# Selected Bibliography for Statistical Methods (and Clinical Papers) for Assessing Correlates of Vaccine Protection

Peter Gilbert, June 25, 2018

Bolded citations are most relevant to the overview talk "Statistical Methods for Assessing Correlates of Vaccine Protection"

#### Assessing Correlates of Risk (CoRs) of Infection or Disease

Prentice (1986), Barlow (1994), Therneau and Li (1999), Dunning (2008), Breslow et al. (2009b), Fong et al. (2017), Sun et al. (2017)

Prentice (1986), Self and Prentice (1988), Pepe and Fleming (1991), Wacholder et al. (1991), Langholz and Thomas (1991), Barlow (1994), Storsaeter et al. (1998), Barlow et al. (1999), Therneau and Li (1999), Heagerty and Pepe (1999), Borgan et al. (2000), Vessey et al. (2001), Chan et al. (2002), Li et al. (2002), Jodar et al. (2003), Chatterjee et al. (2003), Kulich and Lin (2004), Scheike and Martinussen (2004), Gilbert et al. (2005), Dunning (2006), Breslow and Wellner (2007), Cai and Zheng (2007), Huang et al. (2007), Chatterjee and Chen (2007), Langholz and Jiao (2007), Huang et al. (2007), Li et al. (2008), Dunning (2008), Huang and Pepe (2009), Breslow et al. (2009a), Breslow et al. (2009b), Haynes et al. (2012), Fong et al. (2017), Sun et al. (2017)

## Summaries/Comparisons of Correlates of Protection Frameworks/Nomenclature

Qin et al. (2007), Plotkin and Gilbert (2012), Gilbert et al. (2015), Buyse et al. (2016)

Buyse and Molenberghs (1998), Burzykowski et al. (2005), Alonso et al. (2006), Weir and Walley (2006), Qin et al. (2007), Sadoff and Wittes (2007), Plotkin and Gilbert (2012), Gilbert et al. (2015), Buyse et al. (2016)

#### Assessing Valid Prentice Surrogate Endpoints

## Prentice (1989), Gilbert et al. (2008), Price et al. (2018)

Prentice (1989), Freedman et al. (1992), Fleming and DeMets (1996), Lin et al. (1997), Siber (1997), Czeschinski et al. (2000), Bura and Gastwirth (2001), Chan et al. (2002), Catanzaro et al. (2006), Siber et al. (2007), Kohberger et al. (2008), Gilbert et al. (2008), Gilbert et al. (2015)

#### Estimated Optimal Surrogate (Within the Prentice Framework)

Price et al. (2018)

#### Assessing Principal Stratification/Specific Correlates of Vaccine Efficacy

Follmann (2006), Gilbert and Hudgens (2008), Huang et al. (2013), Gabriel et al. (2015), Sachs and Gabriel (2016)

Follmann (2000), Frangakis and Rubin (2002), Wang and Taylor (2002), Taylor et al. (2005), Follmann (2006), Gilbert and Hudgens (2008), Gilbert et al. (2008), Qin et al. (2008), Gallop et al. (2009), Gilbert et al. (2009), Wolfson and Gilbert (2010), Gilbert et al. (2011a), Gilbert et al. (2011b), Huang and Gilbert (2011), Pearl (2011), Zigler and Belin (2012), Huang et al. (2013), Miao et al. (2013), VanderWeele (2013), Gilbert et al. (2014), Gabriel and Gilbert (2014), Gabriel et al. (2015), Gabriel and Follmann (2016), Sachs and Gabriel (2016), Luedtke and Wu (2018)

#### Assessing Meta-Analysis/General Correlates of Protection

### Gail et al. (2000), Gabriel EE (2016)

Daniels and Hughes (1997), Buyse et al. (2000), Gail et al. (2000), Alonso et al. (2004), Molenberghs et al. (2008), Gabriel EE (2016), Gabriel EE (2017)

#### Other Statistical Methods on Assessing Immune Correlates of Protection

Robins and Greenland (1992), Robins (1995), Pearl (2000), Hughes (2002), Zhao et al. (2008), Joffe and Greene (2009), Pearl and Bareinboim (2011), Li et al. (2013), Lendle et al. (2013), Pearl and Bareinboim (2014)

#### **Clinical Papers on Correlates of Protection**

### Moodie et al. (2018)

Plotkin (2008), Plotkin (2010), Gilbert et al. (2014), Gilbert et al. (2017), Moodie et al. (2018)

## Sieve Analysis Statistical Methods for Categorical Pathogen Types (e.g., Serotypes, Genotypes) Mostly via Discrete Competing Risks Survival Analysis

