Case Examples

bayesDP R package

- **Analysis types**
  - Single-arm: treatment data only
  - Two-arm: treatment + control data
- **Data sources**
  - Current data
  - Historical data
- **Endpoints**
  - Binomial counts – `bdpbinomial()`
  - Normal means – `bdpnormal()`
  - Survival outcomes – `bdpsurvival()`

Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data

- Historical data: 28 events in 450 patients
  - Historical event rate of 28/450 ≈ 0.06
- New data: 8 events in 200 patients
  - Current event rate of 8/200 = 0.04

- Simply concatenating the current+historical data gives an event rate of (28+8)/(450+200) ≈ 0.06

```
### Estimate model via bayesDP using defaults
fit <- bdpbinomial(y_t  = 8,  N_t  = 200,
                   y0_t = 28, N0_t = 450)
```

Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data (cont'd)

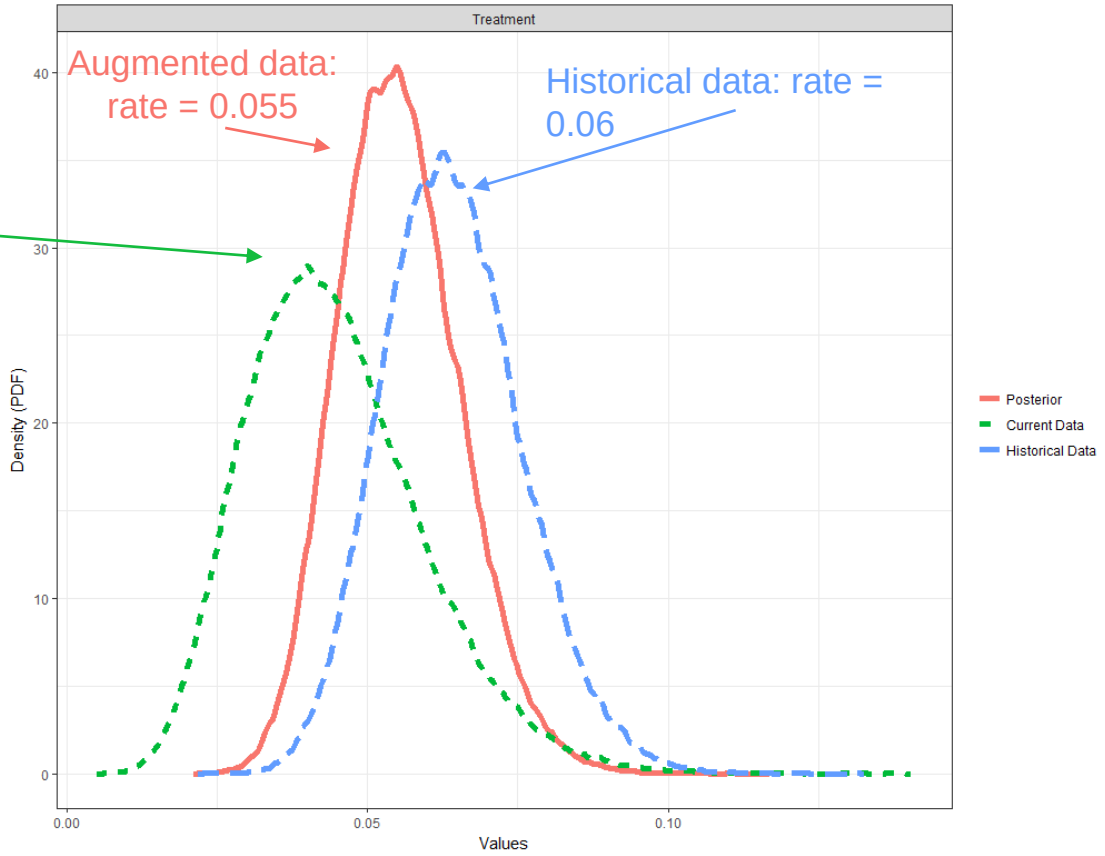
```
> summary(fit)

    One-armed bdp binomial

Current treatment data: 8 and 200
Historical treatment data: 28 and 450
Stochastic comparison (p_hat) - treatment (current vs. historical data): 0.8561
Discount function value (alpha) - treatment: 0.7021
95 percent confidence interval:
 0.0373  0.0764
augmented sample estimate:
probability of success
 0.0547
```

Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data (cont'd)

```
> plot(fit,
       type="posteriors")
```

