

Bibliography for Module 8 on Immunological Correlates of Protection

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Tier 1: Correlates of Risk

Barlow (1994), Barlow et al. (1999), Borgan et al. (2000), Breslow et al. (2009a), Breslow et al. (2009b), Breslow and Wellner (2007), Cai and Zheng (2007), Chan et al. (2002), Chen et al. (1990), Dunning (2006), Durham et al. (1998), Gilbert et al. (2005), Haynes et al. (2012), Huang et al. (2007), Jodar et al. (2003), Kulich and Lin (2004), Langholz and Jiao (2007), Langholz and Thomas (1991), Li et al. (2002), Li et al. (2008), Pepe and Fleming (1991a), Prentice (1986), Salk et al. (1943), Scheike and Martinussen (2004), Self and Prentice (1988), Siber (1997), Storsaeter et al. (1998), Therneau and Li (1999), Wacholder et al. (1991), Vessey et al. (2001)

Tier 2: Specific Surrogates of Protection / Correlates of Protection

Catanzaro et al. (2006), Chan et al. (2002), Czeschinski et al. (2000), Dunning (2008), Fleming and DeMets (1996), Follmann (2000), Follmann (2006), Frangakis and Rubin (2002a), Freedman et al. (1992), Gallop et al. (2009), Gilbert and Hudgens (2008a), Gilbert et al. (2008a), Gilbert et al. (2009), Gilbert et al. (2011a), Hallstrom et al. (2001), Huang and Gilbert (2011a), Huang et al. (2013), Kohberger et al. (2008), Lin et al. (1997), Miao et al. (2013), Pearl (2011), Prentice (1989), Qin et al. (2008), Siber (1997), Siber et al. (2007), Taylor et al. (2005), Wang and Taylor (2002), Wolfson and Gilbert (2010a), Zigler and Belin (2012)

Tier 3: General Surrogates of Protection

Alonso et al. (2004), Buyse et al. (2000), Daniels and Hughes (1997a), Gail et al. (2000), Pearl and Bareinboim (2011), Sadoff and Wittes (2007)

Multiple Tiers

Alonso et al. (2006), Burzykowski et al. (2005), Buyse and Molenberghs (1998), Hughes (2002), Joffe and Greene (2009), Pearl (2000), Plotkin and Gilbert (2012), Qin et al. (2007a), Robins and Greenland (1992), Rosenbaum (1984), Weir and Walley (2006)

General

Buchbinder et al. (2008), Chan and Bohidar (1998), Chan et al. (2004), Flynn et al. (2005), Halloran and Struchiner (1995), Hsieh and Lavori (1998), Lachin and Foulkes (1986), Lakatos (1988), Oxman et al. (2005), Plotkin (2008), Plotkin (2010), Robins (1995), Rubin (1986), Rerks-Ngarm et al. (2009), Schoenfeld (1981), Schoenfeld (1982), Smith et al. (1984), Organization (1993), Zhao et al. (2008), Li et al. (2013)

Session 10: Elaborations of Statistical Methods for Assessing Specific Surrogates of Protection

Bura and Gastwirth (2001), Chatterjee et al. (2003), Chatterjee and Chen (2007), Daniels and Hughes (1997b), Follmann (2006), Frangakis and Rubin (2002b), Gilbert and Hudgens (2008b), Gilbert et al. (2008b), Gilbert et al. (2011b), Heagerty and Pepe (1999), Huang et al. (2007), Huang and Gilbert (2011b), Huang and Pepe (2009), Molenberghs et al. (2008), Pepe and Fleming (1991b), Prentice (1989), Qin et al. (2007b), Schmader et al. (2012), Wolfson and Gilbert (2010b)

Main Sieve Analysis Articles

Binary endpoint sieve, “sieve conditions” for prospective interpretation based on retrospective analysis Gilbert et al. (1998)

Comparison of methods, refinement of “sieve conditions” Gilbert (2000)

Sieve analysis overview, examples, discussion of “sieve conditions” Gilbert et al. (2001)

Genome scanning methods Gilbert et al. (2008)

RV144 V2 sieve analysis results Rolland, Edlefsen et al. (2012) Liao et al. (2013)

Categorical Models and Inference Methods

Multinomial logistic regression (MLR) Gilbert et al. (1998), Cox (1970), Anderson (1972)

Exact inference for MLR Hirji (1992)

Ordered stereotype model Anderson (1984)

Cumulative logit model McCullagh (1980)

Likelihood ratio chi-squared test Armitage (1971)

Test for trend in odds ratios Breslow et al. (1980)

Linear-by-linear association test Agresti (1990) (pg. 284)

MLR asymptotic chi-squared test Zelen (1991)

Generalized logistic regression (GLR) Gilbert et al. (1999), Gilbert et al. (2000)

Discrete Competing Risks

Cause-specific hazards Prentice et al. (1978), Gray (1988), Aly et al. (1994)