### Prentice et al. (1978), Benkeser et al. (2018b)

Prentice et al. (1978), Gray (1988), Lunn and McNeil (1995), Gilbert et al. (1998), Gilbert et al. (2000), Sun (2001), Gilbert et al. (2001), Sun et al. (2008), Gilbert et al. (2008), Benkeser et al. (2018b), Benkeser et al. (2018a)

## Sieve Analysis Statistical Methods for Continuous Amino Acid Sequence Distances (e.g., Hamming Distances)

## Sun and Gilbert (2012), Gilbert and Sun (2015)

Gilbert et al. (2008), Sun et al. (2009), Sun and Gilbert (2012), Sun et al. (2013), Juraska and Gilbert (2013), Gilbert and Sun (2015), Sun et al. (2016), Juraska and Gilbert (2016)

## Main Applied Clinical Sieve Analysis Articles (Assessment of How Vaccine Efficacy Depends on AA Sequence Pathogen Features)

## Rolland<sup>\*</sup> et al. (2012), Neafsey et al. (2015)

Rolland et al. (2011), Rolland<sup>\*</sup> et al. (2012), Neafsey et al. (2015), Edlefsen et al. (2015), Hertz et al. (2016), deCamp et al. (2017)

## Correlates of Risk of Pathogen Type-Specific Outcomes

## Yang et al. (2017)

Yang et al. (2017), Sun et al. (2018), Lee et al. (2018)

## References

- 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.
- 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.
- Benkeser, D., Carone, M. and Gilbert, P. (2018a). Data-adaptive estimation of vaccine sieve effects in hiv and malaria phase iii trials. *Journal of the American Statistical Association*
- Benkeser, D., Carone, M. and Gilbert, P. (2018b). Improved estimation of the cumulative incidence of rare outcomes. *Statistics in Medicine* **37**, 280–293.
- Borgan, L., Langholz, B., Samuelson, S. and Pogoda, J. (2000). Exposure stratified casecohort designs. *Lifetime Data Analysis* 6, 39–58.
- 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 data. *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. PMCID:.
- 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 of surrogate endpoints in meta-analyses of randomized experiments. *Biostatistics* 1, 49–67.
- Buyse, M., Molenberghs, G., Paoletti, X., Oba, K., Alonso, A., der Elst, W. and Burzykowski, T. (2016). Statistical evaluation of surrogate endpoints with examples from cancer clinical trials. *Biometrical Journal* 58, 104–132.
- 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. PMCID:.
- 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.
- 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.
- 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. PMCID:.
- Daniels, M. and Hughes, M. (1997). Meta-analysis for the evaluation of potential surrogate markers. Statistics in Medicine 16, 1965–1982.
- deCamp, A., Rolland, M., Edlefsen, P., Sanders-Buell, E., Hall, B., Magaret, C. and et al. (2017). Sieve analysis of breakthrough hiv-1 sequences in hvtn 505 identifies vaccine pressure targeting the cd4 binding site of env-gp120. *PLoS ONE* **12**, e0185959.

- 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. PMCID:.
- Edlefsen, P. T., Rolland, M., Hertz, T., Tovanabutra, S., Gartland, A. J., deCamp, A. C., Magaret, C. A., Ahmed, H., Gottardo, R., Juraska, M. et al. (2015). Comprehensive sieve analysis of breakthrough hiv-1 sequences in the rv144 vaccine efficacy trial. *PLoS Computational Biology* **11**, e1003973–e1003973.
- Fleming, T. and DeMets, D. (1996). Surrogate endpoints in clinical trials: Are we being misled? Annals of Internal Medicine 125, 605–613.
- 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.
- Fong, Y., Gilbert, P. and Permar, S. (2017). chngpt: Threshold regression model estimation and inference with applications in immunological assay data. *BMC Bioinformatics* 18.
- Frangakis, C. and Rubin, D. (2002). 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.
- Gabriel, E. and Follmann, D. (2016). Augmented trial designs for evaluation of principal surrogates. *Biostatistics* pages 453–467.
- Gabriel, E. and Gilbert, P. (2014). Evaluating principle surrogate endpoints with time-toevent data accounting for time-varying treatment efficacy. *Biostatistics* **15**, 251–265.
- Gabriel, E. E., Sachs, M. C. and Gilbert, P. B. (2015). Comparing and combining biomarkers as principle surrogates for time-to-event clinical endpoints. *Statistics in Medicine* 34, 381– 395.
- Gabriel EE, Daniels MJ, H. M. (2016). Comparing biomarkers as trial level general surrogates. Biometrics 72, 1046–1054.
- Gabriel EE, Sachs MC, H. M. (2017). Evaluation and comparison of predictive individual-

level general surrogates. *Biostatistics* **0**, 1–18.