Current data: rate = 0.04



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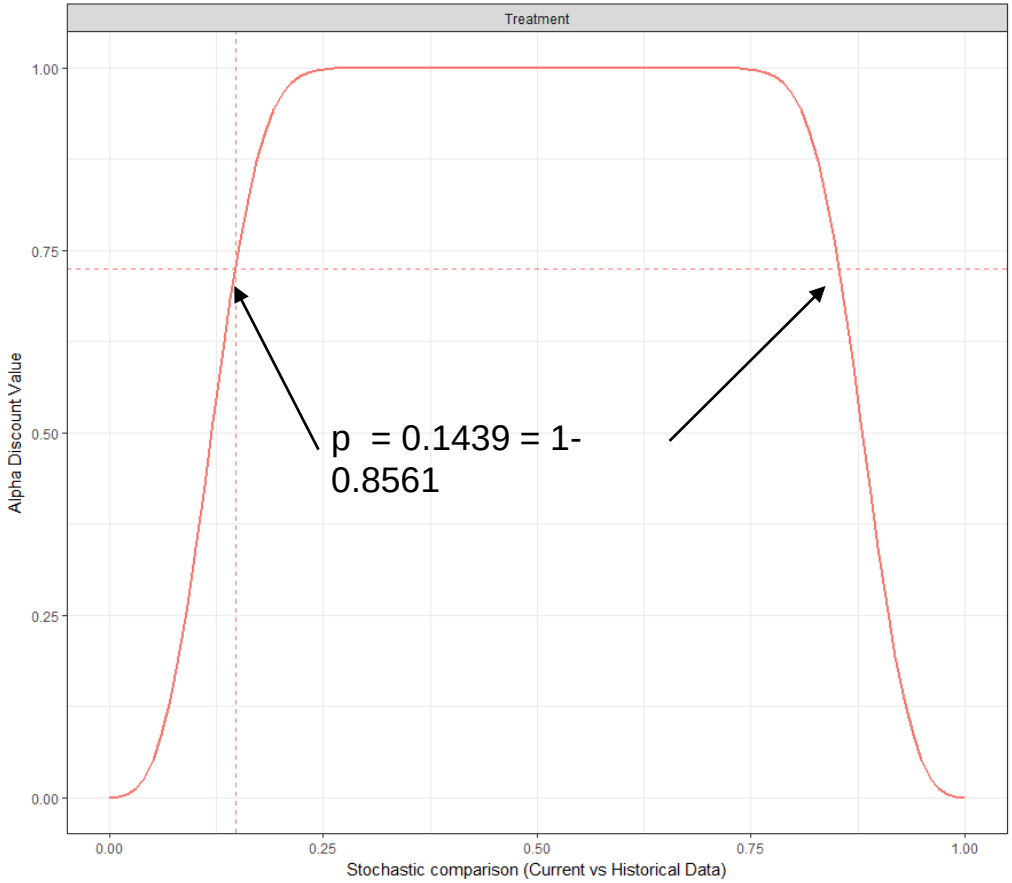
Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data (cont'd)

```
> plot(fit, type="discount")
```

Discount function details:

- Weibull CDF
  - Shape = 3
  - Scale = 0.135
  - Symmetric around 0.5



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Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data

- Fix max weight (alpha) at 0.1 → give 10% weight to the historical data

```
### Estimate model via bayesDP using defaults
fit <- bdpbinomial(y_t  = 8,  N_t  = 200,
                   y0_t = 28, N0_t = 450,
                   alpha_max = 0.1,
                   fix_alpha = TRUE)
```

Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data (cont'd)

