

## Paired difference analysis (summary)

### Exploration and description

1. Read the research question, verify sample is paired and outcome is quantitative.
2. If not already given, calculate within-pair differences (DELTA's).
3. Calculate  $\bar{x}_d$ ,  $s_d$ , and  $n_d$ . If data are asymmetrical, report the 5-point summary.
4. Plot the DELTA's and explore the distribution's location, spread, and shape.

### Estimation

1. Read the research question, verify that the data are quantitative and based on paired samples. Confirm that the parameter of interest is  $\mu_d$ .
2. Calculate the point estimate  $\bar{x}_d$  and the  $SEM_d = \frac{s_d}{\sqrt{n}}$
3. Calculate the  $(1 - \alpha)100\%$  CI for  $\mu_d = \bar{x}_d \pm t_{1-\frac{\alpha}{2}, n-1} \cdot SEM_d$
4. Report the point estimate for the mean difference, direction of the difference (increase or decrease), and the CI. Round appropriately (approx. 3 significant digits). Include units of measure.
5. Sample size for limiting the margin of error was also covered in this chapter.

### NHST

1. Read the research question, verify that the data are quantitative and based on paired samples. Confirm that the parameter of interest is  $\mu_d$ . State  $H_0: \mu_d = 0$ .
2. Calculate  $t_{\text{stat}} = \frac{\bar{x}_d - \text{expected mean difference when } H_0 \text{ true}}{SEM_d}$ ;  $df = n - 1$
3. Determine  $P$  value (app).
4. Report the point estimate, note direction of the difference (increase or decrease), and the  $P$  value. Use plain language and round appropriately; be kind to your reader.

Sample size to limit  $m$  was also considered earlier in this chapter.

### Illustration using OATBRAN data

**Exploration and description.** This cross over trial looked at within-pair differences on oatbran and corn flake diet. There were  $n = 14$  paired observations. **Data, procedures, and calculations are shown throughout in this chapter.** Here, we present only the interpretation of key results. Differences (CORNFLK – OATBRAN, mmol/l) are fairly symmetrical (stemplot below) with  $\bar{x}_d = 0.362$ , median approx. 0.35, and  $s_d = 0.406$ .

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-0 | 42
 0 | 011334
 0 | 6677
x1

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**Estimation.** The oat bran diet *decreased* cholesterol by an *average* of 0.362 mmol/l, 95% CI for  $\mu_d = (0.129 - 0.597)$  mmol/l.

**NHST.** The decline of 0.362 mmol/l was statistically significant ( $P = .0053$ ).

## **Estimation of a proportion (step-by-step summary)**

**Step 1. Review the research question and identify the parameter.** Read the research question. Verify that we have a single sample that addresses a binomial proportion ( $p$ ).

**Step 2. Point estimate.** Calculate the sample proportion ( $\hat{p}$ ) as the point estimate of the parameter.

**Step 3. Confidence interval.** Determine whether the  $z$  (normal approximation) formula can be used with the “ $npq$  rule.” If so, determine the  $z$  percentile for the given level of confidence (table) and the standard error of the proportion  $SEP = \sqrt{\frac{\hat{p}\hat{q}}{n}}$ . Apply the formula  $\hat{p} \pm (z_{1-\alpha/2})(SEP)$ .

**Step 4. Interpret the results.** In plain language report what proportion and the variable it address. Report the confidence interval; being clear about what population is being addressed. Reported results should be rounds as appropriate to the reader.

### Illustration

Of 2673 people surveyed, 170 have risk factor X. We want to determine the population prevalence of the risk factor with 95% confidence.

**Step 1.** Prevalence is the proportion of individuals with a binary trait. Therefore we wish to estimate parameter  $p$ .

**Step 2.**  $\hat{p} = 170 / 2673 = .06360 = 6.4\%$ .

**Step 3.**  $n\hat{p}\hat{q} = 2673(.0636)(1-.0636) = 159 \rightarrow z$  method OK.

$$SEP = \sqrt{\frac{\hat{p}\hat{q}}{n}} = \sqrt{\frac{(.0636)(1-.0636)}{2673}} = .00472$$

The 95% CI for  $p = \hat{p} \pm (z_{1-\alpha/2})(SEP) = 0.0636 \pm 1.96 \cdot .00472 = .0636 \pm .0093 = (.0543, .0729)$   
 $= (5.4\%, 7.3\%)$

**Step 4.** The prevalence in the sample was 6.4%. The prevalence in the population is between 5.4% and 7.3% with 95% confidence.

## The Exact Binomial Test

The exact binomial test is suited to test a binomial proportion from a single sample. A step-by-step analysis of the exact binomial test is presented.

**Step 1. Review the research question and identify the null hypothesis.** Read the research question. Verify that we have a single sample that addresses a binomial proportion. Identify the value of binomial parameter  $p$  when there is truly “no difference.” Write the null hypothesis in this form:

$$H_0: p = \text{the value of } p \text{ if } H_0 \text{ is true}$$

Calculate the sample proportion ( $\hat{p}$ ) to see how much it differs from the value proposed by the null hypothesis.

**Step 2. In lieu of a test statistic, determine the binomial pmf that applies under  $H_0$ .** Since this test is based on exact probabilities, there is no test statistic *per se*. Instead, list the pmf that applies when  $H_0$  is true.

$$\text{When } H_0 \text{ is true, } X \sim b(n, p)$$

where  $n$  is the sample size and  $p$  is the assumed value of  $p$  when  $H_0$  is true.

**Step 3: Determine the  $P$  value.** The  $P$  value is the probability of observing the data or data more extreme. When we are looking for an increase in the number of successes,  $P$  value =  $\Pr(X \geq x)$  where  $x$  is the observed number of successes. When we are looking for a decrease in the number of successes,  $P$  value =  $\Pr(X \leq x)$ .

**Step 4: Interpret results in narrative form.** Note the sample proportion, direction of the observed difference (increase or decrease), and  $P$  value. When the  $P$  value is small (say, less than .10), the evidence against the null hypothesis cannot easily be explained by chance (“statistical significance”).

### Illustration

Suppose a treatment has an expected success rate of 0.25. We observe successful treatment in 2 out of 3 patients. Is this observation worthy of note, i.e., is it statistically significant?

Step 1.  $H_0: p = .25$ . Note that  $\hat{p} = \frac{2}{3} = .6667$ .

Step 2. Under  $H_0$ ,  $X \sim b(3, .25)$

$X$	$\Pr(X = x)$
0	0.4219
1	0.4219
2	0.1406
3	0.0156

Step 3.  $P = \Pr(X \geq 2) = \Pr(X = 2) + \Pr(X = 3) = 0.1406 + 0.0156 = 0.1562$

Step 4: The difference between the observed proportion (2 of 3) and the expected proportion under the null hypothesis is explicable by chance, i.e., not statistically significant ( $P = .1562$ ).