1}[1], \text{sd}=\text{prior.lambda.hp2}[1])$ . If <code>prior.lambda</code> is "Improper", then the prior on each element of $\lambda$ is the improper prior $\lambda^{-1}$ .
<code>prior.lambda.hp1</code>	(Only applies if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of first hyperparameters of the prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>prior.lambda.hp2</code>	(Only applies if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of second hyperparameters of the prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>prior.lambda0.hp1</code>	(Only applies if <code>shared.blh</code> is FALSE and if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of first hyperparameters of the prior on $\lambda_0$ . We assume the same distribution choice for the prior for $\lambda_0$ and $\lambda$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .



prior.lambda0.hp2	(Only applies if shared.blh is FALSE and if prior.lambda is "Gamma" or "Log-normal") Vector of second hyperparameters of the prior on $\lambda_0$ . We assume the same distribution choice for the prior for $\lambda_0$ and $\lambda$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{-5}$ for all the elements of $\lambda_0$ .
lower.limits	Vector of lower limits for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is -100 for $\beta$ and 0 for $\lambda$ and $\lambda_0$ (may not be appropriate for all situations).
upper.limits	Vector of upper limits for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 100 for all parameters (may not be appropriate for all situations).
slice.widths	Vector of initial slice widths for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 0.1 for all parameters (may not be appropriate for all situations).
current.data	Logical value indicating whether current data is included. The default is TRUE. If FALSE, only historical data is included in the analysis, and the posterior samples can be used as a discrete approximation to the sampling prior in <a href="#">power.phm.fixed.a0</a> and <a href="#">power.phm.random.a0</a> .
nMC	Number of iterations (excluding burn-in samples) for the slice sampler. The default is 10,000.
nBI	Number of burn-in samples for the slice sampler. The default is 250.

### Details

The proportional hazards model with piecewise constant hazard is implemented. We assume  $\beta$  is the regression coefficients. We assume the first column of the covariate matrix is the treatment indicator, and the corresponding parameter is  $\beta_1$ . The baseline hazards of the current data are denoted by  $\lambda$ . The baseline hazards of the historical data are denoted by  $\lambda_0$ . If the baseline hazards are shared between the historical and current data, then  $\lambda_0 = \lambda$ .

Posterior samples are obtained through slice sampling. The default lower limits are -100 for  $\beta$  and 0 for  $\lambda$  and  $\lambda_0$ . The default upper limits for the parameters are 100. The default slice widths for the parameters are 0.1. The defaults may not be appropriate for all situations, and the user can specify the appropriate limits and slice width for each parameter.

### Value

Posterior samples of  $\beta$ ,  $\lambda$  and  $\lambda_0$  (if baseline hazards are not shared between the current and historical data) are returned.

### References

Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.

Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.

Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

### See Also

[power.phm.fixed.a0](#)

### Examples

```
set.seed(1)
# Simulate current data
n <- 50
P <- 4
time <- round(rexp(n, rate=0.5),1)
event <- rep(1,n)
X <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S <- c(rep(1,n/2),rep(2,n/2))

# Simulate two historical datasets
n <- 100
time1 <- round(rexp(n, rate=0.5),1)
event1 <- rep(1,n)
X1 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S1 <- c(rep(1,n/2),rep(2,n/2))
time2 <- round(rexp(n, rate=0.7),1)
event2 <- rep(1,n)
X2 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S2 <- c(rep(1,n/2),rep(2,n/2))
historical <- list(list(time=time1, event=event1, X=X1, S=S1),
                  list(time=time2, event=event2, X=X2, S=S2))

# a0 is 0.3 for the first historical dataset and 0.6 for the second
a0 <- c(0.3, 0.6)

# We choose three intervals for the first stratum and two intervals for the second stratum
n.intervals <- c(3,2)
change.points <- list(c(1,2), 2)

nMC <- 1000 # nMC should be larger in practice
nBI <- 50

result <- phm.fixed.a0(time=time, event=event, X=X, S=S,
                      historical=historical, a0=a0, n.intervals=n.intervals,
                      change.points=change.points, nMC=nMC, nBI=nBI)

# posterior mean of beta
colMeans(result$beta_samples)
```

```

# posterior mean of baseline hazards for stratum 1
colMeans(result$lambda_samples[[1]])
# posterior mean of baseline hazards for stratum 2
colMeans(result$lambda_samples[[2]])
# posterior mean of historical baseline hazards for stratum 1
colMeans(result$lambda0_samples[[1]])
# posterior mean of historical baseline hazards for stratum 2
colMeans(result$lambda0_samples[[2]])

```

---

phm.random.a0	<i>Model fitting for the proportional hazards model with piecewise constant hazard and random <math>a_0</math></i>
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### Description