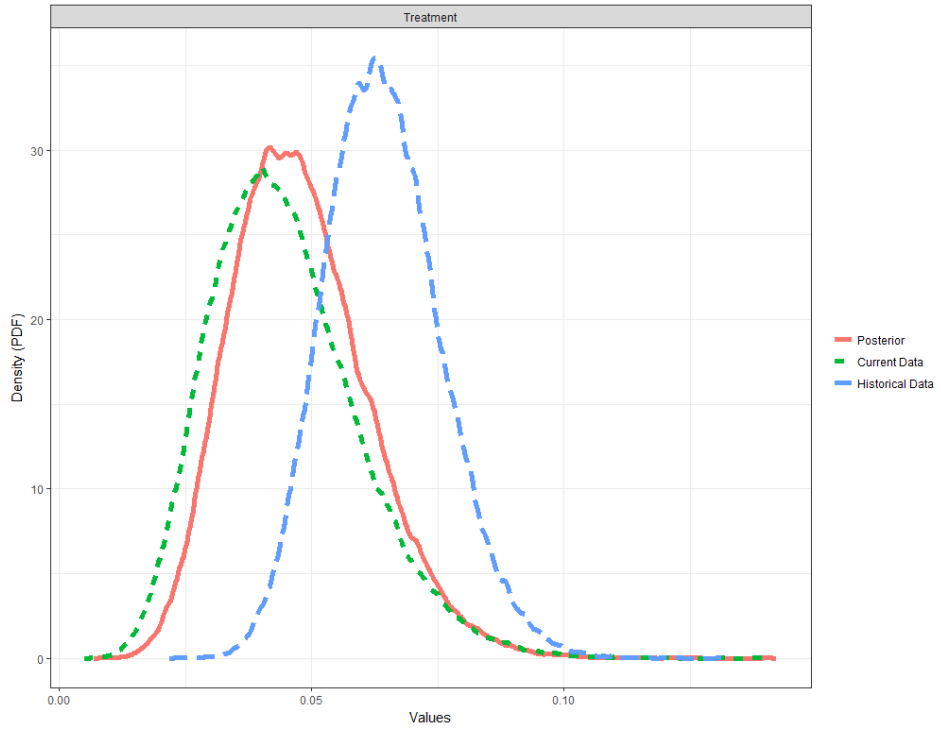
- Fix max weight (alpha) at 0.1

```
> summary(fit)

    One-armed bdp binomial

Current treatment data: 8 and 200
Historical treatment data: 28 and 450
Stochastic comparison (p_hat) - treatment (current vs. historical data): 0.8561
Discount function value (alpha) - treatment: 0.1
95 percent confidence interval:
 0.0244  0.0768
augmented sample estimate:
probability of success
 0.0462
```

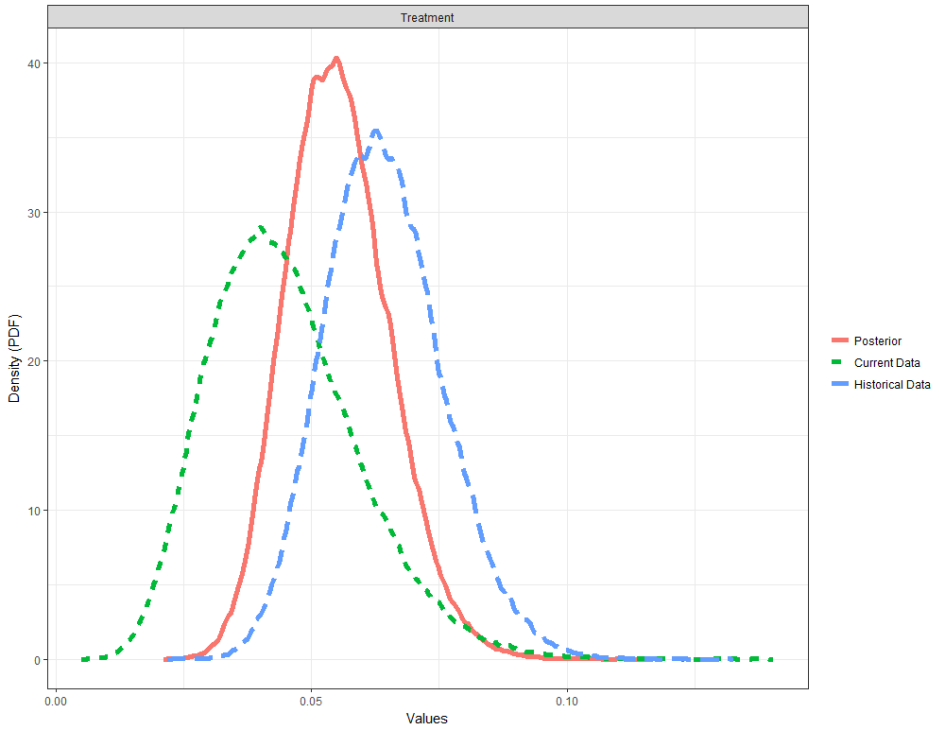
Single-Arm: Binomial Count Endpoint

Similar event rates between current and historical data (cont'd)

alpha = 0.1



alpha = 0.7021



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Two-Arm: Normal Mean Endpoint

