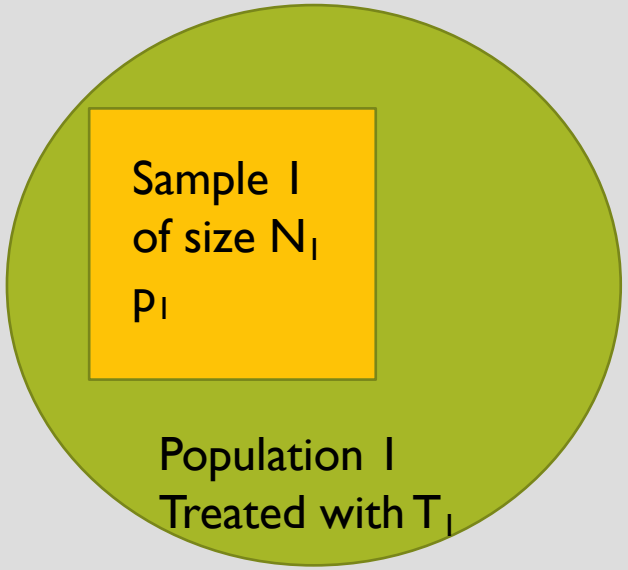
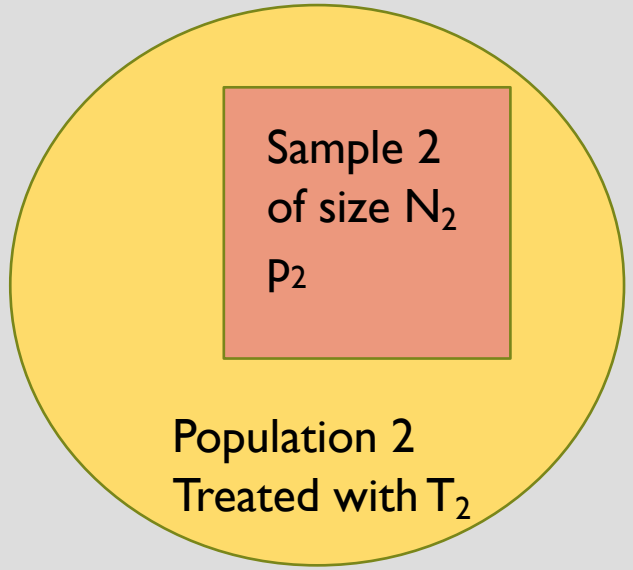
IS TREATMENT T_1 BETTER THAN T_2?

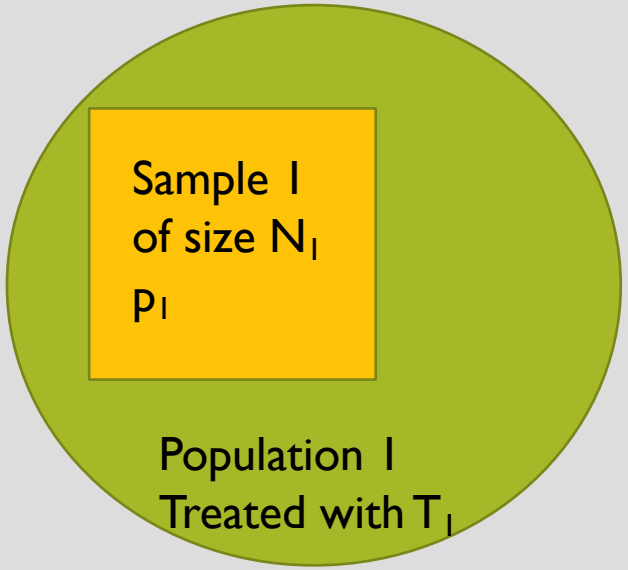
- Give T_1 to N_1 patients and T_2 to N_2 patients
  - Often $N_1 = N_2 = N$
- Record success rates p_1 of T_1 and p_2 of T_2

