# Case Study 3: Dressings

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STAT 8801 Statistical Consulting

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# Recap of Situation: What is the Question?

- Goal: compare a new dressing for minor operations to a standard dressing
- Experiment procedure:
  - two sites selected on each subject $\rightarrow$
  - wounds be simulated at each site $\rightarrow$
  - each subject has new dressing at one site and standard dressing at the other
- Measurement procedure:
  - outcomes (on a 1-5 scale): pain, infection level, healing speed, etc.
  - repeated at 7 days, 14 days, and 42 days after the surgery

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  - time to event: healing time
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- Collect as detailed personal information as possible at baseline (e.g., gender, age, socioeconomic status, etc.)
- Make efforts to ensure no subjects dropping out the study before it ends

# Sample Size: How Many Patients are Needed?

- FDA's requirement

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$$\frac{2(z_\alpha + z_\beta)^2}{[(\mu_1 - \mu_2)/\sigma]^2}$$

  outcomes measured across timepoints:[1]

$$\frac{2(z_\alpha + z_\beta)^2(1 + (n-1)\rho)}{[n(\mu_1 - \mu_2)/\sigma]^2}$$

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- Realistic constraints: money, time, effort

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# Data Analysis: How to Carry Out the Data Analysis?

Consider this question: is the new dressing more effective in reducing infection level at day 7 compared to the standard dressing?

| Subject | Trt(D7) | Infection(D7) |
|---|---|---|
| 1 | New | 2 |
| 1 | Std | 4 |
| 2 | New | 4 |
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Key: Within-subject observations (e.g., rows 1 and 2) are correlated, whereas between-subject observations (e.g., subjects 1 and 2) are independent.

Approaches:

- Simplify responses
- Control for subject id
- Model the within-subject correlation explicitly

# Data Analysis: How to Carry Out the Data Analysis? (cont.)

Simplify responses: one response for each subject

- Case I: continuous outcomes:
  - Compute the difference between the infection score under new dressing and that under standard dressing for each subject

| Subject | $I_{new}$(D7) | $I_{std}$(D7) | $\delta$ |
|---|---|---|---|
| 1 | 2 | 4 | -2 |
| 2 | 4 | 4 | 0 |
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  - Paired $t$-test: mean($\delta$) different from zero?

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  - Paired $t$-test: mean($\delta$) different from zero?
- Case II: binary outcomes:
  - Choose a threshold, e.g., 3

| Subject | $I_{new}$(D7) | $I_{std}$(D7) | Profile |
|---|---|---|---|
| 1 | 2 | 4 | LH |
| 2 | 4 | 4 | HH |
| ... | | | |

  - Pearson's residual test

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- Control for subject id: subjects as fixed blocks (consumes a lot of degrees of freedom but requires weaker assumption)

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- Similar logic extends into repeated measures analysis (e.g., if responses from all three measurement occasions are considered)

  ```
  > lmer(infection ~ factor(trt) + (1|subject) +
  (1|subject: day))
  ```