Model fitting using the normalized power prior for the proportional hazards model with piecewise constant hazard and random  $a_0$

### Usage

```

phm.random.a0(
  time,
  event,
  X,
  S,
  historical,
  n.intervals,
  change.points = NULL,
  prior.beta.mvn = NULL,
  prior.beta.mean = rep(0, 50),
  prior.beta.sd = rep(1000, 50),
  prior.lambda0.hp1 = rep(10^(-5), 50),
  prior.lambda0.hp2 = rep(10^(-5), 50),
  prior.a0.shape1 = rep(1, 10),
  prior.a0.shape2 = rep(1, 10),
  prior.lambda.hp1 = rep(10^(-5), 50),
  prior.lambda.hp2 = rep(10^(-5), 50),
  lower.limits = NULL,
  upper.limits = rep(100, 50),
  slice.widths = rep(0.1, 50),
  nMC = 10000,
  nBI = 250
)

```

**Arguments**

<code>time</code>	Vector of follow up times.
<code>event</code>	Vector of status indicators. Normally 0=alive and 1=dead.
<code>X</code>	Matrix of covariates. The first column must be the treatment indicator.
<code>S</code>	Vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.
<code>historical</code>	List of historical dataset(s). Each historical dataset is stored in a list which contains four <i>named</i> elements: <code>time</code> , <code>event</code> , <code>X</code> and <code>S</code> . <ul style="list-style-type: none"> <li>• <code>time</code> is a vector of follow up times.</li> <li>• <code>event</code> is a vector of status indicators. Normally 0=alive and 1=dead.</li> <li>• <code>X</code> is a matrix of covariates. The first column must be the treatment indicator.</li> <li>• <code>S</code> is a vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.</li> </ul>
<code>n.intervals</code>	Vector of integers, indicating the number of intervals for the baseline hazards for each stratum. The length of the vector should be equal to the total number of strata.
<code>change.points</code>	List of vectors. Each vector in the list contains the change points for the baseline hazards for each stratum. The length of the list should be equal to the total number of strata. For a given stratum, if there is only one interval, then <code>change.points</code> should be NULL for that stratum. By default, we assign the change points so that the same number of events are observed in all the intervals in the pooled current and historical data.
<code>prior.beta.mvn</code>	List of multivariate normal approximations of the normalized power prior for $\beta$ . Each element in the list is a list with three components, the mean vector, the covariance matrix and the weight of the multivariate normal distribution. The normalized power prior for $\beta$ is approximated by the weighted mixture of the multivariate normal distributions provided. By default ( <code>prior.beta.mvn=NULL</code> ), a single multivariate normal distribution is assumed. The user can use the <a href="#">approximate.prior.beta</a> function to obtain samples of $\beta$ from the normalized power prior, and use any mixture of multivariate normals to approximate the normalized power prior for $\beta$ .
<code>prior.beta.mean</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of means of the normal initial prior on $\beta$ . The default value is zero for all the elements of $\beta$ .
<code>prior.beta.sd</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of standard deviations of the normal initial prior on $\beta$ . The default value is $10^3$ for all the elements of $\beta$ .
<code>prior.lambda0.hp1</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of first hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{-5}$ for all the elements of $\lambda_0$ .

<code>prior.lambda0.hp2</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of second hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{-5}$ for all the elements of $\lambda_0$ .
<code>prior.a0.shape1</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of the first shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
<code>prior.a0.shape2</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of the second shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
<code>prior.lambda.hp1</code>	Vector of first hyperparameters of the Gamma prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{-5}$ for all the elements of $\lambda$ .
<code>prior.lambda.hp2</code>	Vector of second hyperparameters of the Gamma prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{-5}$ for all the elements of $\lambda$ .
<code>lower.limits</code>	Vector of lower limits for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is -100 for $\beta$ and 0 for $\lambda$ (may not be appropriate for all situations).
<code>upper.limits</code>	Vector of upper limits for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 100 for all parameters (may not be appropriate for all situations).
<code>slice.widths</code>	Vector of initial slice widths for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 0.1 for all parameters (may not be appropriate for all situations).
<code>nMC</code>	Number of iterations (excluding burn-in samples) for the slice sampler. The default is 10,000.
<code>nBI</code>	Number of burn-in samples for the slice sampler. The default is 250.

## Details

The proportional hazards model with piecewise constant hazard is implemented. We assume  $\beta$  is the regression coefficients. We assume the first column of the covariate matrix is the treatment indicator, and the corresponding parameter is  $\beta_1$ . Here  $a_0$  is modeled as random with a normalized power prior.

