

Contents

	Preface	<i>page xi</i>
1	Introduction	1
	1.1 Why is statistical analysis so important for clinical research?	1
2	Designing a study	8
	2.1 How do I choose a research question?	8
	2.2 How do I choose a study design?	11
	2.3 What are the differences between randomized and observational studies?	11
	2.4 What are the different types of randomized controlled trials?	18
	2.5 What are the different methods of allocating subjects within a randomized design?	20
	2.6 What are the different types of observational studies?	23
	2.7 Do I need to specify a particular hypothesis for my study?	32
	2.8 Can I specify an alternative hypothesis with a specific direction?	33
	2.9 Can my study have more than one question?	34
	2.10 What kind of measures should I use?	35
	2.11 How many subjects will I need for my study?	36
	2.12 How do I obtain an institutional review board approval to perform a research study?	37
3	Data management	38
	3.1 How do I manage my data?	38
	3.2 What procedures should I follow in collecting data?	38
	3.3 How do I create data collection instruments?	39
	3.4 How do I enter my data?	43
	3.5 How do I clean my data?	45

viii	Contents	
	3.6 How do I recode a variable?	45
	3.7 How do I transform a variable?	50
	3.8 When will I need to derive variables?	50
	3.9 When should I export my data to a statistical program?	50
4	Univariate statistics	52
	4.1 How should I describe my data?	52
	4.2 How should I describe my interval and ordinal variables?	52
	4.3 How should I describe my dichotomous variables?	57
	4.4 How should I describe my nominal variables?	59
	4.5 How should I describe my ordinal variables?	60
	4.6 How should I describe events that occur over time?	60
5	Bivariate statistics	66
	5.1 How do I assess an association between two variables?	66
	5.2 How do I assess an association between two dichotomous variables (comparison of proportions)?	66
	5.3 How do I test an association between a nominal variable and a dichotomous variable or between two nominal variables?	77
	5.4 How do I test an association involving an interval variable? (When do I use parametric statistics versus non-parametric statistics?)	79
	5.5 How do I test an association of a dichotomous variable with an interval variable?	84
	5.6 How do I test an association of a nominal variable with an interval variable?	88
	5.7 How do I test an association between two interval variables? (How do I determine if an association is linear?)	92
	5.8 How do I test an association of two variables when one or both of the variables are ordinal?	100
	5.9 How do I compare outcomes that occur over time?	102
	5.10 How do I analyse repeated observations of the same subject?	107
	5.11 How do I test bivariate associations with matched data?	116
6	Multivariable statistics	120
	6.1 What is multivariable analysis? Why is it necessary?	120
	6.2 How do I choose what type of multivariable analysis to use?	123
	6.3 What should I do if my outcome variable is ordinal or nominal?	123

6.4	How do I assess the impact of an individual variable on an outcome in a multivariable analysis?	124
6.5	What assumptions underlie multivariable models?	125

7

Sample size calculations 127

7.1	How do I determine the number of subjects needed for my study?	127
7.2	How do I determine the sample size needed for univariate statistics?	129
7.3	How do I determine the sample size needed for a univariate analysis of a dichotomous variable (proportion)?	130
7.4	How do I determine the sample size needed for a univariate analysis of an interval variable (mean)?	131
7.5	How do I determine the sample size needed for bivariate analysis?	131
7.6	How do I determine the sample size needed for comparison of two proportions (two dichotomous variables)?	133
7.7	How do I determine the sample size needed for comparison of two means (association of a dichotomous variable with a normally distributed interval variable)?	134
7.8	How do I determine the sample size needed for comparison of two normally distributed interval variables (Pearson's correlation coefficient)?	135
7.9	How do I determine the sample size needed for comparison of two survival times (log-rank statistic)?	135
7.10	How do I determine the sample size needed for multivariable analyses?	136
7.11	How do I determine the sample size needed to prove that two treatments are equal?	137
7.12	What if the sample size needed exceeds the sample size I can obtain?	138

8

Studies of diagnostic and prognostic tests (predictive studies) 141

8.1	How do predictive studies differ from explanatory studies?	141
8.2	What are sensitivity and specificity, and how are they related to one another?	143
8.3	What are the positive and negative predictive values of a test?	144
8.4	How do I determine the accuracy of a test?	145
8.5	How do I calculate the characteristics of a test with an interval scale?	146

x	Contents	
	8.6 What is Bayes' theorem?	148
	8.7 How do I choose the best standard for predictive studies?	153
	8.8 What population should I use for determining the predictive ability of a test?	154
	8.9 How is validity determined for predictive studies?	154
9	Statistics and causality	155
	9.1 When can statistical association establish causality?	155
	9.2 Can the results be statistically significant and clinically unimportant?	161
	9.3 Can the results be statistically insignificant and clinically important?	163
10	Special topics	165
	10.1 What is the difference between the relative risk and the absolute risk?	165
	10.2 What other effect measures are available in addition to relative risk and absolute risk?	165
	10.3 Do I need to use statistical analysis if I have population data?	170
	10.4 How do I choose what statistical program to use for analyzing data?	171
11	Publishing research	172
	11.1 How do I write my study up for publication?	172
	11.2 How do I determine authorship for the paper?	174
	11.3 How do I resolve disagreements about authorship?	175
	11.4 How do I decide what journal to send the paper to?	176
	11.5 What if my paper is rejected but I am asked to revise and resubmit it?	179
	11.6 What if my paper is rejected?	180
	11.7 How should I deal with the media?	181
12	Conclusion	183
	12.1 Would you review the steps for designing and analyzing data from a clinical study?	183
	Index	185