References

A. Abadie and G.W. Imbens. Large sample properties of matching estimators for average treatment effects. *Econometrica*, 74:235–267, 2006.

G.D. Adamson, J. de Mouzon, P. Lancaster, K.-G. Nygren, E. Sullivan, and F. Zegers-Hochschild. World collaborative report on in vitro fertilization, 2000. *Fertil Steril*, 85:1586–1622, 2006.

A. Afifi and S. Azen. *Statistical Analysis: A Computer Oriented Approach*. Academic, New York, 2nd edition, 1979.

H. Akaike. Information theory and an extension of the maximum likelihood principle. In B.N. Petrov and F. Csaki, editors, *Second International Symposium on Information Theory*, Budapest, 1973. Academiai Kiado.

K. Akazawa, T. Nakamura, and Y. Palesch. Power of logrank test and Cox regression model in clinical trials with heterogenous samples. *Stat Med*, 16(5):583–597, 1997.

C. Ambroise and G.J. McLachlan. Selection bias in gene extraction on the basis of microarray gene-expression data. *Proc Natl Acad Sci*, 99(10):6562–6566, 2002.

P.K. Andersen, O. Borgan, R.D. Gill, and N. Keiding. *Statistical Models Based on Counting Processes*. Springer, Berlin Heidelberg New York, 1993.

J.A. Anderson. Separate sample logistic discrimination. *Biometrika*, 59:19–35, 1972.

K.M. Anderson, P.W.F. Wilson, P.M. Odell, and W.B. Kannel. An updated coronary risk profile. a statement for health professionals. *Circulation*, 83:356–362, 1991.

A.C. Atkinson and A. Biswas. Adaptive biased-coin designs for skewing the allocation proportion in clinical trials with normal responses. *Stat Med*, 24(16): 2477–2492, 2005.

E. Balciunaite, A. Spektor, N.H. Lents, H. Cam, H. Te Riele, A. Scime, M.A. Rudnicki, R. Young, and B.D. Dynlacht. Pocket protein complexes are recruited to distinct targets in quiescent and proliferating cells. *Mol Cell Biol*, 25(18):8166–78, 2005.

U. Bandyopadhyay and A. Biswas. Adaptive designs for normal responses with prognostic factors. *Biometrika*, 88(2):409–419, 2001.

D.R. Bangsberg, F. Hecht, E.D. Charlebois, M. Chesney, and A.R. Moss. Comparing objectives measures of adherence to hiv antiretroviral therapy: Electronic medication monitors and unannounced pill counts. *AIDS Behav*, 5:275–281, 2001.

W.E. Barlow, E. White, R. Ballard-Barbash, P.M. Vacek, L. Titus-Ernstoff, P.A. Carney, J.A. Tice, D.S. Buist, B.M. Geller, R. Rosenberg, B.C. Yankaskas, and K. Kerlikowske. Prospective breats cancer risk prediction model for women undergoing screening mammography. *J Natl Cancer Inst*, 98(17):1204–1214, 2006.

R. Baron and D. Kenny. The moderator-mediator variable distinction in social psychological research: Conceptual, strategic, and statistical considerations. *J Personal Soc Psychol*, 51:1173–1182, 1986.

J. Baselga. Herceptin alone or in combination with chemotherapy in the treatment of HER2-positive metastatic breast cancer: Pivotal trials. *Oncology*, 61(S2):14–21, 2001.

C.J. Basten, B.S. Weir, and Z.B. Zeng. *QTL Cartographer*, 2001. URL http://statgen.ncsu.edu/qtlcart/.

O. Bembom and M.J. van der Laan. A practical illustration of the importance of realistic individualized treatment rules in causal inference. *Electron J Stat*, 1: 574–596, 2007a.

O. Bembom and M.J. van der Laan. Comment: Statistical methods for analyzing sequentially randomized trials. *J Natl Cancer Inst*, 99(21):1577–1582, 2007b.