Trick for assessing differential vaccine efficacy Lunn and McNeil (1995)

Linear rank tests Hu and Tsai (1999), Luo and Turnbull (1999), Sun (2001), McKeague et al. (2001)

Leaky vaccine Halloran et al. (1992)

Unbiased beta-hats for RCTs with low infection rates Rhodes et al. (1996)

Continuous Distance/Mark Models and Inference Methods

Nonparametric and semiparametric estimation and testing of mark-specific vaccine efficacy Gilbert et al. (2008), Sun et al. (2009)

Nonparametric and semiparametric estimation and testing of mark-specific vaccine efficacy accounting for missing marks/genetic distances Sun and Gilbert (2012)

Extensions to handle multiple genetic distances Sun et al. (2013), Juraska and Gilbert (2013)

Genome Scanning

- Hamming weights; VESPA software KORBER and MYERS (1992)
- HIV-specific substitution matrices Nickle et al. (2007)
- Amino acid physicochemical properties and antibody binding Hopp and Woods (1981)
- Protein conformation and antigen accessibility Hopp (1984)
- Using known antibody epitopes to predict antigenicity Welling et al. (1985)
- Previous approaches to AA divergence metrics: standardized Euclidean and Kullback-Leibler Wu et al. (2001)
- Previous approaches to AA divergence metrics: Mahalanobis Kowalski et al. (2002)
- Adding small positive constants to two-sample statistics in microarray analysis Efron et al. (2001), Tusher et al. (2001), Guo et al. (2003), Lönnstedt and Speed (2002)
- Mahalanobis asymptotics Johnson and Wichern (2002) pg. 285
- Pan's method for p-value pooling Pan (2003)
- Reasons to upweight positions in HIV Wyatt et al. (1998), Wei et al. (2003)
- Multiplicity adjustment Tarone (1990), Benjamini and Hochberg (1995), Gilbert (2005)

Data Examples

- Hepatitis B example Szmunn et al. (1981)
- HIV-1 Ordinal V3 tip sequence example Gilbert et al. (1998), Berman et al. (1997)
- STEP trial Buchbinder et al. (2008)

REFERENCES

- Agresti, A. (1990). Analysis of categorical data.
- Alonso, A., Molenberghs, G., Burzykowski, T. and et al. (2004). Prentice's approach and the meta-analytic paradigm: a reflection on the role of statistics in the evaluation of surrogate endpoints. *Biometrics* **60**, 724–728.
- Alonso, A., Molenberghs, G., Geys, H., Buyse, M. and Vangeneugden, T. (2006). A unifying approach for surrogate marker validation based on Prentice's criteria. *Statistics in Medicine* **25**, 205–221.

- Aly, E., Kochar, S. and McKeague, I. (1994). Some tests for comparing cumulative incidence functions and cause-specific hazard rates. *Journal of the American Statistical Association* pages 994–999.
- Anderson, J. (1972). Separate sample logistic discrimination. *Biometrika* **59**, 19–35.
- Anderson, J. (1984). Regression and ordered categorical variables. *Journal of the Royal Statistical Society. Series B (Methodological)* pages 1–30.
- Armitage, S. (1971). Methods in medical research.
- Barlow, W. (1994). Robust variance estimation for the case-cohort design. *Biometrics* **50**, 1064–1072.
- Barlow, W., Ichikawa, L., Rosber, D. and Izumi, S. (1999). Analysis of case-cohort designs. *Journal of Clinical Epidemiology* **52**, 1165–1172.
- Benjamini, Y. and Hochberg, Y. (1995). Controlling the false discovery rate: a practical and powerful approach to multiple testing. *Journal of the Royal Statistical Society. Series B (Methodological)* pages 289–300.
- Berman, P., Gray, A., Wrin, T., Vennari, J., Eastman, D., Nakamura, G., Francis, D., Gorse, G. and Schwartz, D. (1997). Genetic and immunologic characterization of viruses infecting mn-rgp120-vaccinated volunteers. *Journal of Infectious Diseases* **176**, 384.
- Borgan, L., Langholz, B., Samuelson, S. and Pogoda, J. (2000). Exposure stratified case-cohort designs. *Lifetime Data Analysis* **6**, 39–58.
- Breslow, N., Day, N. et al. (1980). *Statistical methods in cancer research. Vol. 1. The analysis of case-control studies.*, volume 1. Distributed for IARC by WHO, Geneva, Switzerland.
- Breslow, N., Lumley, T., Ballantyne, C., Chambless, L. and Kulich, M. (2009a). Improved horvitz-thompson estimation of model parameters from two-phase stratified samples: Applications in epidemiology. *Statistical Biosciences* **1**, 32–49.
- Breslow, N., Lumley, T., Ballantyne, C., Chambless, L. and Kulich, M. (2009b). Using the whole cohort in the analysis of case-cohort dat. *American Journal of Epidemiology* **169**, 1398–1405.
- Breslow, N. and Wellner, J. (2007). Weighted likelihood for semiparametric models and two-phase stratified samples, with application to Cox regression. *Scandinavian Journal of Statistics* **34**, 86–102.
- Buchbinder, S., Mehrotra, D., Duerr, A., Fitzgerald, D., Mogg, R., Li, D., Gilbert, P., Lama,