The normalized power prior for  $\beta$  is approximated by a weighted mixture of multivariate normal distributions provided in `prior.beta.mvn`. The user can use the [approximate.prior.beta](#) function to obtain samples of  $\beta$  from the normalized power prior, and use any mixture of multivariate

normals to approximate the normalized power prior for  $\beta$ . By default, a single multivariate normal distribution is assumed.

Posterior samples are obtained through slice sampling. The default lower limits are -100 for  $\beta$  and 0 for  $\lambda$ . The default upper limits for the parameters are 100. The default slice widths for the parameters are 0.1. The defaults may not be appropriate for all situations, and the user can specify the appropriate limits and slice width for each parameter.

### Value

Posterior samples of  $\beta$  and  $\lambda$  are returned.

### References

Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.

Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.

Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

### See Also

[power.phm.random.a0](#) and [approximate.prior.beta](#)

### Examples

```
set.seed(1)
# Simulate current data
n <- 50
P <- 4
time <- round(rexp(n, rate=0.5),1)
event <- rep(1,n)
X <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S <- c(rep(1,n/2),rep(2,n/2))

# Simulate two historical datasets
n <- 100
time1 <- round(rexp(n, rate=0.5),1)
event1 <- rep(1,n)
X1 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S1 <- c(rep(1,n/2),rep(2,n/2))
time2 <- round(rexp(n, rate=0.7),1)
event2 <- rep(1,n)
X2 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S2 <- c(rep(1,n/2),rep(2,n/2))
historical <- list(list(time=time1, event=event1, X=X1, S=S1),
                  list(time=time2, event=event2, X=X2, S=S2))
```

```

# We choose three intervals for the first stratum and two intervals for the second stratum
n.intervals <- c(3,2)
change.points <- list(c(1,2), 2)

# Get samples from the approximate normalized power prior for beta
nMC <- 100 # nMC should be larger in practice
nBI <- 50
prior.beta <- approximate.prior.beta(historical, n.intervals, change.points=change.points,
                                     prior.a0.shape1=c(1,1), prior.a0.shape2=c(1,1),
                                     nMC=nMC, nBI=nBI)

prior_beta_mu=colMeans(prior.beta)
prior_beta_sigma=cov(prior.beta)

# Approximate the discrete sames with a single multivariate normal with weight one
prior.beta.mvn <- list(list(prior_beta_mu, prior_beta_sigma, 1))

result <- phm.random.a0(time=time, event=event, X=X, S=S,
                       historical=historical, n.intervals=n.intervals,
                       change.points=change.points,
                       prior.beta.mvn=prior.beta.mvn,
                       nMC=nMC, nBI=nBI)

# posterior mean of beta
colMeans(result$beta_samples)
# posterior mean of baseline hazards for stratum 1
colMeans(result$lambda_samples[[1]])
# posterior mean of baseline hazards for stratum 2
colMeans(result$lambda_samples[[2]])

```

---

power.phm.fixed.a0      *Power/type I error calculation for the proportional hazards model with  
piecewise constant hazard and fixed  $a_0$*

---

### Description

Power/type I error calculation using power priors for the proportional hazards model with piecewise constant hazard and fixed  $a_0$

### Usage

```

power.phm.fixed.a0(
  historical,
  a0,
  n.subjects,
  n.events,
  n.intervals,

```

```

change.points = NULL,
shared.blh = FALSE,
samp.prior.beta,
samp.prior.lambda,
x.samples = matrix(),
s.samples = NULL,
dist.enroll,
param.enroll,
rand.prob = 0.5,
prob.drop = 0,
param.drop = 0,
dist.csr = "Constant",
param.csr = 10000,
min.follow.up = 0,
max.follow.up = 10000,
prior.beta = "Normal",
prior.beta.mean = rep(0, 50),
prior.beta.sd = rep(1000, 50),
prior.lambda = "Gamma",
prior.lambda.hp1 = rep(10^(-5), 50),
prior.lambda.hp2 = rep(10^(-5), 50),
prior.lambda0.hp1 = rep(10^(-5), 50),
prior.lambda0.hp2 = rep(10^(-5), 50),
lower.limits = NULL,
upper.limits = rep(100, 50),
slice.widths = rep(0.1, 50),
nMC = 10000,
nBI = 250,
delta = 0,
nullspace.ineq = ">",
gamma = 0.95,
N = 10000
)

```

### Arguments

historical	List of historical dataset(s). Each historical dataset is stored in a list which contains four <i>named</i> elements: time, event, X and S. <ul style="list-style-type: none"> <li>time is a vector of follow up times.</li> <li>event is a vector of status indicators. Normally 0=alive and 1=dead.</li> <li>X is a matrix of covariates. The first column must be the treatment indicator.</li> <li>S is a vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.</li> </ul>
a0	Vector containing numbers between 0 and 1 indicating the discounting parameter value for each historical dataset. The length of the vector should be equal to the length of historical.
n.subjects	Number of subjects enrolled.