O. Bembom and M.J. van der Laan. Data-adaptive selection of the truncation level for inverse-probability-of-treatment-weighted estimators. Technical Report 230, Division of Biostatistics, University of California, Berkeley, 2008.

O. Bembom, W.J. Fessel, R.W. Shafer, and M.J. van der Laan. Data-adaptive selection of the adjustment set in variable importance estimation. 231, Division of Biostatistics, University of California, Berkeley, 2008.

O. Bembom, M.L. Petersen, S.-Y. Rhee, W.J. Fessel, S.E. Sinisi, R.W. Shafer, and M.J. van der Laan. Biomarker discovery using targeted maximum likelihood estimation: Application to the treatment of antiretroviral resistant HIV infection. *Stat Med*, 28:152–72, 2009.

Y. Benjamini and Y. Hochberg. Controlling the false discovery rate: A practical and powerful approach to multiple testing. *J R Stat Soc Ser B*, 57:289–300, 1995.

E.R. Berndt. *The Practice of Econometrics*. Addison–Wesley, New York, 1991.

J. Bhattacharya and W. Vogt. Do instrumental variables belong in propensity scores? Technical Report 343, National Bureau of Economic Research, 2007.

P.J. Bickel, C.A.J. Klaassen, Y. Ritov, and J. Wellner. *Efficient and adaptive estimation for semiparametric models*. Springer, Berlin Heidelberg New York, 1997.

W.Z. Billewicz. The efficiency of matched samples: An empirical investigation. *Biometrics*, 21(3):623–644, 1965.

M.D. Birkner, A.E. Hubbard, and M.J. van der Laan. Data adaptive pathway testing. Technical Report 197, Division of Biostatistics, University of California, Berkeley, 2005.

J. Boivin, L. Bunting, J.A. Collins, and K.G. Nygren. International estimates of infertility prevalence and treatment-seeking: Potential need and demand for infertility medical care. *Hum Reproduct*, 22:1506–1512, 2007.

G.E.P. Box and N.R. Draper. *Empirical Model-Building and Response Surfaces*. Wiley, Hoboken, 1987.

V.L. Boyartchuk, K.W. Broman, R.E. Mosher, S.E.F. D'Orazio, M.N. Starnbach, and W.F. Dietrich. Multigenic control of listeria monocytogenes susceptibility in mice. *Nat Genet*, 2001.

H. Bozdogan. Choosing the number of component clusters in the mixture model using a new informational complexity criterion of the inverse fisher information matrix. In O. Opitz, B. Lausen, and R. Klar, editors, *Information and Classification*. Springer, Berlin Heidelberg New York, 1993.

H. Bozdogan. Akaike's information criterion and recent developments in information complexity. *J Math Psychol*, 44:62–91, 2000.

H. Brady. Causation and explanation in social science. In J.M. Box-Steffensmeier, H.E. Brady, and D. Collier, editors, *The Oxford Handbook of Political Methodology*. Oxford, New York, 2008.

L. Breiman. Heuristics of instability and stabilization in model selection. *Ann Stat*, 24(6):2350–2383, 1996a.

L. Breiman. Out-of-bag estimation. Technical Report, Department of Statistics, University of California, Berkeley, 1996b.

L. Breiman. Stacked regressions. *Mach Learn*, 24:49–64, 1996c.

L. Breiman. Bagging predictors. *Mach Learn*, 24(2):123–140, 1996d.

L. Breiman. Arcing classifiers. *Ann Stat*, 26:801–824, 1998.

L. Breiman. Random forests - random features. Technical Report 567, Department of Statistics, University of California, Berkeley, 1999.

L. Breiman. *Notes on setting up, using, and understanding random forests V3.0*, 2001a.

L. Breiman. Random forests. *Mach Learn*, 45:5–32, 2001b.

L. Breiman and P. Spector. Submodel selection and evaluation in regression. The *X* random case. *Int Stat Rev*, 60:291–319, 1992.