- J., Marmor, M., del Rio, C., McElrath, M., Casimiro, D., Gottesdiener, K., Chodakewitz, J., Corey, L. and Robertson, M. (2008). Efficacy assessment of a cell-mediated immunity HIV-1 vaccine (the Step Study): A double-blind, randomised, placebo-controlled, test-of-concept trial. *Lancet* **372**, 1881–1893.
- Buchbinder, S. P., Mehrotra, D. V., Duerr, A., Fitzgerald, D. W., Mogg, R., Li, D. et al. (2008). Efficacy assessment of a cell-mediated immunity HIV-1 vaccine (the Step Study): a double-blind, randomised, placebo-controlled, test-of-concept trial. *LANCET* **372**, 1881–1893.
- Bura, R. and Gastwirth, J. (2001). The binary regression quantile plot: Assessing the importance of predictors in binary regression visually. *Biometrical Journal* **43**, 5–21.
- Burzykowski, T., Molenberghs, G. and Buyse, M. (2005). *The Evaluation of Surrogate Endpoints*. Springer.
- Buyse, M. and Molenberghs, G. (1998). Criteria for the validation of surrogate endpoints in randomized experiments. *Biometrics* **54**, 1014–1029.
- Buyse, M., Molenberghs, G., Burzykowski, T., Renard, D. and Geys, H. (2000). The validation on surrogate endpoints in meta-analyses of randomized experiments. *Biostatistics* **1**, 49–67.
- Cai, J. and Zheng, D. (2007). Power calculation for case-cohort studies with nonrare events. *Biometrics* **63**, 1288–1295.
- Catanzaro, A., Koup, R., Roederer, M. and et al. (2006). Safety and immunogenicity evaluation of a multiclade HIV-1 candidate vaccine delivered by a replication-defective recombinant adenovirus vector. *Journal of Infectious Diseases* **194**, 1638–1649.
- Chan, I. and Bohidar, N. (1998). Exact power and sample size for vaccine efficacy studies. *Communications in Statistics, Theory and Methods* **27**, 1305–1322.
- Chan, I., Shu, L., Matthews, H., Chan, C., Vessey, R., Sadoff, J. and Heyse, J. (2002). Use of statistical models for evaluating antibody response as a correlate of protection against varicella. *Statistics in Medicine* **21**, 3411–3430.
- Chan, I., Wang, W. and Heyse, J. (2004). *Exact power and sample size for vaccine efficacy studies*. Informa Healthcare.
- Chatterjee, N. and Chen, Y. (2007). A semiparametric pseudo-score method for analysis of two-phase studies with continuous phase-i covariates. *Lifetime Data Analysis* **13**, 607–622.

- Chatterjee, N., Chen, Y. and Breslow, N. (2003). A pseudoscore estimator for regression problems with two-phase sampling. *Journal of the American Statistical Association* **98**, 158–168.
- Chen, R., Markowitz, L., Albrecht, P., Stewart, J., Mofenson, L., Preblud, S. and Orenstein, W. (1990). Measles antibody: reevaluation of protective titers. *The Journal of Infectious Diseases* **162**, 1036–1042.
- Cox, D. (1970). *Analysis of binary data*. Methuen.
- Czeschinski, P., Binding, N. and Witting, U. (2000). Hepatitis A and hepatitis B vaccinations: immunogenicity of combined vaccine and of simultaneously or separately applied single vaccines. *Vaccine* **18**, 1074–1080.
- Daniels, M. and Hughes, M. (1997a). Meta-analysis for the evaluation of potential surrogate markers. *Statistics in Medicine* **16**, 1965–1982.
- Daniels, M. and Hughes, M. (1997b). Meta-analysis for the evaluation of potential surrogate markers. *Statistics in Medicine* **16**, 1965–1982.
- Dunning, A. (2006). A model for immunological correlates of protection. *Statistics in Medicine* **25**, 1485–1497.
- Dunning, A. (2008). Comment on “Evaluating a surrogate endpoint at three levels, with application to vaccine development.”. *Statistics in Medicine* **27**, 6268–6270.
- Durham, L., Longini, I., Halloran, M., Clemens, J., Nizam, A. and Rao, M. (1998). Estimation of vaccine efficacy in the presence of waning: application to cholera vaccines. *American Journal of Epidemiology* **147**, 948–959.
- Efron, B., Tibshirani, R., Storey, J. and Tusher, V. (2001). Empirical bayes analysis of a microarray experiment. *Journal of the American Statistical Association* **96**, 1151–1160.
- Fleming, T. and DeMets, D. (1996). Surrogate endpoints in clinical trials: Are we being misled? *Annals of Internal Medicine* **125**, 605–613.
- Flynn, N., Forthal, D., Harro, C., Judson, F., Mayer, K., Para, M., Gilbert, P. and rgp120 HIV Vaccine Study Group, T. (2005). Placebo-controlled phase 3 trial of recombinant glycoprotein 120 vaccine to prevent HIV-1 infection. *Journal of Infectious Diseases* **191**, 654–65.
- Follmann, D. (2000). On the effect of treatment among treatment compliers: An analysis of the multiple risk factor intervention trial. *Journal of the American Statistical Association*