n.events	Number of events at which the trial will stop.
n.intervals	Vector of integers, indicating the number of intervals for the baseline hazards for each stratum. The length of the vector should be equal to the total number of strata.
change.points	List of vectors. Each vector in the list contains the change points for the baseline hazards for each stratum. The length of the list should be equal to the total number of strata. For a given stratum, if there is only one interval, then change.points should be NULL for that stratum. By default, we assign the change points so that the same number of events are observed in all the intervals in the historical data. These change points are used for data generation. The change points used during model fitting are assigned by default so that the same number of events are observed in all the intervals in the pooled historical and generated current data.
shared.blh	Logical value indicating whether baseline hazard parameters are shared between the current and historical data. If TRUE, baseline hazard parameters are shared. The default value is FALSE.
samp.prior.beta	Matrix of possible values of $\beta$ to sample (with replacement) from. Each row is a possible $\beta$ vector (a realization from the sampling prior for $\beta$ ).
samp.prior.lambda	List of matrices, where each matrix represents the sampling prior for the baseline hazards for each stratum. The number of columns of each matrix should be equal to the number of intervals for that stratum.
x.samples	(Only applies when there is no historical dataset) matrix of possible values of covariates from which covariate vectors are sampled with replacement.
s.samples	(Only applies when there is no historical dataset) vector of possible values of the stratum index from which the stratum indices are sampled with replacement.
dist.enroll	Distribution for enrollment times. The choices are "Uniform" or "Exponential".
param.enroll	Parameter for the distribution of enrollment times. If dist.enroll is "Uniform", the enrollment times follow Unif(0, param.enroll). If dist.enroll is "Exponential", the enrollment times follow Exponential(rate=param.enroll).
rand.prob	Randomization probability for the treated group. The default value is 0.5.
prob.drop	Probability of subjects dropping out of the study (non-administrative censoring). The default value is zero.
param.drop	Parameter for dropout time simulations. The dropout times follow Unif(0, param.drop). The default value is zero.
dist.csr	Distribution for (administrative) censorship times. The choices are "Uniform", "Constant" and "Exponential". The default choice is "Constant".
param.csr	Parameter for the (administrative) censorship times. If dist.csr is "Uniform", the censorship times follow Unif(0, param.csr). If dist.csr is "Constant", the censorship times of all subjects are equal to param.csr. If dist.csr is "Exponential", the censorship times follow Exponential(rate=param.csr). The default value is $10^4$ .
min.follow.up	Minimum amount of time for which subjects are followed up. The default value is zero.

<code>max.follow.up</code>	Maximum amount of time for which subjects are followed up. The default value is $10^4$ .
<code>prior.beta</code>	Prior used for $\beta$ . The choices are "Uniform" and "Normal". If <code>prior.beta</code> is "Uniform", the uniform improper prior is used. If <code>prior.beta</code> is "Normal", independent normal priors are used for each element of $\beta$ . The default choice is "Normal".
<code>prior.beta.mean</code>	(Only applies if <code>prior.beta</code> is "Normal") vector of means of the normal prior on $\beta$ . The default value is zero for all the elements of $\beta$ .
<code>prior.beta.sd</code>	(Only applies if <code>prior.beta</code> is "Normal") vector of standard deviations of the normal prior on $\beta$ . The default value is $10^3$ for all the elements of $\beta$ .
<code>prior.lambda</code>	Prior used for $\lambda$ . The choices are "Gamma", "Log-normal" and "Improper". The default choice is "Gamma". If <code>prior.lambda</code> is "Gamma", then the prior on the first element of $\lambda$ is $\text{Gamma}(\text{shape}=\text{prior.lambda.hp1}[1], \text{rate}=\text{prior.lambda.hp2}[1])$ . If <code>prior.lambda</code> is "Log-normal", then the prior on the first element of $\lambda$ is $\text{Log-normal}(\text{mean}=\text{prior.lambda.hp1}[1], \text{sd}=\text{prior.lambda.hp2}[1])$ . If <code>prior.lambda</code> is "Improper", then the prior on each element of $\lambda$ is the improper prior $\lambda^{-1}$ .
<code>prior.lambda.hp1</code>	(Only applies if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of first hyperparameters of the prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>prior.lambda.hp2</code>	(Only applies if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of second hyperparameters of the prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>prior.lambda0.hp1</code>	(Only applies if <code>shared.blh</code> is FALSE and if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of first hyperparameters of the prior on $\lambda_0$ . We assume the same distribution choice for the prior for $\lambda_0$ and $\lambda$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
<code>prior.lambda0.hp2</code>	(Only applies if <code>shared.blh</code> is FALSE and if <code>prior.lambda</code> is "Gamma" or "Log-normal") Vector of second hyperparameters of the prior on $\lambda_0$ . We assume the same distribution choice for the prior for $\lambda_0$ and $\lambda$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
<code>lower.limits</code>	Vector of lower limits for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is -100 for $\beta$ and 0 for $\lambda$ and $\lambda_0$ (may not be appropriate for all situations).