L. Breiman, J.H. Friedman, R. Olshen, and C.J. Stone. *Classification and Regression Trees*. Chapman & Hall, Boca Raton, 1984.

N.E. Breslow. Statistics in epidemiology: The case-control study. *J Am Stat Assoc*, 91:14–28, 1996.

N.E. Breslow and N.E. Day. *Statistical Methods in Cancer Research: Volume 1 – The Analysis of Case-Control Studies*. International Agency for Research on Cancer, Lyon, 1980.

N.E. Breslow, N.E. Day, K.T. Halvorsen, R.L. Prentice, and C. Sabal. Estimation of multiple relative risk functions in matched case-control studies. *Am J Epidemiol*, 108(4):299–307, 1978.

K.W. Broman. Mapping quantitative trait loci in the case of a spike in the phenotype distribution. *Genetics*, 2003.

F. Bunea, A.B. Tsybakov, and M.H. Wegkamp. Aggregation and sparsity via L1 penalized least squares. In G. Lugosi and H.-U. Simon, editors, *COLT*, volume

4005 of *Lecture Notes in Computer Science*, Berlin Heidelberg New York, 2006. Springer.

F. Bunea, A.B. Tsybakov, and M.H. Wegkamp. Aggregation for gaussian regression. *Ann Stat*, 35(4):1674–1697, 2007a.

F. Bunea, A.B. Tsybakov, and M.H. Wegkamp. Sparse density estimation with L1 penalties. In N.H. Bshouty and C. Gentile, editors, *COLT*, volume 4539 of *Lecture Notes in Computer Science*, Berlin Heidelberg New York, 2007b. Springer.

Centers for Disease Control and Prevention. 1993 revised classification system for HIV infection and expanded surveillance case definition for AIDS among adolescents and adults. *Morbid Mortal W Rep*, 41:1–19, 1992.

P. Chaffee, A.E. Hubbard, and M.J. van der Laan. Permutation-based pathway testing using the super learner algorithm. Technical Report 263, Division of Biostatistics, University of California, Berkeley, 2010.

A. Chambaz and M.J. van der Laan. Targeting the optimal design in randomized clinical trials with binary outcomes and no covariate. Technical Report 258, Division of Biostatistics, University of California, Berkeley, 2010.

C.-C. Chang and C.-J. Lin. *LIBSVM: A library for support vector machines*, 2001. URL http://www.csie.ntu.edu.tw/ cjlin/libsvm.

H.A. Chipman and R.E. McCulloch. *BayesTree: Bayesian methods for tree-based models*, 2009. URL http://CRAN.R-project.org/package=BayesTree. R package version 0.3-1.

H.A. Chipman, E.I. George, and R.E. McCulloch. BART: Bayesian additive regression trees. *Ann Appl Stat*, 4(1):266–298, 2010.

N.A. Christakis and T.I. Iwashyna. The health impact of health care on families: A matched cohort study of hospice use by decedents and mortality outcomes in surviving, widowed spouses. *Soc Sci Med*, 57(3):465–475, 2003.

S. Chu. Pricing the C's of diamond stones. *J Stat Educ*, 9(2), 2001.

E. Clappier, W. Cuccuini, A. Kalota, A. Crinquette, J.M. Cayuela, W.A. Dik, A.W. Langerak, B. Montpellier, B. Nadel, P. Walrafen, O. Delattre, A. Aurias, T. Leblanc, H. Dombret, A.M. Gewirtz, A. Baruchel, F. Sigaux, and J. Soulier. The C-MYB locus is involved in chromosomal translocation and genomic duplications in human t-cell acute leukemia (T-ALL), the translocation defining a new T-ALL subtype in very young children. *Blood*, 110(4):1251–61, 2007.

