

Dalla Lana School of Public Health, University of Toronto

CHL 5225 H – Advanced Statistical Methods for Clinical Trials

Course Grading and Evaluation Policies

There will be three assignments and a presentation project. The three assignments will be primarily the statistical analysis of data. The first assignment will be given on September 22nd and is due on October 6th. The three assignments and presentation project will be weighed equally for the final grade for the course.

The presentation project can be the presentation of (i) a recent statistical methodology paper pertaining to clinical trials, (ii) an interesting statistical methodology issue in a recently published report of a clinical trial or (iii) the presentation of the analysis of data from a real (non-trivial) clinical trial. The topic for each project must be approved by a course instructor. A **two to three page report** of the presentation is also required. A list of potential papers for the presentation project is given below.

Project Ideas

If you select one of these projects, you must get approval of the corresponding instructor.

Week 2 (Willan, September 22nd)

Willan AR, Pater J. Using baseline measurements in the two-period crossover trial. *Controlled Clinical Trials* 1986; **7**: 282-289.

Week 4 (Raboud, October 6th)

Thiébaut ACM, Bénichou J. Choice of time-scale in Cox's model analysis of epidemiologic cohort data: a simulation study. *Statistics in Medicine* 2004; **23**: 3803-3820.

Hutson AD. Bootstrap-type confidence intervals for quantiles of the survival distribution. *Statistics in Medicine* 2001; **20**: 1693-1702.

Borkowf CB. A simple hybrid variance estimator for the Kaplan-Meier survival function. *Statistics in Medicine* 2005; **24**: 827-851.

Fleming TR, DeMets DL. Surrogate end points in clinical trials. Are we being misled? *Annals of Internal Medicine* 1996; **125**: 605-613.

Kafadar K, Prorok PC. A data-analytic approach for estimating lead time and screening benefit based on survival curves in randomized cancer screening trials. *Statistics in Medicine* 1994; **13**: 569-86.

Rosner GL. Bayesian monitoring of clinical trials with failure-time endpoints. *Biometrics* 2005; **61**: 239-245.

Wei LJ. The accelerated failure time model: a useful alternative to the Cox regression model in survival analysis. *Statistics in Medicine* 1992; **11**: 1871-1879.

Week 5 (Pintilie, October 13th)

Shao J *et al.* Statistical inference for cancer trials with treatment switching. *Statistics in Medicine* 2005; **24**: 1783-1790.

Mandel M *et al.* Evaluating survival model performance: a graphical approach. *Statistics in Medicine* 2005; **24**: 1933-1945.

Friedlin B, Korn EL. Testing treatment effects in the presence of competing risks. *Statistics in Medicine* 2005; **24**: 1703-1712.

Weeks 6 and 7 (Raboud, October 20th & 27th)

Hanley JA, Negassa A, deB Edwardes MD, Forrester JE. Statistical analysis of correlated data using generalized estimating equations: An orientation. *Amer J Epidemiol* 2003; **154**: 364-375.

Marshall JA, Scarbro S, Shetterly SM, Jones RH. Improving power with repeated measures: diet and serum lipids. *Am J Clin Nutr* 1998; **67**: 934-939.

Raman R, Hedeker D. A mixed-effects regression model for three-level ordinal response data. *Statistics in Medicine* 2005; **24**:3331-3345.

Week 8 (Willan, November 3rd)

Willan AR, O'Brien BJ, Leyva RA. Cost-effectiveness analysis when the WTA is greater than the WTP. *Statistics in Medicine* 2001; **20**:3251-3259.

Willan AR, Briggs AH, Hoch JS. Regression methods for covariate adjustment and subgroup analysis for non-censored cost-effectiveness data. *Health Economics* 2004; **13**: 461-475.

Willan AR, Lin DY, Manca A. Regression methods for cost-effectiveness analysis with censored data. *Statistics in Medicine*. 2005; **24**:131-145.

Week 9 (Raboud, November 10th)

Lancaster GA, Dodd S, Williamson PR. Design and analysis of pilot studies: recommendations for good practise. *J Evaluation in Clinical Practise* 2004; **10**: 307-312.

Lenth RV. Some practical guidelines for effective sample size Determination. *The American Statistician* Aug 2001; **55**: 187-193.

Week 10 (Willan, November 17th)

Willan AR, Kowgier ME. Determining optimal sample sizes for multi-stage randomized clinical trials using value of information methods. *Clinical Trials* 2008; **5**:289-300.

Willan AR. Optimal sample size determinations from an industry perspective based on the expected value of information. *Clinical Trials* 2008; **5**:587-594.

Willan AR, Eckermann S. Optimal clinical trial design using value of information methods with imperfect implementation. *Health Economics* DOI: 10.1002/hec.1493.