upper.limits	Vector of upper limits for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 100 for all parameters (may not be appropriate for all situations).
slice.widths	Vector of initial slice widths for parameters ( $\beta$ , $\lambda$ , and $\lambda_0$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 0.1 for all parameters (may not be appropriate for all situations).
nMC	Number of iterations (excluding burn-in samples) for the slice sampler. The default is 10,000.
nBI	Number of burn-in samples for the slice sampler. The default is 250.
delta	Prespecified constant that defines the boundary of the null hypothesis. The default is zero.
nullspace.ineq	Character string specifying the inequality of the null hypothesis. The options are ">" and "<". If ">" is specified, the null hypothesis is $H_0: \beta_1 \geq \delta$ . If "<" is specified, the null hypothesis is $H_0: \beta_1 \leq \delta$ . The default choice is ">".
gamma	Posterior probability threshold for rejecting the null. The null hypothesis is rejected if posterior probability is greater gamma. The default is 0.95.
N	Number of simulated datasets to generate. The default is 10,000.

## Details

The proportional hazards model with piecewise constant hazard is implemented. We assume  $\beta$  is the regression coefficients. We assume the first column of the covariate matrix is the treatment indicator, and the corresponding parameter is  $\beta_1$ . The baseline hazards of the current data are denoted by  $\lambda$ . The baseline hazards of the historical data are denoted by  $\lambda_0$ . If the baseline hazards are shared between the historical and current data, then  $\lambda_0 = \lambda$ .

To perform sample size determination, we test the hypotheses

$$H_0 : \beta_1 \geq \delta$$

and

$$H_1 : \beta_1 < \delta.$$

If historical datasets are provided, the algorithm samples with replacement from the historical covariates to construct the simulated datasets. Otherwise, the algorithm samples with replacement from `x.samples`. One of the arguments `historical` and `x.samples` must be provided.

The sampling prior for the treatment parameter can be generated from a normal distribution (see examples). For example, suppose one wants to compute the power for the hypotheses  $H_0 : \beta_1 \geq 0$  and  $H_1 : \beta_1 < 0$ . To approximate the sampling prior for  $\beta_1$ , one can simply sample from a normal distribution with negative mean, so that the mass of the prior falls in the alternative space. Conversely, to compute the type I error rate, one can sample from a normal distribution with positive mean, so that the mass of the prior falls in the null space.

The sampling prior for the other parameters ( $\beta_2, \dots, \beta_p, \lambda$  and  $\lambda_0$ ) can be generated from the posterior based on the historical data. This can be achieved by the function `phm.fixed.a0` with `current.data` set to `FALSE` (see the vignette).

Posterior samples are obtained through slice sampling. The default lower limits are -100 for  $\beta$  and 0 for  $\lambda$  and  $\lambda_0$ . The default upper limits for the parameters are 100. The default slice widths for the parameters are 0.1. The defaults may not be appropriate for all situations, and the user can specify the appropriate limits and slice width for each parameter.

If a sampling prior with support in the null space is used, the value returned is a Bayesian type I error rate. If a sampling prior with support in the alternative space is used, the value returned is a Bayesian power.

### Value

Power or type I error is returned, depending on the sampling prior used. The posterior probabilities of the alternative hypothesis are returned. The average posterior means of  $\beta$ ,  $\lambda$  and  $\lambda_0$  (if the baseline hazard parameters are not shared) are also returned.

### References

Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.

Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.

Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

### See Also

[phm.fixed.a0](#)