W.S. Cleveland, E. Groose, and W.M. Shyu. Local regression models. In J.M. Chambers and T.J. Hastie, editors, *Statistical Models in S*. Chapman & Hall, Boca Raton, 1992.

W.G. Cochran. Matching in analytical studies. *Am J Public Health*, 43:684–691, 1953.

W.G. Cochran. Analysis of covariance: Its nature and uses. *Biometrics*, 13:261–281, 1957.

W.G. Cochran. The planning of observational studies of human populations. *J R Stat Soc Ser A Gen*, 128(2):234–266, 1965.

W.G. Cochran. *Planning and Analysis of Observational Studies*. Wiley, New York, 1983. Edited posthumously by L.E. Moses and F. Mosteller.

W.G. Cochran and Donald B. Rubin. Controlling bias in observational studies: A review. *Sankhya Ser A*, 35:417–446, 1973.

S.R. Cole and M.A. Hernan. Constructing inverse probability weights for marginal structural models. *Am J Epidemiol*, 168:656–664, 2008.

D. Cook. *Regression Graphics: Ideas for Studying Regression Through Graphics*. Wiley, New York, 1998.

D. Cook and S. Weisberg. *An Introduction to Regression Graphics*. Wiley, New York, 1994.

J. Cornfield. A method of estimating comparative rates from clinical data. applications to cancer of the lung, breast, and cervix. *J Nat Cancer Inst*, 11:1269–1275, 1951.

J. Cornfield. A statistical problem arising from retrospective studies. In J. Neyman, editor, *Proceedings of the 3rd Berkeley symposium, Vol IV*, Berkeley, 1956. University of California Press.

C. Cortes and V. Vapnik. Support-vector networks. *Mach Learn*, 20:273–297, December 1995.

J.P. Costantino, M.H. Gail, D. Pee, S. Anderson, C.K. Redmond, J. Benichou, and H.S. Wieand. Validation studies for models projecting the risk of invasive and total breast cancer incidence. *J Natl Cancer Inst*, 91(18):1541–1548, 1999.

M.C. Costanza. Matching. *Prev Med*, 24:425–433, 1995.

D.R. Cox. *Planning of Experiments*. Wiley, New York, 1958.

R.K. Crump, V.J. Hotz, G.W. Imbens, and O.A. Mitnik. Moving the goalposts: Addressing limited overlap in the estimation of average treatment effects by changing the estimand. Technical Report 330, National Bureau of Economic Research, 2006.

A.S. Dalalyan and A.B. Tsybakov. Aggregation by exponential weighting and sharp oracle inequalities. In N.H. Bshouty and C. Gentile, editors, *COLT*, volume 4539 of *Lecture Notes in Computer Science*, Berlin Heidelberg New York, 2007. Springer.

A.S. Dalalyan and A.B. Tsybakov. Aggregation by exponential weighting, sharp pac-Bayesian bounds and sparsity. *Mach Learn*, 72(1–2):39–61, 2008.

E. de la Rochebrochard, N. Soullier, R. Peikrishvili, J. Guibert, and J. Bouyer. High in vitro fertilization discontinuation rate in France. *Int J Gynecol Obstet*, 103: 74–75, 2008.

E. de la Rochebrochard, C. Quelen, R. Peikrishvili, J. Guibert, and J. Bouyer. Long-term outcome of parenthood project during in vitro fertilization and after discontinuation of unscuccessful in vitro fertilization. *Fertil Steril*, 92:149–156, 2009.

R. Dehejia and S. Wahba. Causal effects in nonexperimental studies: Reevaluating the evaluation of training programs. *J Am Stat Assoc*, 94:1053–1062, 1999.

R. Dehejia and S. Wahba. Propensity score matching methods for nonexperimental causal studies. *Rev Econ Stat*, 84(1):151–161, 2002.

Department of Health and Human Services. Guidelines for the use of antiretroviral agents in HIV-1 infected adults and adolescents. Technical Report, Panel on Clinical Practices for Treatment of HIV Infection, 2004.