- 95**, 1101–1109.
- Follmann, D. (2006). Augmented designs to assess immune response in vaccine trials. *Biometrics* **62**, 1161–1169.
- Frangakis, C. and Rubin, D. (2002a). Principal stratification in causal inference. *Biometrics* **58**, 21–29.
- Frangakis, C. and Rubin, D. (2002b). Principal stratification in causal inference. *Biometrics* **58**, 21–29.
- Freedman, L., Graubard, B. and Schatzkin, A. (1992). Statistical validation of intermediate endpoints for chronic diseases. *Statistics in Medicine* **11**, 167–178.
- Gail, M., Pfeiffer, R., Van Houwelingen, H. and Carroll, R. (2000). On meta-analytic assessment of surrogate outcomes. *Biostatistics* **1**, 231–246.
- Gallop, R., Small, D., Lin, J., Elliott, M., Joffe, M. and Ten Have, T. (2009). Mediation analysis with principal stratification. *Statistics in Medicine* **28**, 1108–1130.
- Gilbert, P. (2000). Comparison of competing risks failure time methods and time-independent methods for assessing strain variations in vaccine protection. *Statistics in medicine* **19**, 3065–3086.
- Gilbert, P. (2005). A modified false discovery rate multiple-comparisons procedure for discrete data, applied to human immunodeficiency virus genetics. *Journal of the Royal Statistical Society: Series C (Applied Statistics)* **54**, 143–158.
- Gilbert, P., Hanna, G., DeGruttola, V., Martinez-Picado, J., Kuritzkes, D., Johnson, V., Richman, D. and D’Aquila, R. (2000). Comparative analysis of HIV type 1 genotypic resistance across antiretroviral trial treatment regimens. *AIDS Research and Human Retroviruses* **16**, 1325–1336.
- Gilbert, P. and Hudgens, M. (2008a). Evaluating candidate principal surrogate endpoints. *Biometrics* **64**, 1146–1154.
- Gilbert, P. and Hudgens, M. (2008b). Evaluating candidate principal surrogate endpoints. *Biometrics* **64**, 1146–1154.
- Gilbert, P., Hudgens, M. and Wolfson, J. (2011a). Commentary on “Principal stratification—a goal or a tool?”. *The International Journal of Biostatistics* .
- Gilbert, P., Hudgens, M. and Wolfson, J. (2011b). Commentary on” principal stratificationa goal or a tool?” by judea pearl. *The International Journal of Biostatistics* **7**, 36.

- Gilbert, P., Lele, S. and Vardi, Y. (1999). Maximum likelihood estimation in semiparametric selection bias models with application to AIDS vaccine trials. *Biometrika* **86**, 27–43.
- Gilbert, P., McKeague, I. and Sun, Y. (2008). The two-sample problem for failure rates depending on a continuous mark: an application to vaccine efficacy. *Biostatistics* **9**, 263–276.
- Gilbert, P., Peterson, M., Follmann, D. and et al. (2005). Correlation between immunologic responses to a recombinant glycoprotein 120 vaccine and incidence of HIV-1 infection in a Phase 3 HIV-1 preventive vaccine trial. *Journal of Infectious Diseases* **191**, 666–677.
- Gilbert, P., Qin, L. and Self, S. (2008a). Evaluating a surrogate endpoint at three levels, with application to vaccine development. *Statistics in Medicine* **27**, 4758–4778.
- Gilbert, P., Qin, L. and Self, S. (2008b). Evaluating a surrogate endpoint at three levels, with application to vaccine development. *Statistics in medicine* **27**, 4758–4778. PMID:PMC2646675.
- Gilbert, P., Qin, L. and Self, S. (2009). Response to Andrew Dunning’s comment on “Evaluating a surrogate endpoint at three levels, with application to vaccine development”. *Statistics in Medicine* **28**, 716–719.
- Gilbert, P., Self, S. and Ashby, M. (1998). Statistical methods for assessing differential vaccine protection against human immunodeficiency virus types. *Biometrics* **54**, 799–814.
- Gilbert, P., Self, S., Rao, M., Naficy, A. and Clemens, J. (2001). Sieve analysis: methods for assessing from vaccine trial data how vaccine efficacy varies with genotypic and phenotypic pathogen variation. *Journal of clinical epidemiology* **54**, 68–85.
- Gilbert, P., Wu, C. and Jobes, D. (2008). Genome scanning tests for comparing amino acid sequences between groups. *Biometrics* **64**, 198–207.
- Gray, R. (1988). A class of k-sample tests for comparing the cumulative incidence of a competing risk. *The annals of statistics* pages 1141–1154.
- Guo, X., Qi, H., Verfaillie, C. and Pan, W. (2003). Statistical significance analysis of longitudinal gene expression data. *Bioinformatics* **19**, 1628–1635.
- Halloran, M., Haber, M. and Longini, I. (1992). Interpretation and estimation of vaccine efficacy under heterogeneity. *American Journal of Epidemiology* **136**, 328–343.
- Halloran, M. and Struchiner, C. (1995). Causal inferences in infectious diseases. *Epidemiology* **6**, 142–151.