### Examples

```
# Simulate two historical datasets
set.seed(1)
n <- 100
P <- 4
time1 <- round(rexp(n, rate=0.5),1)
event1 <- rep(1,n)
X1 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S1 <- c(rep(1,n/2),rep(2,n/2))
time2 <- round(rexp(n, rate=0.7),1)
event2 <- rep(1,n)
X2 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S2 <- c(rep(1,n/2),rep(2,n/2))
historical <- list(list(time=time1, event=event1, X=X1, S=S1),
                  list(time=time2, event=event2, X=X2, S=S2))

# a0 is 0.3 for the first historical dataset and 0.6 for the second
a0 <- c(0.3, 0.6)

n.subjects <- 100
```

```

n.events <- 30

# We choose three intervals for the first stratum and two intervals for the second stratum
n.intervals <- c(3,2)
change.points <- list(c(1,2),1)

# Generate sampling priors

# The null hypothesis here is H0: beta_1 >= 0. To calculate power,
# we can provide samples of beta_1 such that the mass of beta_1 < 0.
# To calculate type I error, we can provide samples of beta_1 such that
# the mass of beta_1 >= 0.
samp.prior.beta1 <- rnorm(100, mean=-1, sd=1)
# Here, mass is put on the alternative region, so power is calculated.
samp.prior.beta <- cbind(samp.prior.beta1, matrix(rnorm(100*(P-1)), 100, P-1))

# Point mass sampling priors are used for lambda
lambda_strat1 <- matrix(c(0.5, 0.5, 0.5), nrow=1)
lambda_strat2 <- matrix(c(0.7, 0.7), nrow=1)
samp.prior.lambda <- list(lambda_strat1, lambda_strat2)

nMC <- 100 # nMC should be larger in practice
nBI <- 50
N <- 5 # N should be larger in practice

result <- power.phm.fixed.a0(historical=historical, a0=a0, n.subjects=n.subjects,
                             n.events=n.events, n.intervals=n.intervals,
                             change.points=change.points,
                             samp.prior.beta=samp.prior.beta,
                             samp.prior.lambda=samp.prior.lambda,
                             dist.enroll="Uniform", param.enroll=0.5,
                             nMC=nMC, nBI=nBI, delta=0, nullspace.ineq=">", N=N)

result$`power/type I error`
result$`average posterior mean of beta`
result$`average posterior mean of lambda`
result$`average posterior mean of lambda0`

```

---

power.phm.random.a0      *Power/type I error calculation for the proportional hazards model with  
piecewise constant hazard and random a0*

---

### Description

Power/type I error calculation using the normalized power prior for the proportional hazards model with piecewise constant hazard and random  $a_0$

**Usage**

```
power.phm.random.a0(
  historical,
  n.subjects,
  n.events,
  n.intervals,
  change.points = NULL,
  samp.prior.beta,
  samp.prior.lambda,
  dist.enroll,
  param.enroll,
  rand.prob = 0.5,
  prob.drop = 0,
  param.drop = 0,
  dist.csr = "Constant",
  param.csr = 10000,
  min.follow.up = 0,
  max.follow.up = 10000,
  prior.beta.mvn = NULL,
  prior.beta.mean = rep(0, 50),
  prior.beta.sd = rep(1000, 50),
  prior.lambda.hp1 = rep(10^(-5), 50),
  prior.lambda.hp2 = rep(10^(-5), 50),
  prior.a0.shape1 = rep(1, 10),
  prior.a0.shape2 = rep(1, 10),
  prior.lambda.hp1 = rep(10^(-5), 50),
  prior.lambda.hp2 = rep(10^(-5), 50),
  lower.limits = NULL,
  upper.limits = rep(100, 50),
  slice.widths = rep(0.1, 50),
  nMC = 10000,
  nBI = 250,
  delta = 0,
  nullspace.ineq = ">",
  gamma = 0.95,
  N = 10000
)
```

**Arguments**

**historical** List of historical dataset(s). Each historical dataset is stored in a list which contains four *named* elements: time, event, X and S.

- time is a vector of follow up times.
- event is a vector of status indicators. Normally 0=alive and 1=dead.
- X is a matrix of covariates. The first column must be the treatment indicator.
- S is a vector of integers, where each integer represents the stratum that the subject belongs to. For example, if there are three strata, S can take values 1, 2 or 3.

n.subjects	Number of subjects enrolled.
n.events	Number of events at which the trial will stop.
n.intervals	Vector of integers, indicating the number of intervals for the baseline hazards for each stratum. The length of the vector should be equal to the total number of strata.
change.points	List of vectors. Each vector in the list contains the change points for the baseline hazards for each stratum. The length of the list should be equal to the total number of strata. For a given stratum, if there is only one interval, then change.points should be NULL for that stratum. By default, we assign the change points so that the same number of events are observed in all the intervals in the historical data. These change points are used for data generation. The change points used during model fitting are assigned by default so that the same number of events are observed in all the intervals in the pooled historical and generated current data.
samp.prior.beta	Matrix of possible values of $\beta$ to sample (with replacement) from. Each row is a possible $\beta$ vector (a realization from the sampling prior for $\beta$ ).
samp.prior.lambda	List of matrices, where each matrix represents the sampling prior for the baseline hazards for each stratum. The number of columns of each matrix should be equal to the number of intervals for that stratum.
dist.enroll	Distribution for enrollment times. The choices are "Uniform" or "Exponential".
param.enroll	Parameter for the distribution of enrollment times. If dist.enroll is "Uniform", the enrollment times follow Unif(0, param.enroll). If dist.enroll is "Exponential", the enrollment times follow Exponential(rate=param.enroll).
rand.prob	Randomization probability for the treated group. The default value is 0.5.
prob.drop	Probability of subjects dropping out of the study (non-administrative censoring). The default value is zero.
param.drop	Parameter for dropout time simulations. The dropout times follow Unif(0, param.drop). The default value is zero.
dist.csr	Distribution for (administrative) censorship times. The choices are "Uniform", "Constant" and "Exponential". The default choice is "Constant".
param.csr	Parameter for the (administrative) censorship times. If dist.csr is "Uniform", the censorship times follow Unif(0, param.csr). If dist.csr is "Constant", the censorship times of all subjects are equal to param.csr. If dist.csr is "Exponential", the censorship times follow Exponential(rate=param.csr). The default value is $10^4$ .
min.follow.up	Minimum amount of time for which subjects are followed up. The default value is zero.
max.follow.up	Maximum amount of time for which subjects are followed up. The default value is $10^4$ .
prior.beta.mvn	List of multivariate normal approximations of the normalized power prior for $\beta$ . Each element in the list is a list with three components, the mean vector, the covariance matrix and the weight of the multivariate normal distribution. The normalized power prior for $\beta$ is approximated by the weighted mixture of the multivariate normal distributions provided. By default (prior.beta.mvn=NULL),

