Effective Sample Size Using RBesT

RShiny App Walkthrough

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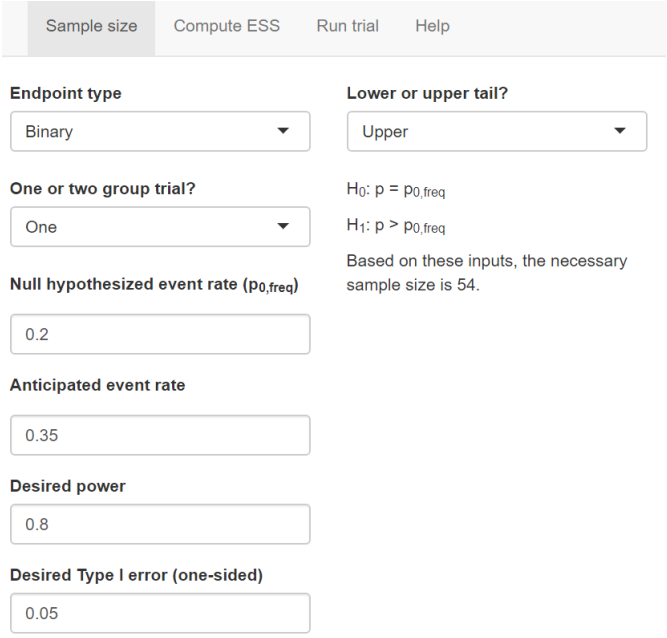
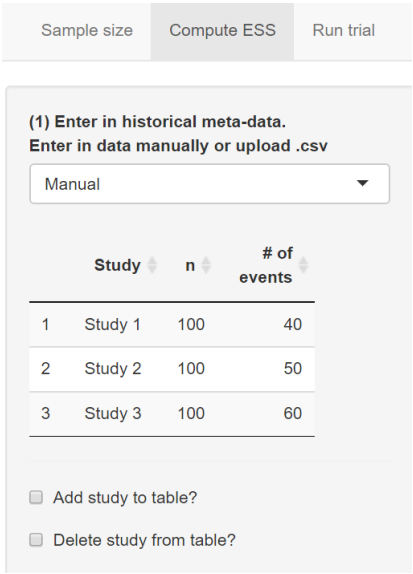
In this document, I briefly walk through a Shiny App to compute the effective sample size (ESS) using Sebastian Weber's RBesT package.

Sample size



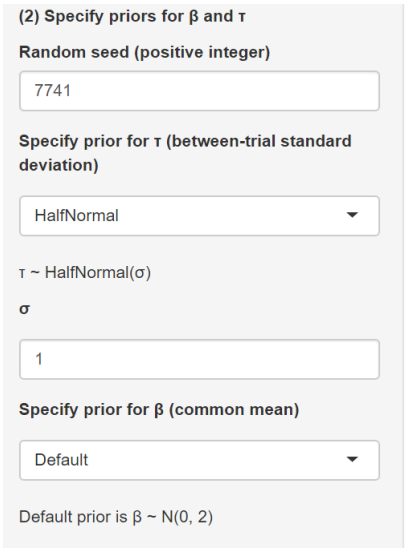
A trialist will often begin designing a trial by computing the sample size necessary to achieve a given power and Type I error rate. Because our ultimate goal is have you see the potential savings in number of patients enrolled, the first tab prompts you to make this calculation. The workflow is relatively straightforward, and both the hypotheses set and printed sample size dynamically update as you make changes to the inputs in this tab.

