

Bayesian Clinical Trials

Course introduction

Instructors

Ileana Baldi Assistant Professor of Biostatistics Dept. of Cardiac,
Thoracic and Vascular Sciences University of Padova

Paola Berchialla Assistant Professor of Biostatistics Dept of Clinical
and Biological Sciences University of Torino

Outline - part I

9.15-9.45 **Brief introduction to phase I and phase IIB trials**
both single-stage and two-stage with proportion as outcome.

9.45-10.30 **Dose-finding phase I and the CRM method**

10.30-11.00 **Real data case study**

11.15-12.00 **An introduction to the Beta-Binomial model**
binomial likelihood, beta prior, conjugacy, posterior density and
posterior predictive distribution

12.00-12.30 *Hands-on* **example** informative vs. non-informative
beta priors

Outline - part II

13.30-14.30 **Bayesian Sample Size Determination for Binomial Proportions** choose a sample size that allows one to estimate the accuracy to within a desired credible interval width (ACC, ALC, WOC) choose a sample size n for which the probability of a *successful trial* is large enough, in the sense that it exceeds a specified threshold (single threshold designs and extensions)

14.30-15.00 **Case study** to compare ACC, ALC, WOC

15.00-15.30 **Case study** to compare two-stage phase II with different priors vs. Simon

15.45-16.30 **Insight into building priors**

16.30-17.30 “Our” design???

Pre-requisites

- ▶ There are no formal pre-requisites
- ▶ Analysis will be performed in the R programming language:
<http://www.r-project.org/>
- ▶ Rstudio <http://www.rstudio.com/products/rstudio/> is recommended
- ▶ The following R package will be used in class:
 - ▶ SampleSizeProportions
 - ▶ LearnBayes
 - ▶ shiny
 - ▶ BRugs

Getting the slides

- ▶ The slides for this course were created with Rmarkdown:
<http://rmarkdown.rstudio.com/>.
- ▶ They are available from
<https://github.com/berkeley3/bayesianCT-course>.
- ▶ To re-compile the slides:
 - ▶ Download the directory containing the lecture from Github
 - ▶ In R open the .Rmd file and set the working directory to the lecture directory
 - ▶ Click the *KnitHTML* button on Rstudio or run the following commands:

```
library(rmarkdown)
render("index.Rmd")
```