E. Dimitriadou, K. Hornik, F. Leisch, D. Meyer, and A. Weingessel. *e1071: Misc functions of the Department of Statistics (e1071)*, 2009. URL http://CRAN.R-project.org/package=e1071. R package version 1.5-22.

T.A. Diprete and H. Engelhardt. Estimating causal effects with matching methods in the presence and absence of bias cancellation. *Sociol Meth Res*, 32(4):501–528, 2004.

S. Dudoit and M.J. van der Laan. Asymptotics of cross-validated risk estimation in estimator selection and performance assessment. *Stat Methodol*, 2(2):131–154, 2005.

S. Dudoit and M.J. van der Laan. *Resampling Based Multiple Testing with Applications to Genomics*. Springer, Berlin Heidelberg New York, 2008.

S. Dudoit, J. Fridlyand, and T. P. Speed. Comparison of discrimination methods for the classification of tumors using gene expression data. *J Am Stat Assoc*, 97(457): 77–87, 2002.

B. Efron. Better bootstrap confidence intervals. *J Am Stat Assoc*, 82(397):171–185, 1987.

B. Efron and T. Hastie. lars. R package, 2007.

B. Efron and R. J. Tibshirani. *An Introduction to the Bootstrap*. Chapman & Hall, Boca Raton, 1993.

B. Efron, T. Hastie, I. Johnstone, and R. Tibshirani. Least angle regression. *Ann Stat (with discussion)*, 32(2):407–499, 2004.

S.S. Emerson. Issues in the use of adaptive clinical trial designs. *Stat Med*, 25: 3270–3296, 2006.

Food and Drug Administration. Critical path opportunities list. Technical Report, U.S. Department of Health and Human Services, Food and Drug Administration, 2006.

D.A. Freedman. *Statistical Models: Theory and Practice*. Cambridge, New York, 2005.

D.A. Freedman. Statistical models for causation: What inferential leverage do they provide? *Eval Rev*, 30:691–713, 2006.

D.A. Freedman. On regression adjustments to experimental data. *Adv Appl Math*, 40:180–193, 2008a.

D.A. Freedman. On regression adjustments to experiments with several treatments. *Ann Appl Stat*, 2:176–96, 2008b.

D.A. Freedman. Randomization does not justify logistic regression. *Stat Sci*, 23: 237–249, 2008c.

D.A. Freedman and R.A. Berk. Weighting regressions by propensity scores. *Eval Rev*, 3:392–409, 2008.

D.A. Freedman, D.B. Petitti, and J.M. Robins. On the efficacy of screening for breast cancer. *Int J Epidemiol*, 33:43–55, 2004.

D.H. Freedman. *Wrong: Why Experts Keep Failing Us—And How to Know When Not to Trust Them*. Little, Brown and Company, New York, 2010.

R. Freedman. Incomplete matching in ex post facto studies. *Am J of Soc*, 55(5): 485–487, 1950.

J.H. Friedman. Multivariate adaptive regression splines. *Ann Stat*, 19(1):1–141, 1991.

J.H. Friedman. Flexible metric nearest neighbor classification. Technical Report, Department of Statistics, Stanford University, 1994.

J.H. Friedman. Greedy function approximation: A gradient boosting machine. *Ann Stat*, 29:1189–1232, 2001.

J.H. Friedman, T.J. Hastie, and R.J. Tibshirani. Regularization paths for generalized linear models via coordinate descent. *J Stat Softw*, 33(1), 2010a.

J.H. Friedman, T.J. Hastie, and R.J. Tibshirani. *glmnet: Lasso and elastic-net regularized generalized linear models*, 2010b. URL http://CRAN.R-project.org/package=glmnet. R package version 1.1-5.

