### Bibliography for Module 8 on Immunological Correlates of Protection and Sieve Analysis of Pathogen Sequences

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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)

## Session: 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), 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 of stratified mark-specific proportional hazards models with missing marks. (), ()

#### 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 Szmuness et al. (1981)

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

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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.

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