- Hallstrom, A., McAnulty, J., Wilkoff, B., Follmann, D., Raitt, M., Carlson, M., Gillis, A., Shih, H., Powell, J., Duff, H. and Halperin, B. (2001). Patients at lower risk of arrhythmia occurrence: A subgroup in whom implantable defibrillators may not offer benefit. *Journal of the American College of Cardiology* **37**, 1093–1099.
- Haynes, B., Gilbert, P., McElrath, M. and et al. (2012). Immune correlates analysis of the ALVAC-AIDSVAX HIV-1 vaccine efficacy trial. *New England Journal of Medicine* **366**, 1275–1286.
- Heagerty, P. J. and Pepe, M. S. (1999). Semiparametric estimation of regression quantiles with application to standardizing weight for height and age in u.s. children. *Applied Statistics* **48**, 533–551.
- Hirji, K. (1992). Computing exact distributions for polytomous response data. *Journal of the American Statistical Association* pages 487–492.
- Hopp, T. (1984). Protein antigen conformation: folding patterns and predictive algorithms; selection of antigenic and immunogenic peptides. *Annali Sclavo. Collana monografica* **1**, 47.
- Hopp, T. and Woods, K. (1981). Prediction of protein antigenic determinants from amino acid sequences. *Proceedings of the National Academy of Sciences* **78**, 3824.
- Hsieh, F. and Lavori, P. (1998). Sample size calculations for the Cox proportional hazards regression model with nonbinary covariates. *Controlled Clinical Trials* **21**, 552–560.
- Hu, X. and Tsai, W. (1999). Linear rank tests for competing risks model. *Statistica Sinica* **9**, 971–984.
- Huang, Y. and Gilbert, P. (2011a). Comparing biomarkers as principal surrogate endpoints. *Biometrics* **67**, 1442–1451.
- Huang, Y., Gilbert, P. and Wolfson, J. (2013). Design and estimation for evaluating principal surrogate markers in vaccine trials. *Biometrics* .
- Huang, Y. and Gilbert, P. B. (2011b). Comparing biomarkers as principal surrogate endpoints. *Biometrics* **67**, 1442–1451. PMID:PMC3163011.
- Huang, Y. and Pepe, M. (2009). A parametric roc model-based approach for evaluating the predictiveness of continuous markers in case–control studies. *Biometrics* **65**, 1133–1144. PMID:PMC2794984.
- Huang, Y., Pepe, M. and Feng, Z. (2007). Evaluating the predictiveness of a continuous

- marker. *Biometrics* **63**, 1181–1188.
- Huang, Y., Sullivan Pepe, M. and Feng, Z. (2007). Evaluating the predictiveness of a continuous marker. *Biometrics* **63**, 1181–1188.
- Hughes, M. (2002). Evaluating surrogate endpoints. *Controlled Clinical Trials* **23**, 703–707.
- Jodar, L., Butler, J., Carlone, G., Dagan, R., Goldblatt, D., Kyhty, H., Klugman, K., Plikaytis, B., Siber, G., Kohberger, R., Chang, I. and Cherian, T. (2003). Serological criteria for evaluation and licensure of new pneumococcal conjugate vaccine formulations for use in infants. *Vaccine* **21**, 3265–3272.
- Joffe, M. and Greene, T. (2009). Related causal frameworks for surrogate outcomes. *Biometrics* **65**, 530–538.
- Johnson, R. and Wichern, D. (2002). *Applied multivariate statistical analysis*, volume 4. Prentice hall Upper Saddle River, NJ.
- Juraska, M. and Gilbert, P. (2013). Mark-specific hazard ratio model with multivariate continuous marks: An application to vaccine efficacy. *Biometrics* .
- Kohberger, R., Jemiole, D. and Noriega, F. (2008). Prediction of pertussis vaccine efficacy using a correlates of protection model. *Vaccine* **26**, 3518–3521.
- KORBER, B. and MYERS, G. (1992). Signature pattern analysis: a method for assessing viral sequence relatedness. *AIDS research and human retroviruses* **8**, 1549–1560.
- Kowalski, J., Pagano, M. and DeGruttola, V. (2002). A nonparametric test of gene region heterogeneity associated with phenotype. *Journal of the American Statistical Association* **97**, 398–408.
- Kulich, M. and Lin, D. (2004). Improving efficiency of relative-risk estimation in case-cohort studies. *Journal of the American Statistical Association* **99**, 832–844.
- Lachin, J. and Foulkes, M. (1986). Evaluation of sample size and power for analyses of survival with allowance for nonuniform patient entry, losses to follow-up, non-compliance, and stratification. *Biometrics* **42**, 507–519.
- Lakatos, E. (1988). Sample size based on the log-rank statistic in complex clinical trials. *Biometrics* **44**, 229–241.
- Langholz, B. and Jiao, J. (2007). Computational methods for case-cohort studies. *Computational Statistics and Data Analysis* **51**, 3737–3748.
- Langholz, B. and Thomas, D. (1991). Efficiency of cohort sampling designs: Some surprising