Different means for current and historical treatment data, similar means for current and historical control data

- Historical treatment: mean = 50, sd = 5, N = 250
- Historical control: mean = 55, sd = 5, N = 250

- Current treatment: mean = 45, sd = 5, N = 250
- Current control: mean = 55, sd = 5, N = 250

```
### Estimate model via bayesDP using defaults
fit <- bdpnormal(mu_t  = 45, sigma_t  = 5, N_t  = 250,
                 mu0_t = 50, sigma0_t = 5, N0_t = 250,
                 mu_c  = 55, sigma_c  = 5, N_c  = 250,
                 mu0_c = 55, sigma0_c = 5, N0_c = 250)
```

Two-Arm: Normal Mean Endpoint

Different means for current and historical treatment data, similar means for current and historical control data (cont'd)

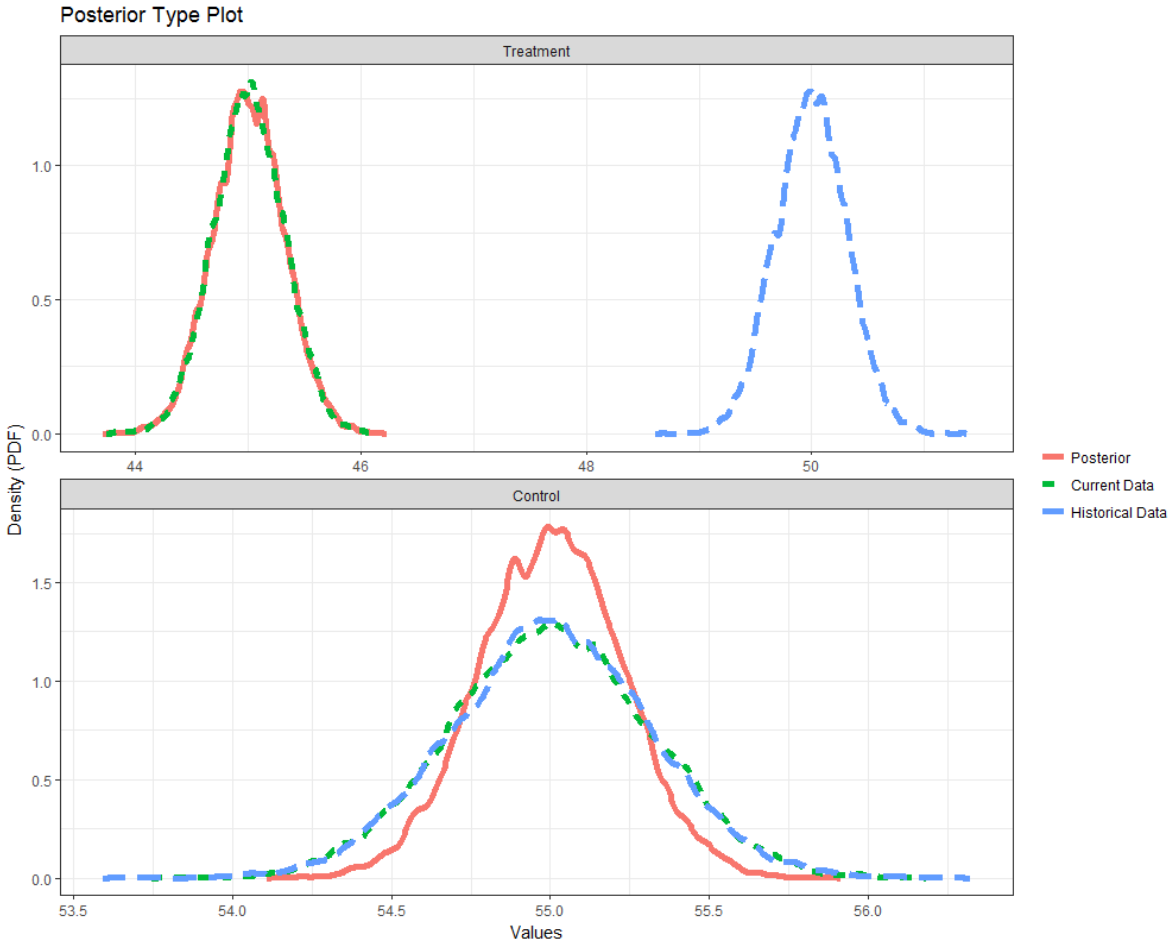
```
> summary(fit)

    Two-armed bdp normal

data:
  Current treatment: mu_t = 45, sigma_t = 5, N_t = 250
  Current control: mu_c = 55, sigma_c = 5, N_c = 250
  Historical treatment: mu0_t = 50, sigma0_t = 5, N0_t = 250
  Historical control: mu0_c = 55, sigma0_c = 5, N0_c = 250
Stochastic comparison (p_hat) - treatment (current vs. historical data): 0
Stochastic comparison (p_hat) - control (current vs. historical data): 0.4961
Discount function value (alpha) - treatment: 0
Discount function value (alpha) - control: 1
95 percent confidence interval: -10.7757  -9.2469
augmented sample estimates:
treatment group  control group
          45.00          55.00
```