Compute ESS

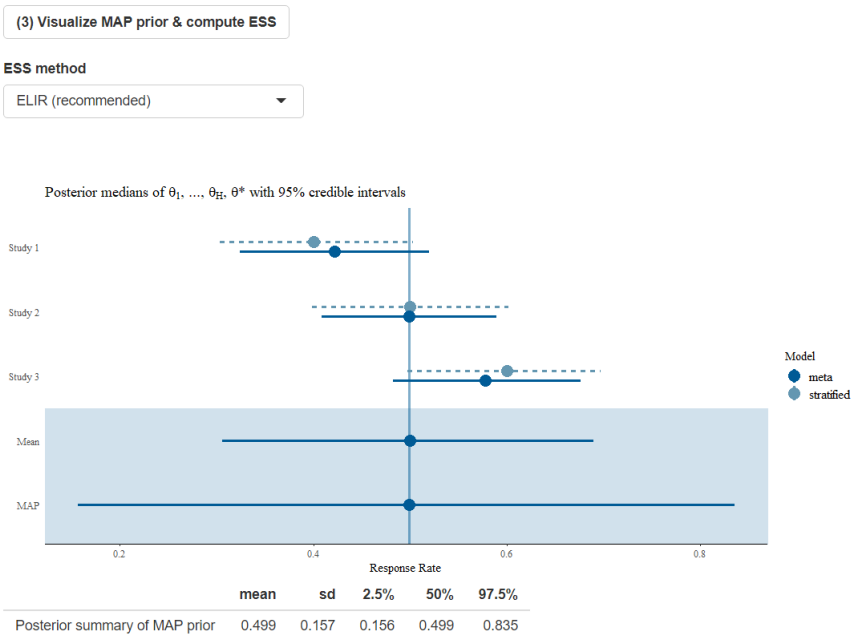


Use the first tab to specify the type of endpoint and whether or not your analysis should use historical data. If the *Use historical trial meta-data* box is checked, you can either edit the table directly or upload a .csv of data.

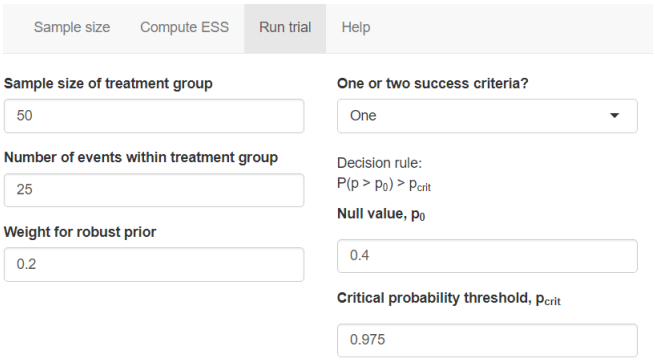
If you prefer to enter data into the table directly, check the *Add study to table?* checkbox, enter in the new information, and click *Add study* to add a study. To delete a study, use the *Delete study from table?* checkbox and specify the row you'd like to delete. If you choose to upload a .csv of 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.



Next, specify the random seed and hyper-priors on τ and β. A default prior for β will be suggested to you. To ignore this and specify a different prior for β, 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 β must be Gaussian.



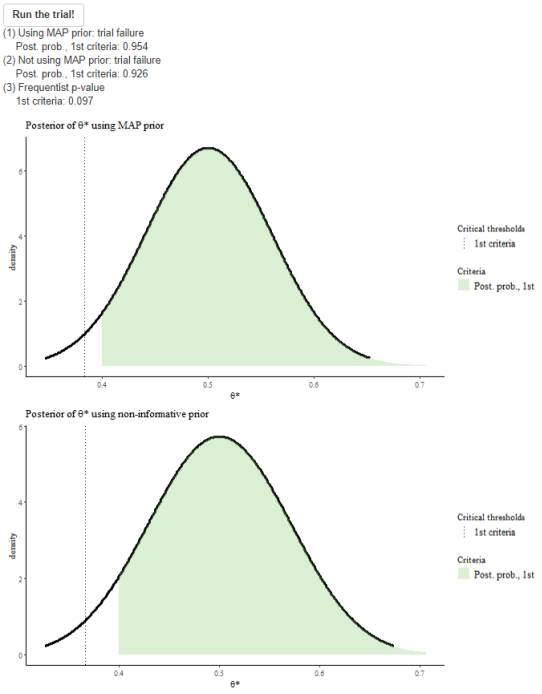
To create a forest plot to visualize the meta-data and MAP prior, click the *(3) Visualize meta-data & compute ESS* button; it should take around 10 seconds for the plot to appear. You can select an ESS method of ELIR (recommended), Moment, or Morita. See the methodology and code walkthrough for the mathematical details behind each.



Use the final tab to conduct the final trial analysis under different hypothetical scenarios. Fill in the sample size and observed statistics at final analysis. 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.

This section also asks you 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.

Run trial



Finally, click the *Run trial!* button to run the hypothetical trials. Five sets of results will output:

1. The final trial result, using the MAP prior, with posterior probabilities listed for each success criterion
2. The final trial result, using a non-informative (not MAP) prior, with posterior probabilities listed for each success criterion
3. The p-value yielded from a standard frequentist test
4. A plotted posterior distribution of the treatment effect using the MAP prior; the critical value based on the decision rule is indicated by a vertical line, and the posterior probabilities of all the success criteria are shaded in
5. A plotted posterior distribution of the treatment effect using a non-informative (not MAP) prior; the critical value based on the decision rule is indicated by a vertical line, and the posterior probabilities of all the success criteria are shaded in