D.A. Fruman, S.B. Snapper, C.M. Yballe, L. Davidson, J.Y. Yu, F.W. Alt, and L.C. Cantley. Impaired B cell development and proliferation in absence of phosphoinositide 3-kinase p85-alpha. *Science*, 283:393–397, 1999.

T. Fujii, S. Nomoto, K. Koshikawa, Y.Yatabe, O. Teshigawara, T. Mori, S. Inoue, S. Takeda, and A. Nakao. Overexpression of pituitary tumor transforming gene 1 in HCC is associated with angiogenesis and poor prognosis. *Hepatology*, 43: 1267–1275, 2006.

M.H. Gail. Adjusting for covariates that have the same distribution in exposed and unexposed cohorts. In S. H. Moolvankar and R. L. Prentice, editors, *Modern Statistical Methods in Chronic Disease Epidemiology*. Wiley, New York, 1986.

M.H. Gail, L.A. Brinton, D.P. Byar, D.K. Corle, S.B. Green, C. Schairer, and J.J. Mulvihill. Projecting individualized probabilities of developing breast cancer for white females who are being examined annually. *J Natl Cancer Inst*, 81(24): 1879–1886, 1989.

S. Galiani, P. Gertler, and E. Schargrodsky. Water for life: The impact of the privatization of water services on child mortality. *J Polit Econ*, 113(1):83–120, 2005.

Y. Ge and S. Dudoit. Multiple testing procedures, multtest. R package, 2002. URL www.bioconductor.org.

S. Geisser. The predictive sample reuse method with applications. *J Am Stat Assoc*, 70(350):320–328, 1975.

A. Gelman, A. Jakulin, M.G. Pittau, and Y.-S. Su. A weakly informative default prior distribution for logistic and other regression models. *Ann Appl Stat*, 2(3): 1360–1383, 2009.

A. Gelman, Y.-S. Su, M. Yajima, J. Hill, M.G. Pittau, J. Kerman, and T. Zheng. *arm: Data analysis using regression and multilevel/hierarchical models*, 2010. URL http://CRAN.R-project.org/package=arm. R package version 1.3-02.

R.D. Gill. Non- and semiparametric maximum likelihood estimators and the von mises method (part 1). *Scand J Stat*, 1989.

R.D. Gill and J.M. Robins. Causal inference in complex longitudinal studies: Continuous case. *Ann Stat*, 29(6):1785–1811, 2001.

H.L. Golub. The need for more efficient trial designs. *Stat Med*, 25:3231–3235, 2006.

T.R. Golub, D.K. Slonim, P. Tamayo, C. Huard, M. Gaasenbeek, J.P. Mesirov, H. Coller, M.L. Loh, J.R. Downing, M.A. Caligiuri, C.D. Bloomfield, and E.S.

Lander. Molecular classification of cancer: Class discovery and class prediction by gene expression monitoring. *Science*, 286:531–537, 1999.

S. Greenland. Multivariate estimation of exposure-specific incidence from case-control studies. *J Chron Dis*, 34:445–453, 1981.

S. Greenland. Model-based estimation of relative risks and other epidemiologic measures in studies of common outcomes and in case-control studies. *Am J Epidemiol*, 160(4):301–305, 2004.

S. Gruber and M.J. van der Laan. An application of collaborative targeted maximum likelihood estimation in causal inference and genomics. *Int J Biostat*, 6(1), 2010a.

S. Gruber and M.J. van der Laan. A targeted maximum likelihood estimator of a causal effect on a bounded continuous outcome. *Int J Biostat*, 6(1):Article 26, 2010b.

S. Gruber and M.J. van der Laan. tmle: An R package for targeted maximum likelihood estimation. Technical Report 275, Division of Biostatistics, University of California, Berkeley, 2011.

L. Györfi, M. Kohler, A. Krzyżak, and H. Walk. *A Distribution-Free Theory of Nonparametric Regression*. Springer, Berlin Heidelberg New York, 2002.