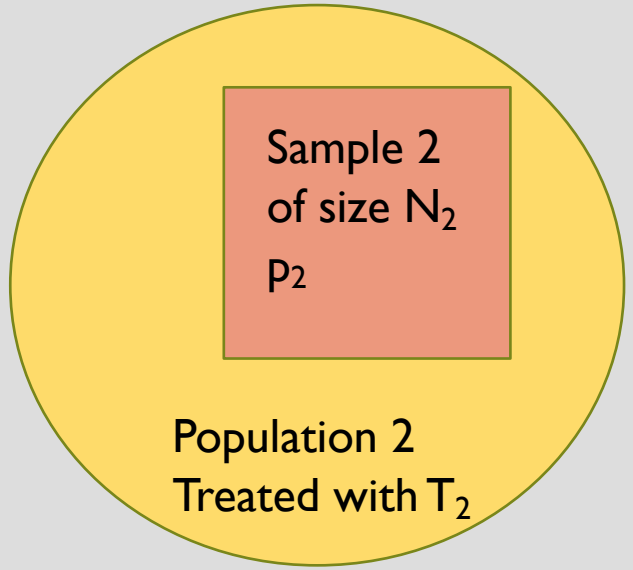




IS TREATMENT T_1 BETTER THAN T_2?

- Give T_1 to N_1 patients and T_2 to N_2 patients
  - Often $N_1 = N_2 = N$
  - Record success rates p_1 of T_1 and p_2 of T_2
- Statistics view to the Question
  - 1. Is p_1 ***statistically significantly*** larger than p_2?
    - I.e. is "$p_1 > p_2$" likely to hold also in other samples?
  - 2. How much ***uncertainty*** are there in the estimated success rates?
    - I.e. where estimates from other samples would be likely to reside?
- Crucial Q for the study design:
  - 3. **How should we choose N_1 and N_2?**





HYPOTHESIS TESTING

- H_0 (NULL HYPOTHESIS): There is no difference in success rates between the treatments
- H_A (ALTERNATIVE HYPOTHESIS): There is a difference in success rates between the treatments
- **Significance level** α: "Reject H_0" and "accept H_A" if P-value (calculated assuming H_0) is $< \alpha$
  - If α is defined before the experiment, then the proportion of false rejection of H_0 would be α in repeated experiments
  - By making α small (say 0.00001) we can protect from false positive findings (Type I errors) but increase false negative findings (Type II errors)
  - By keeping α larger (say 0.05) we have more statistical power to reject H_0 but we are more likely to make a false positive finding (Type I error)

Suomeksi:

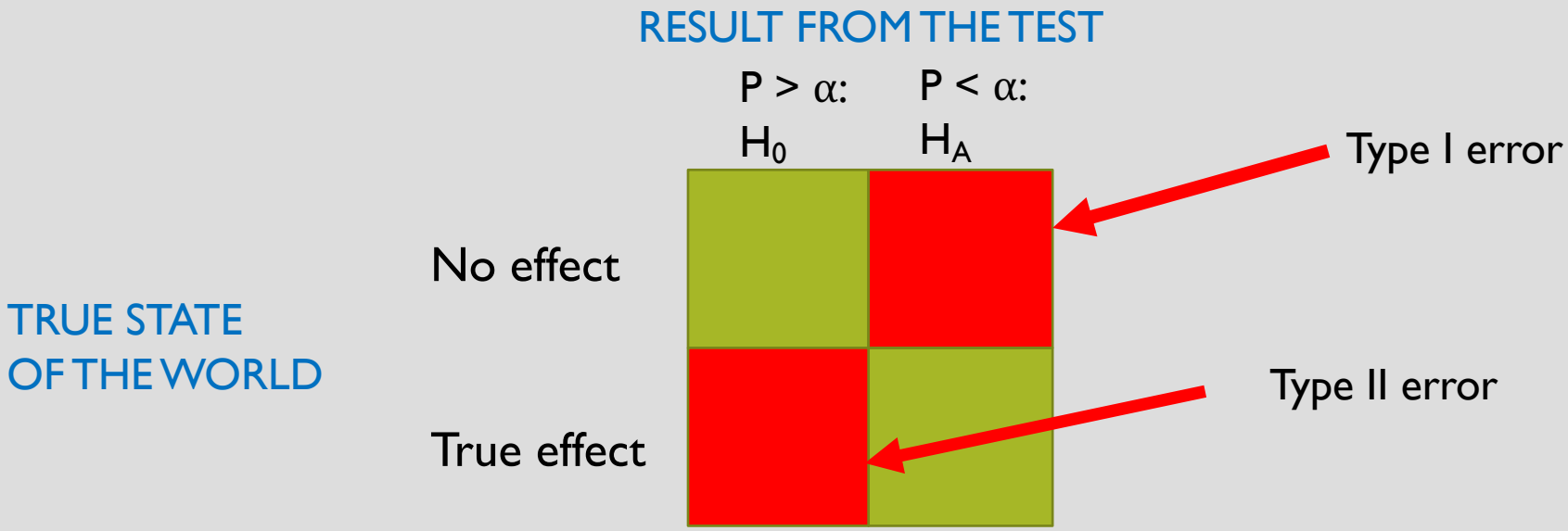
Merkitsevyystaso

Hylkää / hyväksy

Tyypin I / II virhe

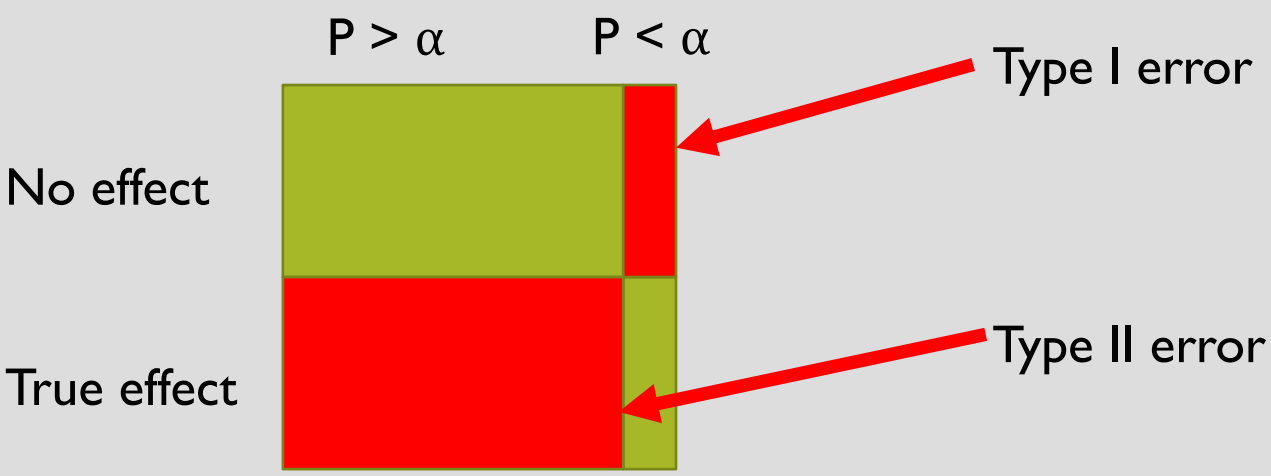
HYPOTHESIS TESTING FRAMEWORK

- Type I error: seeing an effect that is not real, a false positive
- Type II error: failing to see an effect that is real, a false negative
- Significance level α affects how likely different types of errors are to occur