Two-Arm: Normal Mean Endpoint

```
plot(fit,
     type="posteriors")
```



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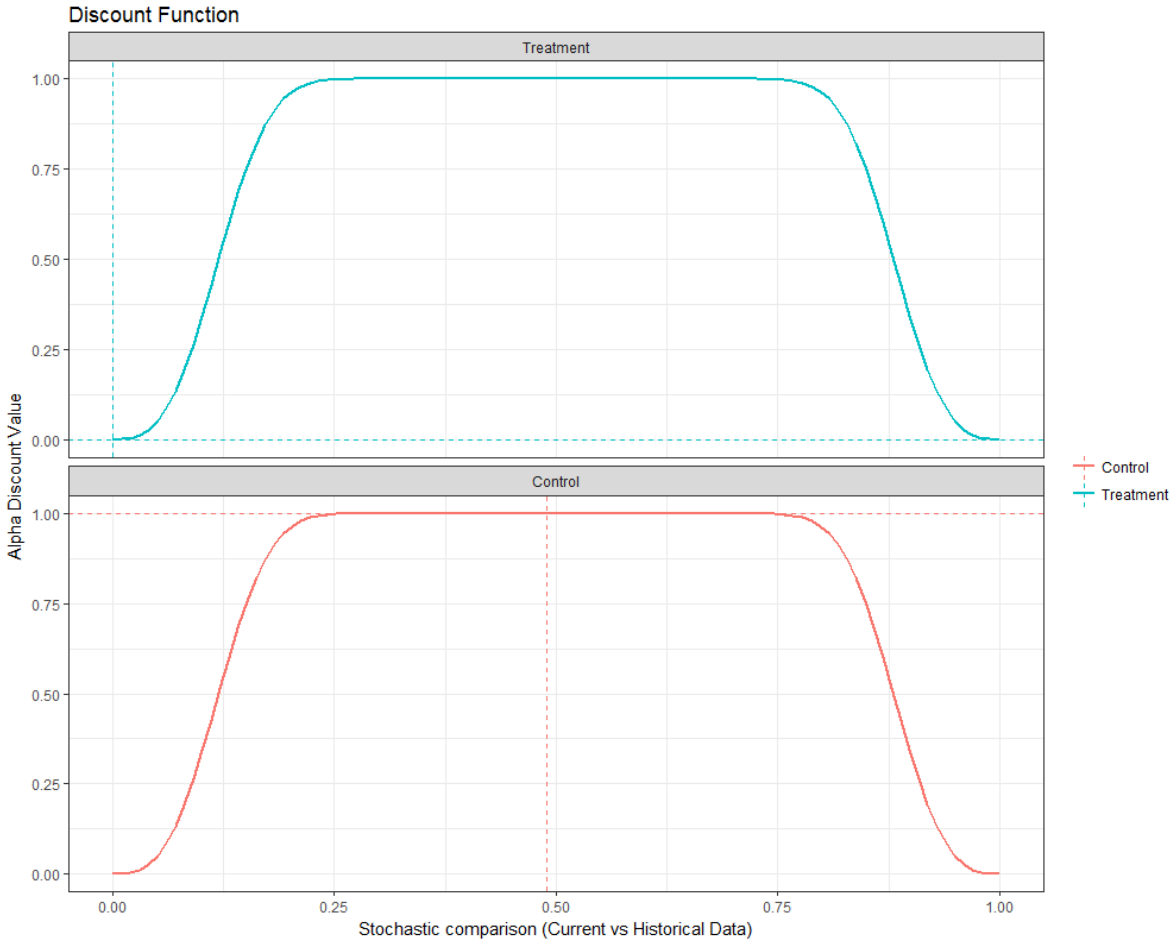
Two-Arm: Normal Mean Endpoint

```
plot(fit,
     type="discount")
```