- 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. PMCID: PMC2669107.
- Gilbert, P., Gabriel, E., Huang, Y. and Chan, I. (2015). Surrogate endpoint evaluation: Principal stratification criteria and the Prentice definition. *Journal of Causal Inference* 3(2), 157–175.
- Gilbert, P., Gabriel, E., Miao, X., Li, X., Su, S.-C. and Chan, I. (2014). Fold-rise in gpELISA titers are an excellent correlate of protection in the Zostavax 022 trial, demonstrated via the vaccine efficacy curve. *The Journal of Infectious Diseases* 10, 1573–1581.
- 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. (2008). 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?" by Judea Pearl. *The International Journal of Biostatistics* 7, Article 1.
- 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., Juraska, M., deCamp, A., Karuna, S., Edupuganti, S., Mgodi, N., Donnell, D. and et al. (2017). Basis and statistical design of the passive HIV-1 antibody mediated prevention (AMP) test-of-concept efficacy trials. *Statistical Communications in Infectious Diseases* 9;1.
- 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. (2008). Evaluating a surrogate endpoint at three levels, with application to vaccine development. *Statistics in Medicine* 27, 4758–4778. PMCID: 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., 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. and Sun, Y. (2015). Inferences on relative failure rates in stratified mark-specific proportional hazards models with missing marks, with application to human immunodeficiency virus vaccine efficacy trials. Journal of the Royal Statistical Society: Series C (Applied Statistics). 64, 49–73.
- Gilbert, P., Wu, C. and Jobes, D. (2008). Genome scanning tests for comparing amino acid sequences between groups. *Biometrics* 64, 198–207.
- Gilbert, P. B., Self, S. G. and Ashby, M. A. (1998). Statistical methods for assessing differential vaccine protection against human immunodeficiency virus types. *Biometrics* pages 799–814.
- 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.
- 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.
- Hertz, T., Logan, M., Rolland, M., Magaret, C., Rademeyer, C., Fiore-Gartland, A., Edlefsen,
  P., DeCamp, A., Ahmed, H., Ngandu, N., Larsen, B., Frahm, N., Marais, J., Thebus, R.,
  Geraghty, D., Hural, J., Corey, L., Kublin, J., Gray, G., McElrath, M., Mullins, J.,
  Gilbert, P. and Williamson, C. (2016). A study of vaccine-induced immune pressure on

breakthrough infections in the phambili phase 2b hiv-1 vaccine efficacy trial. Vaccine 34, 5792 - 5801.

- Huang, Y. and Gilbert, P. (2011). 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* 69, 301–309.
- 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. PMCID:PMC2794984.
- Huang, Y., Pepe, M. and Feng, Z. (2007). Evaluating the predictiveness of a continuous marker. *Biometrics* **63**, 1181–1188. PMCID: PMC3059154.
- 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.
- Juraska, M. and Gilbert, P. (2013). Mark-specific hazard ratio model with multivariate continuous marks: An application to vaccine efficacy. *Biometrics* **69**, 328–337.
- Juraska, M. and Gilbert, P. (2016). Mark-specific hazard ratio model with missing multivariate marks. *Lifetime Data Analysis.* **22**, 606–625.
- Kohberger, R., Jemiolo, D. and Noriega, F. (2008). Prediction of pertussis vaccine efficacy using a correlates of protection model. *Vaccine* 26, 3518–3521.
- 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.
- Langholz, B. and Jiao, J. (2007). Computational methods for case-cohort studies. Computational Statistics and Data Analysis 51, 3737–3748. PMCID:.
- Langholz, B. and Thomas, D. (1991). Efficiency of cohort sampling designs: Some surprising

results. *Biometrics* 47, 1563–1571.

- Lee, U., Sun, Y., Scheike, T. and Gilbert, P. (2018). Analysis of generalized semiparametric regression models for cumulative incidence functions with missing covariates. *Biometrical Journal* in press, xx–xx.
- Lendle, S., Subbaraman, M. and van der Laan, M. (2013). Identification and efficient estimation of the natural direct effect among the untreated. *Biometrics* 69, 310–317.
- 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 6week 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.
- 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.
- Luedtke, A. and Wu, J. (2018). Efficient principally stratified treatment effect estimation in crossover studies with absorptive binary endpoints. *arxiv* **1712.05835**.
- Lunn, M. and McNeil, D. (1995). Applying cox regression to competing risks. *Biometrics* pages 524–532.
- 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. Springer, New York.
- 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.
- Moodie, Z., Juraska, M., Huang, Y., Zhuang, Y., Fong, Y., Carpp, L., Self, S., Chambonneau, L., Small, R., Jackson, N., Noriega, F. and Gilbert, P. (2018). Neutralizing antibody correlates analysis of tetravalent dengue vaccine efficacy trials in Asia and Latin America. *Journal of Infectious Diseases* 217(5), 742–753.