a single multivariate normal distribution is assumed. The user can use the `approximate.prior.beta` function to obtain samples of  $\beta$  from the normalized power prior, and use any mixture of multivariate normals to approximate the normalized power prior for  $\beta$ .

<code>prior.beta.mean</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of means of the normal initial prior on $\beta$ . The default value is zero for all the elements of $\beta$ .
<code>prior.beta.sd</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of standard deviations of the normal initial prior on $\beta$ . The default value is $10^3$ for all the elements of $\beta$ .
<code>prior.lambda0.hp1</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of first hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
<code>prior.lambda0.hp2</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of second hyperparameters of the Gamma prior on $\lambda_0$ . The length of the vector should be equal to the dimension of $\lambda_0$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda_0$ .
<code>prior.a0.shape1</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of the first shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
<code>prior.a0.shape2</code>	(Only applies if <code>prior.beta.mvn=NULL</code> ) Vector of the second shape parameters of the independent beta priors for $a_0$ . The length of the vector should be equal to the number of historical datasets. The default is a vector of one's.
<code>prior.lambda.hp1</code>	Vector of first hyperparameters of the Gamma prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>prior.lambda.hp2</code>	Vector of second hyperparameters of the Gamma prior on $\lambda$ . The length of the vector should be equal to the dimension of $\lambda$ , i.e., the total number of intervals for all strata. The default value is $10^{(-5)}$ for all the elements of $\lambda$ .
<code>lower.limits</code>	Vector of lower limits for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is -100 for $\beta$ and 0 for $\lambda$ (may not be appropriate for all situations).
<code>upper.limits</code>	Vector of upper limits for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 100 for all parameters (may not be appropriate for all situations).
<code>slice.widths</code>	Vector of initial slice widths for parameters ( $\beta$ and $\lambda$ , in this order) to be used by the slice sampler. The length of the vector should be equal to the total number of parameters. The default is 0.1 for all parameters (may not be appropriate for all situations).



nMC	Number of iterations (excluding burn-in samples) for the slice sampler. The default is 10,000.
nBI	Number of burn-in samples for the slice sampler. The default is 250.
delta	Prespecified constant that defines the boundary of the null hypothesis. The default is zero.
nullspace.ineq	Character string specifying the inequality of the null hypothesis. The options are ">" and "<". If ">" is specified, the null hypothesis is $H_0: \beta_1 \geq \delta$ . If "<" is specified, the null hypothesis is $H_0: \beta_1 \leq \delta$ . The default choice is ">".
gamma	Posterior probability threshold for rejecting the null. The null hypothesis is rejected if posterior probability is greater gamma. The default is 0.95.
N	Number of simulated datasets to generate. The default is 10,000.

## Details

The proportional hazards model with piecewise constant hazard is implemented. We assume  $\beta$  is the regression coefficients. We assume the first column of the covariate matrix is the treatment indicator, and the corresponding parameter is  $\beta_1$ . Here  $a_0$  is modeled as random with a normalized power prior.

The normalized power prior for  $\beta$  is approximated by a weighted mixture of multivariate normal distributions provided in `prior.beta.mvn`. The user can use the `approximate.prior.beta` function to obtain samples of  $\beta$  from the normalized power prior, and use any mixture of multivariate normals to approximate the normalized power prior for  $\beta$ . By default, a single multivariate normal distribution is assumed.