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Single-Arm: Survival Endpoint

Different hazard rates for current and his

- Historical treatment: hazard rate of 1/1
- Current treatment: hazard rate of 1/15
  - For this example, assume no censor
- Interest lies in estimating the probability of survival at 10 years
  - Data simulated from exponential distributions; true 10 year survivals:
    - Historical treatment: 0.37
    - Current treatment: 0.51

time	status	treatment	historical
0.5	1	1	0
1.04	1	1	1
8.34	1	1	1
1.66	1	1	1
3.19	1	1	0

```
### Estimate model via bayesDP using defaults
fit <- bdpsurvival(
  Surv(time, status) ~ treatment + historical,
  data      = data_1arm,
  surv_time = 10)
```

Single-Arm: Survival Endpoint

Different hazard rates for current and historical treatment data

```
> summary(fit)

 One-armed bdp survival

Stochastic comparison (p_hat) - treatment (current vs. historical): 0.108
Discount function value (alpha) - treatment: 0.40

Current treatment - augmented posterior summary:
     time n.risk n.event survival std.err lower 95% CI upper 95% CI
   0.4322     50       1   0.9771  0.0067       0.9616       0.9877
   0.4324     49       1   0.9771  0.0067       0.9616       0.9877
   0.5729     48       1   0.9698  0.0088       0.9495       0.9837
   0.8571     47       1   0.9551  0.0129       0.9254       0.9758
   1.4422     46       1   0.9257  0.0210       0.8776       0.9596
   2.9751     45       1   0.8527  0.0397       0.7639       0.9184
   …
```

Single-Arm: Survival Endpoint

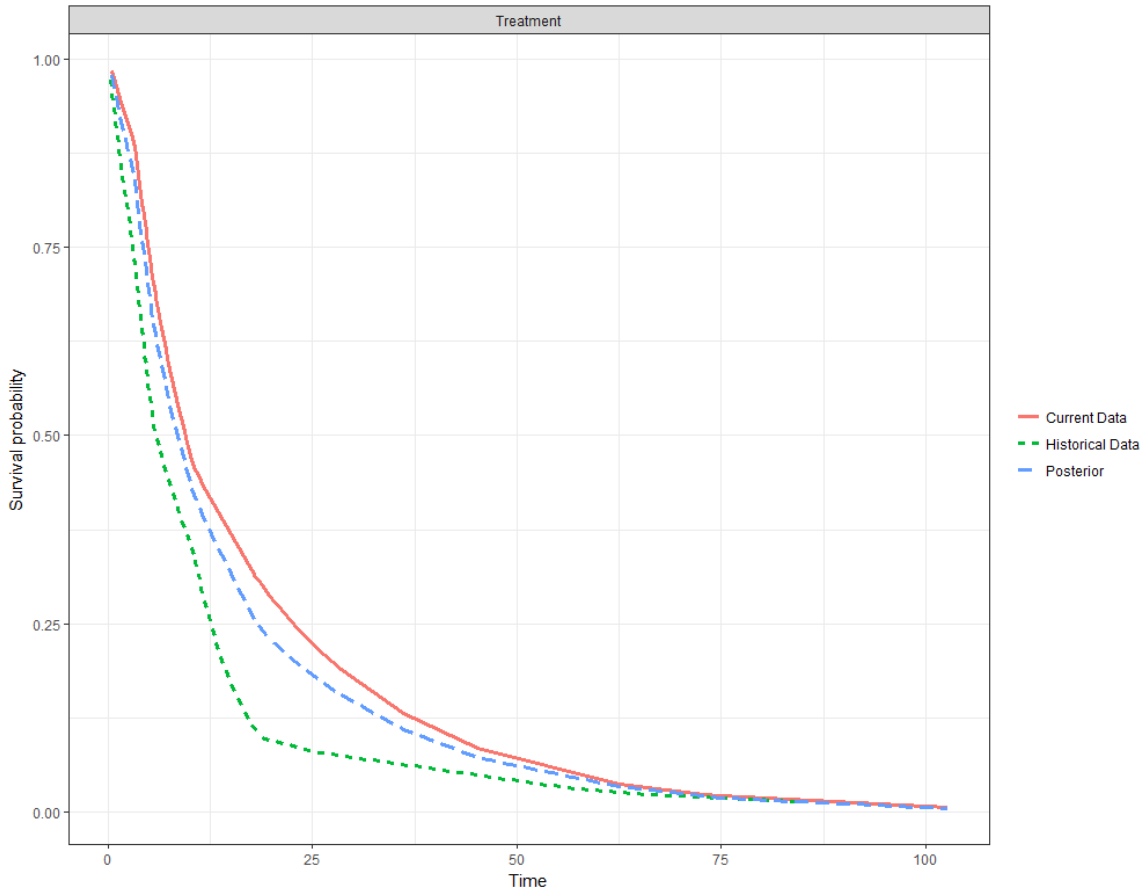
Different hazard rates for current and historical treatment data

```
> print(fit)

  One-armed bdp survival

  n events surv_time  median lower 95% CI upper 95% CI
 50     50        10   0.441       0.3321       0.5542
```

