**Example 0.1.** Investigation 1.5.2: Back Pain, *Neurology*, May 22, 2001 The randomized clinical trial examined whether the drug botulinum toxin A (Botox) is helpful for reducing pain among patients who suffer from chronic low back pain. The 31 subjects who participated in the study were randomly assigned to one of two treatment groups: 16 received a placebo of normal saline and the other 15 received the drug itself. The subjects' pain levels were evaluated at the beginning of the study and again after eight weeks. The researchers found that 2 of the 16 subjects who received the saline experienced a substantial reduction in pain, compared to 9 of the 15 subjects who received the actual drug.

1. Is this an experiment or an observational study?

2. What are the variables? Which variable is which? What are the observational units?

3. Explain the importance of using the "placebo" treatment of saline.

4. Create the two-way table for summarizing the data, putting the explanatory variable as the columns and the response as rows.

5. Calculate the conditional proportions of pain reduction in the two groups. Display the results as a segmented bar graph. Comment on the preliminary analysis.

$$
\begin{aligned}
\text{risk}_{\text{placebo}} = \frac{2}{16} &= 0.125 \\
\text{risk}_{\text{Botox}} = \frac{9}{15} &= 0.6 \\
RR &= 4.8
\end{aligned}
$$

	placebo	Botox	
pain reduction	2	9	11
no pain reduction	14	6	20
	16	15	31
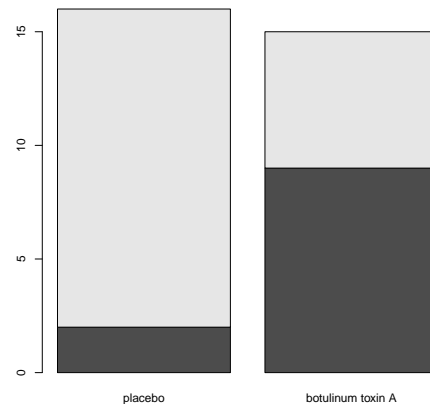

```
backpain <- matrix(c(2,14,9,6),ncol=2,byrow=F)
backpain
backpain.bp <- barplot(backpain)
barplot(backpain, names.arg=c("placebo","botulinum toxin A"))
text(backpain.bp, c(backpain[1,]-1,backpain[2,]+backpain[1,]-1),t(backpain))
```

6. If there was no association between the treatment and the back pain relief, about how many of the 11 "successes" would you expect to see in each group? Did the researchers observe more successes in the saline group than expected (if the drug had no effect) or fewer successes than expected? Is this in the direction conjectured by the researchers?

7. Is is *possible* that the drug has absolutely no effect on back pain? That the differences were simply due to chance or random variability? How likely is that?

Simulation

- 11 red "success" cards (pain reduction); 20 black "failure" cards (no pain reduction)
- randomly deal out (i.e. shuffle) 15 cards to the treatment group and 16 cards to the placebo group.
- count how many people in the treatment group were successes? Repeat 5 times.

0	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15

- process
  - what do the cards represent?
  - what does shuffling the cards represent?
  - what implicit assumption about the two groups did the shuffling of cards represent?
  - what observational units would be represented by the dots on the dotplot?
  - why would we count the number of repetitions with 9 or more "successes"?
- Repeat simulation using the two-way table applet: `http://www.rossmanchance.com/applets/TwowaySim/TwowaySim.html`
- summary
  - How many reps?
  - How many as extreme as the true data?
  - What proportion are at least as extreme as the true data?
  - Do our data support the researchers conjecture?
  - What if the actual data had been 7 successes in the treatment group (and 4 in the placebo group)?