- results. *Biometrics* **47**, 1563–1571.
- Li, S., Chan, I., Matthews, H., Heyse, J., Chan, C., Kutler, B., Kaplan, K., Vessey, S. and Sadoff, J. (2002). Childhood vaccination against varicella: inverse relationship between 6-week postvaccination varicella antibody response and likelihood of long-term breakthrough infection. *Pediatric Infectious Disease Journal* **21**, 337–342.
- Li, S., Parnes, M. and Chan, I. (2013). Determining the cutoff based on a continuous variable to define two populations with application to vaccines. *Journal of Biopharmaceutical Statistics* **23**, 662–680.
- Li, Z., Gilbert, P. and Nan, B. (2008). Weighted likelihood method for grouped survival data in case-cohort studies with application to HIV vaccine trials. *Biometrics* **64**, 1247–1255.
- Liao, H.-X., Bonsignori, M., Alam, S., McLellan, J., Tomaras, G. and et al. (2013). Vaccine induction of antibodies against a structurally heterogeneous site of immune pressure within HIV-1 envelope protein variable regions 1 and 2. *Immunity* **38**, 176–186.
- Lin, D., Fleming, T. and De Gruttola, V. (1997). Estimating the proportion of treatment effect explained by a surrogate marker. *Statistics in Medicine* **16**, 1515–1527.
- Lönnstedt, I. and Speed, T. (2002). Replicated microarray data. *Statistica sinica* **12**, 31–46.
- Lunn, M. and McNeil, D. (1995). Applying cox regression to competing risks. *Biometrics* pages 524–532.
- Luo, X. and Turnbull, B. (1999). Comparing two treatments with multiple competing risks endpoints. *Statistica Sinica* **9**, 985–998.
- McCullagh, P. (1980). Regression models for ordinal data. *Journal of the royal statistical society. Series B (Methodological)* pages 109–142.
- McKeague, I., Gilbert, P. and Kanki, P. (2001). Omnibus tests for comparison of competing risks with adjustment for covariate effects. *Biometrics* **57**, 818–828.
- Miao, C., Li, X., Gilbert, P. and Chan, I. (2013). *A multiple imputation approach for surrogate marker evaluation in the principal stratification causal inference framework. In: Risk Assessment and Evaluation of Predictions.*
- Molenberghs, G., Burzykowski, T., Alonso, A., Assam, P., Tilahun, A. and Buyse, M. (2008). The meta-analytic framework for the evaluation of surrogate endpoints in clinical trials. *Journal of statistical planning and inference* **138**, 432–449.
- Nickle, D., Heath, L., Jensen, M., Gilbert, P., Mullins, J. and Pond, S. (2007). Hiv-specific

