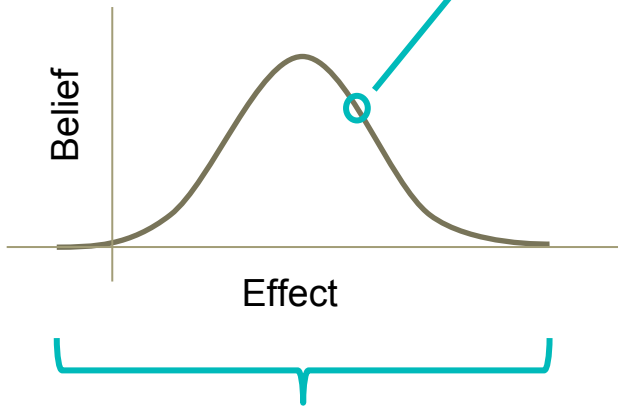
Assurance to estimate the Probability of clinical Success

Statistical power:

The probability of a significant p-value if the effect of the drug is one specific value

Criterion of success



Clinically relevant effect? Secondary endpoints?

Assurance:

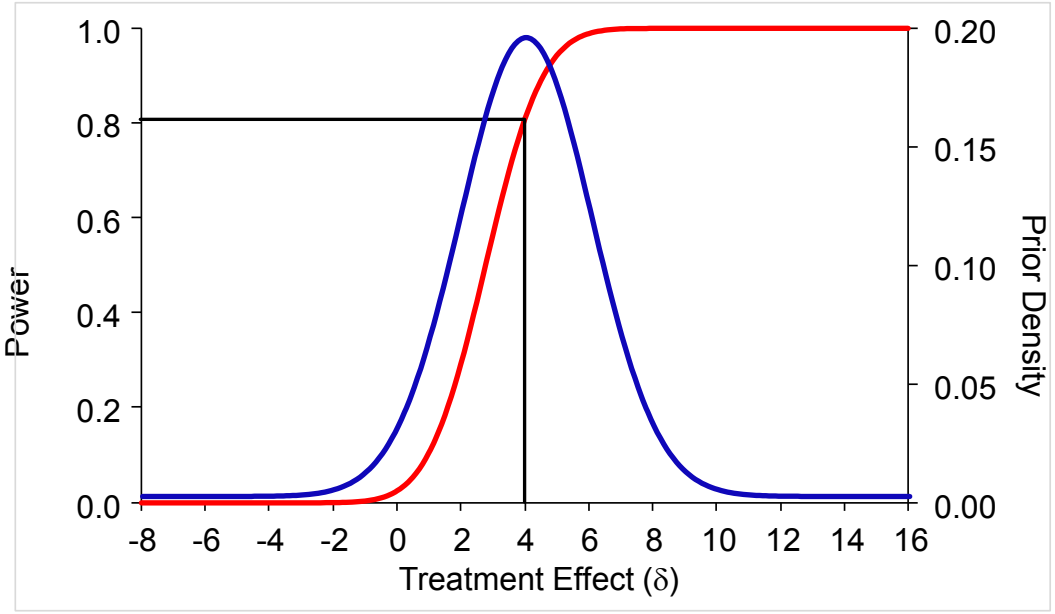
A weighted average power with more elaborate success criteria

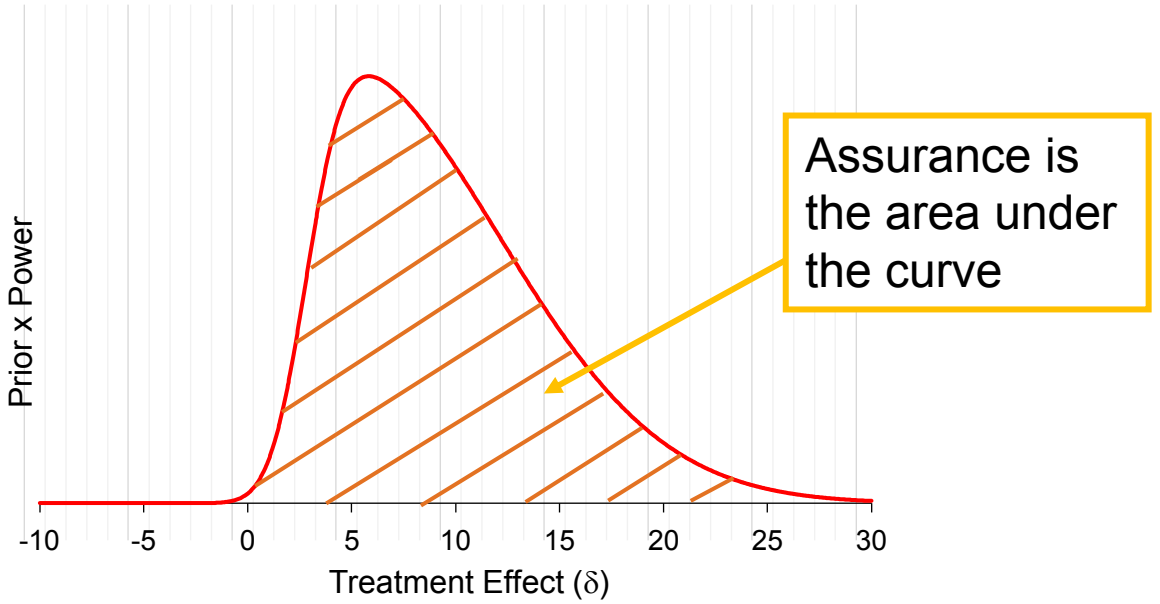
Evaluate "power" for a range of plausible effect sizes and calculate the average while weighing by how much you believe in each effect size

O'Hagan, A., Stevens, J. W., & Campbell, M. J. (2005). Assurance in Clinical Trial Design. *Pharmaceutical Statistics*, *4*(3), 187–201.

FERRING PHARMACEUTICALS

Assurance – simple case with success based on p-value





- The power $1 - \beta(\delta)$ is calculated conditional on δ being a specific value
- Assurance is the unconditional power: $\int_{\delta} \big(1 - \beta(\delta)\big) p(\delta) d\delta$ where $p(\delta)$ is the prior distribution

Assurance has an upper bound that can be below 1

- Unlike power, assurance will typically reach an upper bound below 1 as sample size increases
- The upper bound is the prior probability of meeting the success criteria before data in the proposed study have been collected.
- This probability should not be “too high”, otherwise it is hard to argue that randomization is ethical