L. Haignere. *Paychecks: A Guide to Conducting Salary-Equity Studies for Higher Education Faculty*. American Association of University Professors, Washington, DC, 2nd edition, 2002.

C.S. Haley and S.A. Knott. A simple regression method for mapping quantitative trait loci in line crosses using flanking markers. *Heredity*, 1992.

S.M. Hammer, J.J. Eron, P. Reiss, R.T. Schooley, M.A. Thompson, S. Walmsley, P. Cahn, M.A. Fischl, J.M. Gatell, M.S. Hirsch, D.M. Jacobsen, J.S.G. Montaner, D.D. Richman, P.G. Yeni, and P.A. Volberding. Antiretroviral treatment of adult HIV infection: 2008 recommendations of the International AIDS Society-USA panel. *J Am Med Asoc*, 300(5):555–70, 2008.

F.R. Hampel, E.M. Ronchetti, P.J. Rousseeuw, and W.A. Stahel. *Robust Statistics: The Approach Based on Influence Functions*. Wiley, New York, 1986.

B.B. Hansen. The prognostic analogue of the propensity score. *Biometrika*, 95: 481–8, 2008.

F.E. Harrell, Jr. *Regression Modeling Strategies with Applications to Linear models, Logistic Regression, and Survival Analysis*. Springer, Berlin Heidelberg New York, 2001.

T.J. Hastie. Generalized additive models. In J.M. Chambers and T.J. Hastie, editors, *Statistical Models in S*. Chapman & Hall, Boca Raton, 1992.

T.J. Hastie and R.J. Tibshirani. *Generalized Additive Models*. Chapman & Hall, Boca Raton, 1990.

T.J. Hastie, R.J. Tibshirani, and J.H. Friedman. *The Elements of Statistical Learning: Data Mining, Inference, and Prediction*. Springer, Berlin Heidelberg New York, 2001.

S.C. Heath. Markov chain Monte Carlo segregation and linkage analysis of oligogenic models. *Am J Hum Genet*, 1997.

J. Heckman. Causal parameters and policy analysis in economics: A twentieth century retrospective. *Q J Econ*, 115:45–97, 2000.

J. Heckman. Building bridges between structural and program evaluation approaches to evaluating policy. *J Econ Lit*, 48:356–398, 2010.

J. Heckman, H. Ichimura, and R. Todd. Matching as an econometric evaluation estimator: Evidence from evaluating a job training programme. *Rev of Econ Stud*, 64:605–654, 1997.

M.A. Hernan, B. Brumback, and J.M. Robins. Marginal structural models to estimate the causal effect of zidovudine on the survival of HIV-positive men. *Epidemiol*, 11(5):561–570, 2000.

M.A. Hernan, E. Lanoy, D. Costagliola, and J.M. Robins. Comparison of dynamic treatment regimes via inverse probability weighting. *Basic Clin Pharmacol*, 98: 237–242, 2006.

A.V. Hernández, M.J. Eijkemans, and E.W. Steyerberg. Randomized controlled trials with time-to-event outcomes: How much does prespecified covariate adjustment increase power? *Ann Epidemiol*, 16(1):41–48, 2006.

M.C. Herron and J. Wand. Assessing partisan bias in voting technology: The case of the 2004 New Hampshire recount. *Elect Stud*, 26(2):247–261, 2007.

T.R. Holford, C. White, and J.L. Kelsey. Multivariate analysis for matched case-control studies. *Am J Epidemiol*, 107(3):245–255, 1978.

J.H. Holland and J.S. Reitman. Cognitive systems based on adaptive algorithms. *SIGART Bull*, 63:49–49, 1977.

P.W. Holland. Statistics and causal inference. *J Am Stat Assoc*, 81(396):945–960, 1986.

P.W. Holland. Comment: Causal mechanism or causal effect: Which is best for statistical science? *Stat Sci*, 3(2):186–188., 1988.