- probabilistic models of protein evolution. *PLoS One* **2**, e503.
- Organization, W. H. (1993). The immunological basis for immunization series, module 1. *General Immunology* .
- Oxman, M., Levin, M. and Johnson, G. e. a. (2005). A vaccine to prevent herpes zoster and postherpetic neuralgia in older adults. *New England Journal of Medicine* **352**, 2271–2284.
- Pan, W. (2003). On the use of permutation in and the performance of a class of nonparametric methods to detect differential gene expression. *Bioinformatics* **19**, 1333–1340.
- Pearl, J. (2000). *Causality: models, reasoning, and inference*. Cambridge University Press, London.
- Pearl, J. (2011). Principal stratification– a goal or a tool? *The International Journal of Biostatistics* **7**, Article 20.
- Pearl, J. and Bareinboim, E. (2011). Transportability across studies: A formal approach. *Technical Report* pages 1–33.
- Pepe, M. and Fleming, T. (1991a). A non-parametric method for dealing with mismeasured covariate data. *Journal of the American Statistical Association* **86**, 108–113.
- Pepe, M. and Fleming, T. (1991b). A nonparametric method for dealing with mismeasured covariate data. *Journal of the American Statistical Association* pages 108–113.
- Plotkin, S. and Gilbert, P. (2012). Nomenclature for immune correlates of protection after vaccination. *Clinical Infectious Diseases* .
- Plotkin, S. A. (2008). Vaccines: Correlates of vaccine-induced immunity. *Clinical Infectious Diseases* **47**, 401–409.
- Plotkin, S. A. (2010). Correlates of protection induced by vaccination. *Clinical Vaccine Immunology* **17**, 1055–1065.
- Prentice, R. (1986). A case-cohort design for epidemiologic cohort studies and disease prevention trials. *Biometrika* **73**, 1–11.
- Prentice, R. (1989). Surrogate endpoints in clinical trials: definition and operational criteria. *Statistics in Medicine* **8**, 431–440.
- Prentice, R. L., Kalbfleisch, J. D., Peterson, A. V., J., Flournoy, N., Farewell, V. T. and Breslow, N. E. (1978). The analysis of failure times in the presence of competing risks. *Biometrics* **34**, pp. 541–554.
- Qin, L., Gilbert, P., Corey, L., McElrath, J. and Self, S. (2007a). A framework for assessing an

- immunological correlate of protection in vaccine trials. *The Journal of Infectious Diseases* **196**, 1304–1312.
- Qin, L., Gilbert, P., Corey, L., McElrath, M. and Self, S. (2007b). A framework for assessing immunological correlates of protection in vaccine trials. *Journal of Infectious Diseases* **196**, 1304.
- Qin, L., Gilbert, P., Follmann, D. and Li, D. (2008). Assessing surrogate endpoints in vaccine trials with case-cohort sampling and the Cox model. *Annals of Applied Statistics* **2**, 386–407.
- Rerks-Ngarm, S., Pitisuttithum, P., Nitayaphan, S. and et al. (2009). Vaccination with ALVAC and AIDSVAX to prevent HIV-1 infection in thailand. *New England Journal of Medicine* **361**, 2209–2220.
- Rhodes, P., Halloran, M. and Longini, I. (1996). Counting process models for infectious disease data: distinguishing exposure to infection from susceptibility. *Journal of the Royal Statistical Society, Series B* **58**, 751–762.
- Robins, J. (1995). An analytic method for randomized trials with informative censoring: Part I. *Lifetime Data Analysis* **1**, 241–254.
- Robins, J. and Greenland, S. (1992). Identifiability and exchangeability of direct and indirect effects. *Epidemiology* **3**, 143–155.
- Rolland, M., Edlefsen, P., Larsen, B., Tovanabutra, S., Sanders-Buell, E. and et al. (2012). Increased HIV-1 vaccine efficacy against viruses with genetic signatures in Env V2. *Nature* **490**, 417–420.
- Rosenbaum, P. (1984). The consequence of adjustment for a concomitant variable that has been affected by the treatment. *Journal of the Royal Statistical Society, Series A* **147**, 656–666.
- Rubin, D. (1986). Statistics and causal inference: which ifs have causal answers. *Journal of the American Statistical Association* **81**, 961–962.
- Sadoff, J. and Wittes, J. (2007). Correlates, surrogates, and vaccines. *The Journal of Infectious Diseases* **196**, 1279–1281.
- Salk, J., Menke, W. J. and Francis, T. J. (1943). A clinical, epidemiological and immunological evaluation of vaccination against epidemic influenza. *American Journal of Hygiene* **42**, 57–93.