Baseline hazard parameters for the current and historical data are NOT shared. The baseline hazards of the current data are denoted by  $\lambda$ . The baseline hazards of the historical data are denoted by  $\lambda_0$ . We assume Gamma priors for  $\lambda$  and  $\lambda_0$ .

To perform sample size determination, we test the hypotheses

$$H_0 : \beta_1 \geq \delta$$

and

$$H_1 : \beta_1 < \delta.$$

The sampling prior for the treatment parameter can be generated from a normal distribution (see examples). For example, suppose one wants to compute the power for the hypotheses  $H_0 : \beta_1 \geq 0$  and  $H_1 : \beta_1 < 0$ . To approximate the sampling prior for  $\beta_1$ , one can simply sample from a normal distribution with negative mean, so that the mass of the prior falls in the alternative space. Conversely, to compute the type I error rate, one can sample from a normal distribution with positive mean, so that the mass of the prior falls in the null space.

The sampling prior for the other parameters ( $\beta_2, \dots, \beta_p$  and  $\lambda$ ) can be generated from the posterior based on the historical data. This can be achieved by the function `phm.fixed.a0` with `current.data` set to FALSE (see the vignette).

Posterior samples are obtained through slice sampling. The default lower limits are -100 for  $\beta$  and 0 for  $\lambda$ . The default upper limits for the parameters are 100. The default slice widths for the parameters are 0.1. The defaults may not be appropriate for all situations, and the user can specify the appropriate limits and slice width for each parameter.

If a sampling prior with support in the null space is used, the value returned is a Bayesian type I error rate. If a sampling prior with support in the alternative space is used, the value returned is a Bayesian power.

### Value

Power or type I error is returned, depending on the sampling prior used. The posterior probabilities of the alternative hypothesis are returned. The average posterior means of  $\beta$  and  $\lambda$  are also returned.

### References

Ibrahim, J. G., Chen, M.-H. and Sinha, D. (2001). Bayesian Survival Analysis. New York: Springer Science & Business Media.

Psioda, M. A. and Ibrahim, J. G. (2019). Bayesian clinical trial design using historical data that inform the treatment effect. *Biostatistics* 20, 400–415.

Shen, Y., Psioda, M. A., and Joseph, J. G. (2023). BayesPPD: an R package for Bayesian sample size determination using the power and normalized power prior for generalized linear models. *The R Journal*, 14(4).

### See Also

[phm.random.a0](#) and [approximate.prior.beta](#)

### Examples

```
# Simulate two historical datasets
set.seed(1)
n <- 100
P <- 4
time1 <- round(rexp(n, rate=0.5),1)
event1 <- rep(1,n)
X1 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S1 <- c(rep(1,n/2),rep(2,n/2))
time2 <- round(rexp(n, rate=0.7),1)
event2 <- rep(1,n)
X2 <- matrix(rbinom(n*P,prob=0.5,size=1), ncol=P)
S2 <- c(rep(1,n/2),rep(2,n/2))
historical <- list(list(time=time1, event=event1, X=X1, S=S1),
                  list(time=time2, event=event2, X=X2, S=S2))

n.subjects <- 100
n.events <- 30

# We choose three intervals for the first stratum and two intervals for the second stratum
n.intervals <- c(3,2)
change.points <- list(c(1,2),1)

# Generate sampling priors

# The null hypothesis here is H0: beta_1 >= 0. To calculate power,
```

```

# we can provide samples of beta_1 such that the mass of beta_1 < 0.
# To calculate type I error, we can provide samples of beta_1 such that
# the mass of beta_1 >= 0.
samp.prior.beta1 <- rnorm(100, mean=-1, sd=1)
# Here, mass is put on the alternative region, so power is calculated.
samp.prior.beta <- cbind(samp.prior.beta1, matrix(rnorm(100*(P-1)), 100, P-1))

# Point mass sampling priors are used for lambda
lambda_strat1 <- matrix(c(0.5, 0.5, 0.5), nrow=1)
lambda_strat2 <- matrix(c(0.7, 0.7), nrow=1)
samp.prior.lambda <- list(lambda_strat1, lambda_strat2)

nMC <- 50 # nMC should be larger in practice
nBI <- 50
N <- 5 # N should be larger in practice

result <- power.phm.random.a0(historical=historical, n.subjects=n.subjects,
                             n.events=n.events, n.intervals=n.intervals,
                             change.points=change.points,
                             samp.prior.beta=samp.prior.beta,
                             samp.prior.lambda=samp.prior.lambda,
                             prior.a0.shape1 = c(1,1), prior.a0.shape2 = c(1,1),
                             dist.enroll="Uniform", param.enroll=0.5,
                             nMC=nMC, nBI=nBI, delta=0, nullspace.ineq=">", N=N)

result$`power/type I error`
result$`average posterior mean of beta`
result$`average posterior mean of lambda`

```

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