Single-Arm: Survival Endpoint

Different hazard rates for current and historical treatment data

```
> plot(fit,
       type = “survival”)
```



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Single-Arm: Survival Endpoint

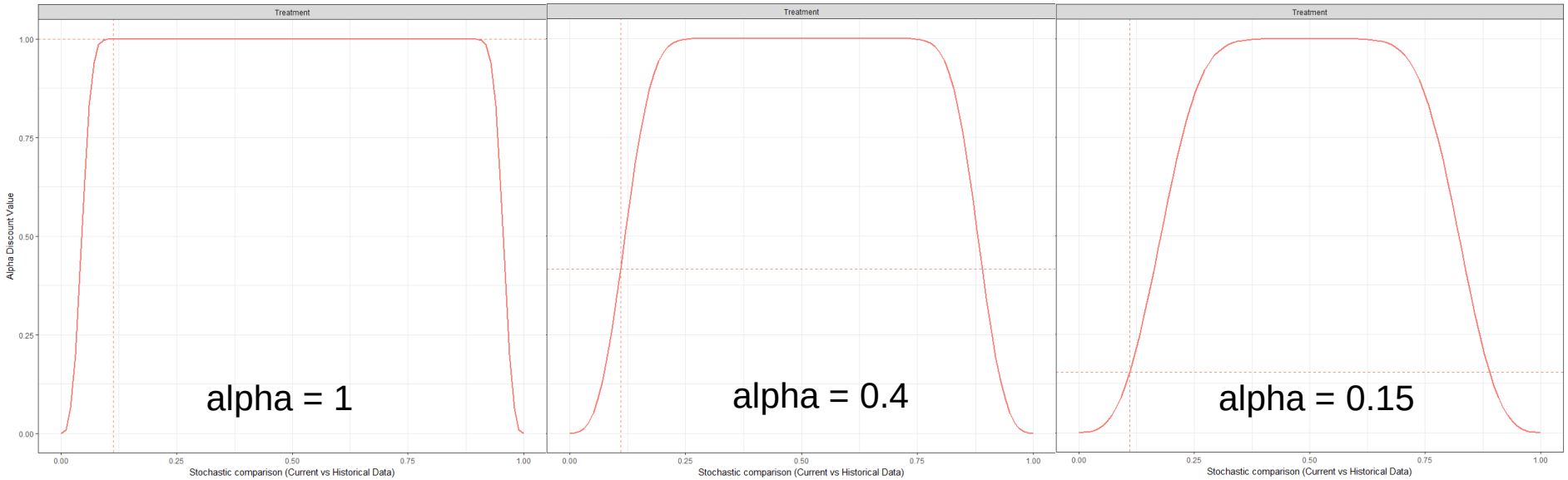
Different hazard rates for current and historical treatment data

- Change the shape of the discount function

```
bdpsurvival(...
  weibull_shape = 3,
  weibull_scale = 0.05)
```

```
bdpsurvival(...
  weibull_shape = 3,
  weibull_scale = 0.135)
```

```
bdpsurvival(...
  weibull_shape = 3,
  weibull_scale = 0.2)
```



p_hat = 0.108

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Two-Arm: Survival Endpoint

Different hazard rates urrent control only

- Same treatment da
- Current control: hazard rate of 1/12
  - Again, assume no censoring
- Interest lies in the hazard ratio comparing treatment and control

Reminder:
- Historical treatment: hazard rate of 1/10
- Current treatment: hazard rate of 1/15

```
### Estimate model via bayesDP using defaults
fit <- bdpsurvival(
  Surv(time, status) ~ treatment + historical,
  data      = data_2arm)
```