- Scheike, T. and Martinussen, T. (2004). Maximum likelihood estimation for Cox's regression model under case-cohort sampling. *Scandinavian Journal of Statistics* **31**, 283–293.
- Schmader, K. E., Levin, M. J., Gnann, J. W., McNeil, S. A., Vesikari, T., Betts, R. F., Keay, S., Stek, J. E., Bundick, N. D., Su, S.-C. et al. (2012). Efficacy, safety, and tolerability of herpes zoster vaccine in persons aged 50–59 years. *Clinical infectious diseases* **54**, 922–928.
- Schoenfeld, D. (1981). The asymptotic properties of nonparametric tests for comparing survival distributions. *Biometrika* **68**, 316–331.
- Schoenfeld, D. (1982). Partial residuals for the proportional hazards regression model. *Biometrika* **69**, 239–241.
- Self, S. and Prentice, R. (1988). Asymptotic distribution theory and efficiency results for case-cohort studies. *Annals of Statistics* **16**, 64–81.
- Siber, G. (1997). Methods for estimating serological correlates of protection. *Developments in Biological Standardization* **89**, 283–296.
- Siber, G., Chang, I., Baker, S., Fernsten, P., O'Brien, K., Santosham, M., Klugman, K., Madhi, S., Paradiso, P. and Kohberger, R. (2007). Estimating the protective concentration of anti-pneumococcal capsular polysaccharide antibodies. *Vaccine* **25**, 3816–3826.
- Smith, P., Rodrigues, L. and Fine, P. (1984). Assessment of the protective efficacy of vaccines against common diseases using case-control and cohort studies. *International Journal of Epidemiology* **13**, 87–93.
- Storsaeter, J., Hallander, H., Gustafsson, L. and Olin, P. (1998). Levels of anti-pertussis antibodies related to protection after household exposure to bordetella pertussis. *Vaccine* **16**, 1907–1916.
- Sun, Y. (2001). Generalized nonparametric test procedures for comparing multiple cause-specific hazard rates. *Journal of Nonparametric Statistics* **13**, 171–207.
- Sun, Y. and Gilbert, P. (2012). Estimation of stratified mark-specific proportional hazards models with missing marks. *Scandinavian Journal of Statistics* **39**, 34–52.
- Sun, Y., Gilbert, P. and McKeague, I. (2009). Proportional hazards models with continuous marks. *Annals of Statistics* **37**, 394–426.
- Sun, Y., Li, M. and Gilbert, P. (2013). Mark-specific proportional hazards model with multivariate continuous marks and its application to HIV vaccine efficacy trials. *Biostatistics* **14**, 60–74.

- Szmunes, W., Stevens, C., Zang, E., Harley, E. and Kellner, A. (1981). A controlled clinical trial of the efficacy of the hepatitis b vaccine (heptavax b): a final report. *Hepatology* **1**, 377–385.
- Tarone, R. (1990). A modified bonferroni method for discrete data. *Biometrics* pages 515–522.
- Taylor, J., Wang, Y. and Thibaut, R. (2005). Counterfactual links to the proportion of treatment effect explained by a surrogate marker. *Biometrics* **61**, 1102–1111.
- Therneau, T. and Li, H. (1999). Computing the Cox model for case-cohort designs. *Lifetime Data Analysis* **5**, 99–112.
- Tusher, V., Tibshirani, R. and Chu, G. (2001). Significance analysis of microarrays applied to the ionizing radiation response. *Proceedings of the National Academy of Sciences* **98**, 5116.
- Vessey, S., Chan, C., Kuter, B., Kaplan, K., Waters, M., Kutzler, D., Carfagno, P., Sadoff, J., Heyse, J., Matthews, H., Li, S. and Chan, I. (2001). Childhood vaccination against varicella: persistence of antibody, duration of protection, and vaccine efficacy. *The Journal of Pediatrics* **139**, 297–304.
- Wacholder, S., Gail, M. and Pee, D. (1991). Selecting an efficient design for assessing exposure-disease relationships in an assembled cohort. *Biometrics* **47**, 63–76.
- Wang, Y. and Taylor, J. (2002). A measure of the proportion of treatment effect explained by a surrogate marker. *Biometrics* **58**, 803–812.
- Wei, X., Decker, J., Wang, S., Hui, H., Kappes, J., Wu, X., Salazar-Gonzalez, J., Salazar, M., Kilby, J., Saag, M. et al. (2003). Antibody neutralization and escape by hiv-1. *Nature* **422**, 307–312.
- Weir, C. and Walley, R. (2006). Statistical evaluation of biomarkers as surrogate endpoints: a literature review. *Statistics in Medicine* **25**, 183–203.
- Welling, G., Weijer, W., van der Zee, R. and Welling-Wester, S. (1985). Prediction of sequential antigenic regions in proteins. *FEBS letters* **188**, 215–218.
- Wolfson, J. and Gilbert, P. (2010a). Statistical identifiability and the surrogate endpoint problem, with application to vaccine trials. *Biometrics* **66**, 1153–1161.
- Wolfson, J. and Gilbert, P. (2010b). Statistical identifiability and the surrogate endpoint problem, with application to vaccine trials. *Biometrics* .
- Wu, T.-J., Hsieh, Y.-C. and Li, L.-A. (2001). Statistical measures of dna sequence dissimi-

- larity under markov chain models of base composition. *Biometrics* **57**, pp. 441–448.
- Wyatt, R., Kwong, P., Desjardins, E., Sweet, R., Robinson, J., Hendrickson, W., Sodroski, J. et al. (1998). The antigenic structure of the hivgp120 envelope glycoprotein. *NATURE-LONDON*- pages 705–710.
- Zelen, M. (1991). Multinomial response models. *Computational statistics & data analysis* **12**, 249–254.
- Zhao, Y., Wang, W. and Chan, I. (2008). Application of survival methodologies in vaccine trials. *FDA/Industry Workshop on Applied Statistics* .
- Zigler, C. and Belin, T. (2012). A bayesian approach to improved estimation of causal effect predictiveness for a principal surrogate endpoint. *Biometrics* **68**, 922–932.