# Probability of Success Using RBesT

## RShiny App Walkthrough

James Normington

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In this document, I briefly walk through a Shiny App to compute a trial's probability of success (PoS) using Sebastian Weber's RBesT package.

## Compute MAP prior



Use the first tab to specify the type of endpoint and whether or not your analysis should use historical data. If the *Use historical trial meta-data* box is checked, you can either edit the table directly or upload a .csv of data.

If you prefer to enter data into the table directly, check the *Add study to table?* checkbox, enter in the new information, and click *Add study* to add a study. To delete a study, use the *Delete study from table?* checkbox and specify the row you'd like to delete. If you choose to upload a .csv of data, change "Manual" to "Upload". Ensure your data are in the format requested by the app, then use your computer browser to upload the file.

**(3) Specify priors on τ and β.**

**Random seed (positive integer)**

> 572

**Specify prior for τ (between-trial standard deviation).**
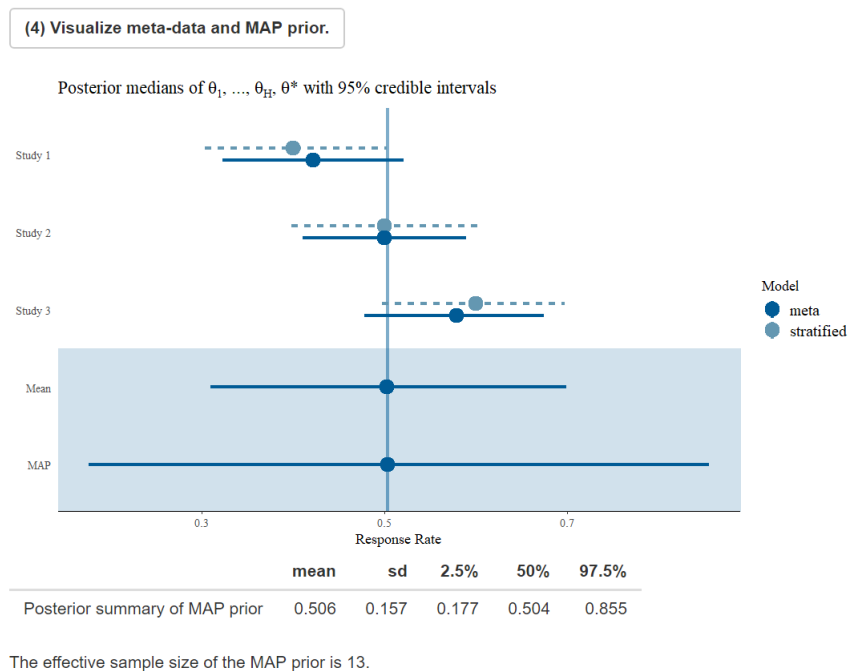
> HalfNormal ▼

τ ~ HalfNormal(σ)

σ

> 1

**Specify prior for β (common mean).**

> Default ▼

Default prior is β ~ N(0, 2)

Next, specify the random seed and hyper-priors on $\tau$ and $\beta$. A default prior for $\beta$ will be suggested to you. To ignore this and specify a different prior for $\beta$, select "Specify" from the drop down and enter in the desired prior mean and standard deviation. A mean of 0 is recommended. The prior distribution on $\beta$ must be Gaussian.

**(4) Visualize meta-data and MAP prior.**

Posterior medians of $\theta_1, ..., \theta_H, \theta^*$ with 95% credible intervals

| | mean | sd | 2.5% | 50% | 97.5% |
|---|---|---|---|---|---|
| Posterior summary of MAP prior | 0.506 | 0.157 | 0.177 | 0.504 | 0.855 |

The effective sample size of the MAP prior is 13.

To create a forest plot to visualize the meta-data and MAP prior, click the *(4) Visualize meta-data and MAP prior* button; it should take around 10 seconds for the plot to appear.

## PoS calculation



Use the second tab to specify one- or two-group trial, the observed statistics at interim, as well as the total target sample size at the end of the trial. The table will dynamically

update as you toggle between "One" and "Two" groups and ☑ and ☐ in the *Concurrent trial data at interim?* box. The table is directly editable.

The code also automatically adds a robust component (e.g., a Uniform(0,1) for binary endpoints, a diffuse Gaussian distribution for Normal endpoints) to the prior mixture distributions. The default value is 0.2. To protect against an overly informative MAP prior, enter in a higher weight (say, 0.4). To allow the MAP prior to be more informative, enter in a lower weight (say, 0.1). It is not recommended to enter in a weight higher than 0.7.

Use this section to specify the definition of trial success. You can specify one or two success criteria and a lower or upper tailed decision. As you toggle these options, the printed Decision rule(s) should dynamically update. If the trial has two groups, you can select a link function for the decision rule. For example, with binary endpoints, you can make a decision based on absolute difference in proportions (identity link), relative difference in odds (logit link), or relative difference in proportion (log link).

Enter in the null hypothesized values and critical probability threshold in accordance with the printed decision rule. Finally, click the *(3) Compute PoS!* button to compute the probability of trial success. If you are not using historical data, this should compute in less than a second. If you are using historical data but not analyzing a concurrent trial, this could take several seconds (though usually not more than one minute). If you are using historical data and using a concurrent trial, this may take a bit longer.