A selection of major references for clinical trials
(Annotated)

R. Chappell, 9/2010

I. A Good Basic Introduction to Biostatistics

Pagano, M. and Gauvreau, K. (2000). *Principles of Biostatistics, 2nd ed.* Duxbury: New York.

Basic introduction to statistical methods useful in clinical research. Includes a floppy disk of datasets.

II. Randomized clinical trials

A. Chang, M. (2008). Adaptive Design Theory and Implementation Using SAS and R. Chapman & Hall/CRC: Boca Raton, FL.

A practical guide to a new and rapidly expanding area.

B. Cook, T.D. and DeMets, D.L. (eds.) (2008). Introduction to Statistical Methods for Clinical Trials. Taylor & Francis: London.

An edited textbook aimed at beginning graduate students in statistics or others with a similar background.

C. Crowley, J. and Ankerst, D.P. (eds.) (2006). handbook of Statistics in Clinical Oncology, 2nd ed. Chapman & Hall/CRC: Boca Raton, FL.

A nice complement to Green, Benedetti, and Crowley (see below). Has many chapters on useful topics such as noninferiority trials, longitudinal analyses with missing data, and genomics in clinical trials.

D. DeMets, D.L., Furberg, C.D., and Friedman, L.M., 3rd ed. (2006). *Data Monitoring in Clinical Trials*. Springer.

A wide-ranging and practical assortment of interesting case studies.

E. Ellenberg, S., Fleming, T.R., and DeMets, D.L. (2002). *Data Monitoring committees in clinical trials: a practical perspective.* Wiley: New York.

F. Fairclough, D.L. (2002). *Design and Analysis of Quality of Life Studies in Clinical Trials.* Chapman & Hall: London.

The first book with extensive description of "quality of life" (psychometric) outcomes by the most noted researcher in the area. It pays close attention to the treatment of missing values and so is useful in other areas of clinical trials as well.

G. Finkelstein, Dianne M. and Schoenfeld, David A. eds (1995). *AIDS Clinical Trials: Guidelines for Design and Analysis.* Wiley: New York.

A variety of articles specific to an area in which methods quickly become out of date.

H. Friedman, L.M., Furberg, C.D., DeMets, D.L. (1998). *Fundamentals of Clinical Trials, 3rd ed*. Wiley: New York.

Basic principles, examples of cardiovascular trials. Good summary of methods and strategy for a nontechnical audience.

I. Geller, N.L., ed. (2004). *Advances in clinical trial biostatistics.*

A nice assortment of topics; good for those looking for thesis topics.

J. Green, S., Benedetti, J., and Crowley, J. (2003). *Clinical Trials in Oncology, 2nd ed.* Chapman and Hall: New York.

Very practical handbook to cancer clinical trials, motivated by experience with SWOG. Light on statistical methods; detailed presentation of data management and other logistical issues.

K. Julious, S.A. (2010). Sample Sizes for Clinical Trials. Chapman & Hall/CRC: Boca Raton, FL.

L. Machin, D., Day, S., and Green, S. (2006). *Textbook of Clinical Trials, 2nd ed.* Wiley: Chichester, England.

An interesting approach to describing clinical trials: this book has a 45-page introduction followed by 37 detailed examples from subdisciplines of cancer, psychiatry, surgery, and many other areas. Very useful for those trying to gain background on the issues in one or more of these areas.

M. Matthews, J.N.S. (2000). *An introduction to randomized controlled clinical trials.* Arnold: London.

A basic overview.

N. McFadden, Eleanor (1997). *Management of Data in Clinical Trials.* Wiley: New York.

A description of this essential aspect of clinical trials, by someone with a great deal of experience doing it.

O. Piantadosi, S. (2005). *Clinical Trials: a Methodologic Perspective, 2nd ed.* Wiley: Chichester.

P. Proschan, M.A., Lan, K.K.G., and Wittes, J.T. *Statistical Monitoring of Clinical Trials.* Springer: New York.

A practical guide to data monitoring. For a more theoretical approach see Jennison and Turnbull.

Q. Senn, S. (2002). *Cross-over trials in clinical research, 2nd ed.* Wiley: New York.

A specific topic, by one of its leading experts.

R. Thall, P.F., ed. (1995). *Recent advances in clinical trial design and analysis.* Kluwer: Boston.

Not so recent any more, but still a useful survey of clinical trial methodology.

S. Jennison, C. and Turnbull, B. (2000). *Group sequential methods with applications to clinical trials.* Chapman & Hall/CRC: London.

A detailed and fairly technical reference in this area by two of its leading experts. For a more applied approach see Proschan, Lan, and Wittes.

III. **Survival Analysis**

A. Andersen, P.K., Borgan, O., Gill, R.D. and Keiding, N. (1991). *Statistical Models Based on Counting Processes.* Springer: New York.

A theoretical treatment of counting processes.

B. Collett, D. (2003). *Modelling survival data in medical research, 2nd ed.* Chapman & Hall: New York.

Nontechnical, lots of examples.

C. Cox, D.R. and Oakes, D. (1984). *Analysis of Survival Data.* Chapman & Hall: New York.

Good brief introduction to survival analysis and applied reference. Clear and simple description of nonparametric and basic parametric methods.

D. Fleming, T.R. and Harrington, D.P. (1991). *Counting Processes and Survival Analysis.* Wiley: New York.

Asymptotic methods generally applicable to counting processes.

E. Kalbfleisch, J.D. and R. Prentice (2003). *The Statistical Analysis of Failure Time Data, 2nd ed.* Wiley: New York.

Advanced description of asymptotics for nonparametric methods, including proportional hazards with time-varying covariates, and more involved parametric techniques. If you are a Ph.D. student and want one book on survival analysis, this should be it (although Cox and Oakes offer a gentler introduction). Recently updated.

F. Klein, J.P. and Moeschberger, M.L. (2003). *Survival Analysis: Techniques for Censored and Truncated Data, 2nd ed.* Springer-Verlag: New York.

A nice applied survival analysis text. Its core material is presented at a moderate level, designed for data analysts but with extra material on more technical topics - it is a large book. It has sections on truncation, interval censoring, Martingale theory, random effects models, and other subjects.

IV. **Encyclopedias**

Wiley's *Encyclopedia of Statistics, 2nd ed.* and *Encyclopedia of Biostatistics, 2nd ed.* contain many entries which are relevant to clinical trials. The second is available on-line at the U.W. Ebling library, http://ebling.library.wisc.edu/ .

V. **Journals**

A. Traditional statistical journals: *JASA, JRSS, Annals of Statistics, Sankhya, Statistical Science*, etc.

B. More specialized statistical journals: *Biometrika, Biometrics.*

C. Most specialized statistical journals: *Statistics in Medicine*, *Clinical Trials* (formerly *Controlled Clinical Trials*), *Biostatistics*. The first is published by the International Society for Clinical Biostatistics; the second by the *Society for Clinical Trials*. *Lifetime Data Analysis* is a journal of survival analysis. *Good Clinical Practice Journal* is a magazine with articles on the practical issues of conducting clinical trials, particularly in an international setting. I do not recommend *Current Clinical Trials*.

D. Medical journals: *JAMA*, *NEJM*, and those of specific disciplines, especially oncology and cardiology, but increasingly others.

VI. **Web Sites**

A. International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) guidelines - http://www.ich.org/ich5.html

B. U.S. Food & Drug Administration (FDA) Center for Drug Evaluation and Research (CDER) guidance documents - http://www.fda.gov/cder/guidance/index.htm

C. NCI web site - http://ctep.info.nih.gov/resources/index.html