- Neafsey, D. E., Juraska, M., Bedford, T., Benkeser, D., Valim, C., Griggs, A., Lievens, M., Abdulla, S., Adjei, S., Agbenyega, T. et al. (2015). Genetic diversity and protective efficacy of the rts, s/as01 malaria vaccine. New England Journal of Medicine 373, 2025–2037.
- 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 of causal and statistical relations: A formal approach. Proceedings of the Twenty-Fifth National Conference on Artificial Intelligence, Menlo Park, CA pages 247–254.
- Pearl, J. and Bareinboim, E. (2014). External validity: From do-calculus to transportability across populations. *Statistical Science* **29**, 579–595.
- Pepe, M. and Fleming, T. (1991). A non-parametric method for dealing with mismeasured covariate data. Journal of the American Statistical Association 86, 108–113.
- Plotkin, S. and Gilbert, P. (2012). Nomenclature for immune correlates of protection after vaccination. *Clinical Infectious Diseases* 54, 1615–1617.
- 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., Kalbfleisch, J., Peterson, A., Fluornoy, N., Farewell, V. and Breslow, N. (1978). The analysis of failure time in the presence of competing risk. *Biometrics* 34, 541–554.
- Price, B., Gilbert, P. and van der Laan, M. (2018). Estimation of the optimal surrogate based on a randomized trial. *Biometrics*.
- Qin, L., Gilbert, P., Corey, L., McElrath, J. and Self, S. (2007). A framework for assessing an immunological correlate of protection in vaccine trials. *The Journal of Infectious Diseases* 196, 1304–1312.

- 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.
- Robins, J. (1995). An analytic method for randomized trials with informative censoring: PartI. 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. and et al. (2012). Increased HIV-1 vaccine efficacy against viruses with genetic signatures in Env V2. *Nature* 490, 417–420. \*Contributed equally.
- Rolland, M., Tovanabutra, S., deCamp, A., Frahm, N., Gilbert, P., Sanders-Buell, E. and et al. (2011). Genetic impact of vaccination on breakthrough HIV-1 sequences from the STEP trial. *Nature Medicine* **17**, 366–371. PMCID: PMC3053571.
- Sachs, M. C. and Gabriel, E. E. (2016). An Introduction to Principal Surrogate Evaluation with the pseual Package. The R Journal 8, 277–292.
- Sadoff, J. and Wittes, J. (2007). Correlates, surrogates, and vaccines. The Journal of Infectious Diseases 196, 1279–1281. PMCID:.
- 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.
- 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.
- 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. PMCID: PMC3601495.
- Sun, Y., Gilbert, P. and McKeague, I. (2009). Proportional hazards models with continuous marks. Annals of Statistics 37, 394–426. PMCID: PMC2762218.
- Sun, Y., Hyun, S. and Gilbert, P. (2008). Testing and estimation of time-varying causespecific hazard ratios with covariate adjustment. *Biometrics* **64**, 1070–1079.
- 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. PMCID: PMC3520499.
- Sun, Y., Li, M. and Gilbert, P. (2016). Goodness-of-fit test of the stratified mark-specific proportional hazards model with continuous mark. *Computational Statistics and Data Analysis* 93, 348–358. PMCID: PMC4598956.
- Sun, Y., Qian, X., Shou, Q. and Gilbert, P. (2017). Analysis of two-phase sampling data with semiparametric additive hazards models. *Lifetime Data Analysis* 23, 377–399.
- Sun, Y., Yang, G., Gilbert, P. and Qi, L. (2018). Hypothesis tests for stratified mark-specific proportional hazards models with missing covariates, with application to hiv vaccine efficacy trials. *Biometrical Journal* 60(3), 516–536.
- 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.
- VanderWeele, T. (2013). Surrogate measures and consistent surrogates. Biometrics 69, 561– 568.
- 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.
- Weir, C. and Walley, R. (2006). Statistical evaluation of biomarkers as surrogate endpoints: a literature review. *Statistics in Medicine* **25**, 183–203.
- Wolfson, J. and Gilbert, P. (2010). Statistical identifiability and the surrogate endpoint problem, with application to vaccine trials. *Biometrics*.
- Yang, G., Sun, Y., Qi, L. and Gilbert, P. (2017). Estimation of stratified mark-specific proportional hazards models under two-phase sampling with application to hiv vaccine efficacy trials. *Statistical Biosciences* 9, 259–283.
- Zhao, Y., Wang, W. and Chan, I. (2008). Application of survival methodologies in vaccine trials. FDA/Industry Workshop on Applied Statistics PMCID:.
- 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.