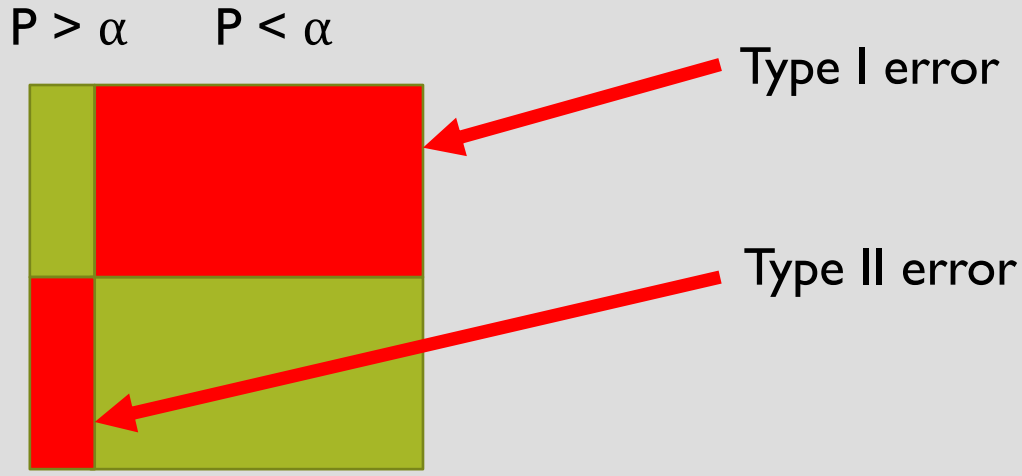


HYPOTHESIS TESTING FRAMEWORK

- Type I error: seeing an effect that is not real, a false positive
- Type II error: failing to see an effect that is real, a false negative
- With small significance level we rarely make Type I errors, but may often make Type II errors
- With large significance level we rarely make Type II errors, but may often make Type I errors



α small e.g. 0.0001



α large e.g. 0.5

INTERPRET PROP.TEST( ) FROM HYPOTHESIS TESTING FRAMEWORK

```r
# (ii)
prop.test(A,N,p.null, correct = FALSE)
```

```
##
##  1-sample proportions test without continuity correction
##
## data:  A out of N, null probability p.null
## X-squared = 17.195, df = 1, p-value = 3.373e-05
## alternative hypothesis: true p is not equal to 0.081
## 95 percent confidence interval:
##  0.08910332 0.10551545
## sample estimates:
##     p
## 0.097
```

A = 485
N = 5000
p.null = 0.081

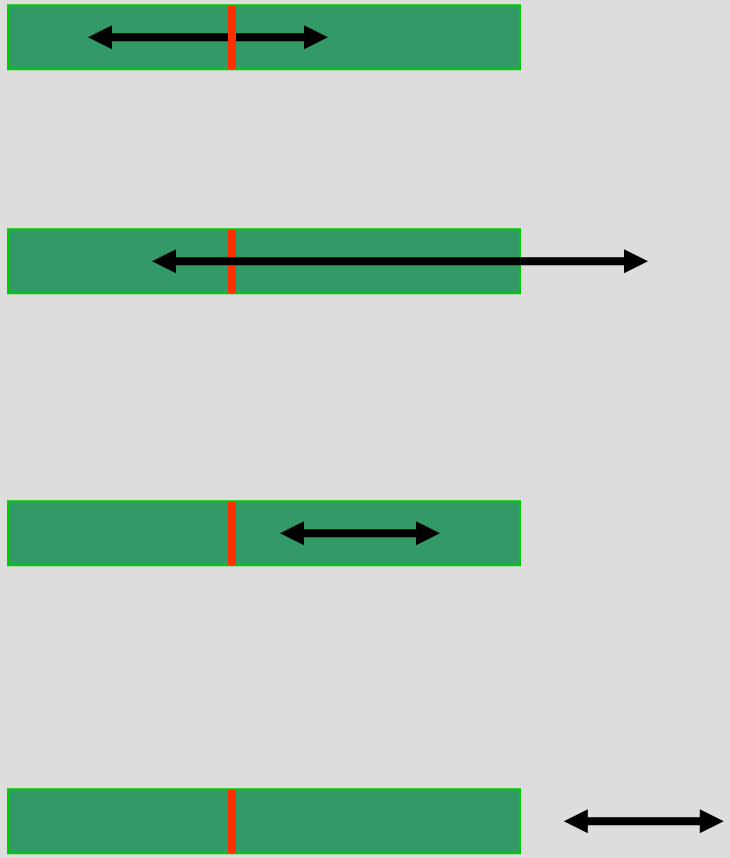
QUESTIONS

- Assume you are designing a study of the effect of a new treatment by a randomized clinical trial
- What is the null hypothesis?
- What is the alternative hypothesis?
- What is the relationship between P-value and significance level?
- What does it mean if we do a Type I error?
- What does it mean if we do a Type II error?
- How would you choose the significance level?
  - That is, which properties of your study affect your choice and what consequences does your choice have?

STATISTICAL VS CLINICAL SIGNIFICANCE

confidence intervals

Null value



- No evidence for departure from null – do not change clinical practice
- May be an important effect, but sample size is too small to be sure – do a larger study
- Statistically significantly different from null value – but not clinically important
- Statistically and clinically important – change practice

Rita Popat