Two-Arm: Survival Endpoint

Different hazard rates for current and historical treatment data, current control only

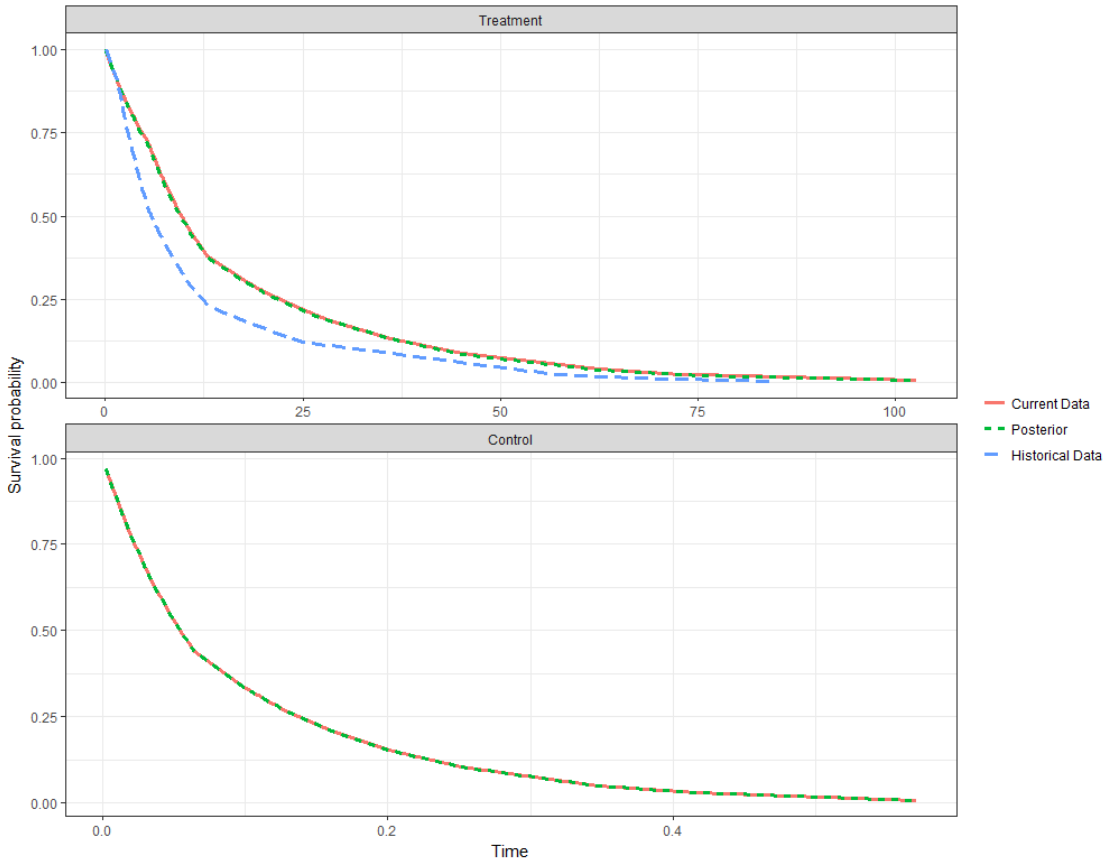
```
> summary(fit)

    Two-armed bdp survival

data:
  Current treatment: n = 50, number of events = 50
  Current control: n = 50, number of events = 50
Stochastic comparison (p_hat) - treatment (current vs. historical): 0.0421
Discount function value (alpha) - treatment: 0.0299

             coef exp(coef) se(coef) lower 95% CI upper 95% CI
treatment -4.692    0.0092   0.5125      -5.7784      -3.7525
```

Log-hazard rate comparing treatment and control

Two-Arm: Survival Endpoint

Different hazard rates for current and historical treatment data, current control only

```
> plot(fit,
       type = “survival”)
```



Coming Soon…

Additional models to be implemented:

- Negative binomial
- Regression
  - Linear
  - Logistic
  - Cox

- Further suggestions??

Contact Information

Contact info:

- Stats/technical questions: donald.r.musgrove@medtronic.com
- Coding questions: sbalcome@mdic.org

CRAN homepage: http://cran.r-project.org/web/packages/bayesDP/index.html

Getting help:

- Vignettes available at the CRAN homepage

Formal bug reporting: http://github.com/balcomes/bayesDP