P.W. Holland and Donald B. Rubin. Causal inference in retrospective studies. In Donald B. Rubin, editor, *Matched Sampling for Causal Effects.* Cambridge, Cambridge, MA, 1988.

T. Hothorn, P. Buhlmann, S. Dudoit, A.M. Molinaro, and M.J. van der Laan. Survival ensembles. *Biostatistics*, 7:355–373, 2006.

F. Hu and W.F. Rosenberger. *The Theory of Response Adaptive Randomization in Clinical Trials*. Wiley, New York, 2006.

A.E. Hubbard, M.J. van der Laan, and J.M. Robins. Nonparametric locally efficient estimation of the treatment specific survival distributions with right censored data and covariates in observational studies. In D. Berry E. Halloran, editor, *Statistical Models in Epidemiology: The Environment and Clinical Trials*. Springer, Berlin Heidelberg New York, 1999.

S.P. Hunger. Chromosomal translocations involving the E2A gene in acute lymphoblastic leukemia: Clinical features and molecular pathogenesis. *Blood*, 87: 1211–1224, 1996.

K. Imai. Do get-out-the-vote calls reduce turnout? the importance of statistical methods for field experiments. *Am Polit Sci Rev*, 99(2):283–300, 2005.

J.P.A. Ioannidis. Why most published research findings are false. *Neonatal Intensive Care J Perinatol Neonatol*, 19(3), 2006.

H. Ishwaran, U.B. Kogalur, E.H. Blackstone, and M.S. Lauer. Random survival forests. *Ann Appl Stat*, 2(3):841–860, 2008.

R. Jackson. Updated new zealand cardiovascular disease risk-benefit prediction guide. *Br Med J*, 320(7236):709–710, 2000.

R.C. Jansen. Interval mapping of multiple quantitative trait loci. *Genetics*, 1993.

C. Jennison and B.W. Turnbull. *Group Sequential Methods with Applications to Clinical Trials*. Chapman & Hall, Boca Raton, 2000.

N.P. Jewell. *Statistics for Epidemiology*. Chapman & Hall, Boca Raton, 2004.

H. Jiang, J. Symanowski, S. Paul, Y. Qu, A. Zagar, and S. Hong. The type I error and power of nonparametric logrank and Wilcoxon tests with adjustment for covariates – a simulation study. *Stat Med*, 27(28):5850–5860, 2008.

C. Jin, J.P. Fine, and B.S. Yandell. A unified semiparametric framework for quantitative trait loci analysis, with application to spike phenotypes. *J Am Stat Assoc*, 2007.

V.A. Johnson, F. Brun-Vezinet, B. Clotet, H.F. Gunthard, D.R. Kuritzkes, D. Pillay, J.M. Schapiro, and D.D. Richman. Update of the drug resistance mutations in HIV-1: December 2009. *Top HIV Med*, 17(5):138–45, 2009.

A. Juditsky, A.V. Nazin, A.B. Tsybakov, and N. Vayatis. Generalization error bounds for aggregation by mirror descent with averaging. In *NIPS*, 2005.

J. Kang and J.L. Schafer. Demystifying double robustness: A comparison of alternative strategies for estimating a population mean from incomplete data (with discussion). *Stat Sci*, 22:523–39, 2007.

W.B. Kannel, D. McGee, and T. Gordon. A general cardiovascular risk profile: The Framingham study. *Am J Cardiol*, 38:46–51, 1976.

C.H. Kao, Z.B. Zeng, and R.D. Teasdale. Multiple interval mapping for quantitative trait loci. *Genetics*, 1999.

S.H. Kaufmann, S.D. Gore, C.B. Miller, R.J. Jones, L.A. Zwelling, E. Schneider, P.J. Burke, and J.E. Karp. Topoisomerase II and the response to antileukemic therapy. *Leukemia Lymphoma*, 29(3-4):217–237, Apr 1998a.

S.H. Kaufmann, J.E. Karp, P.A. Svingen, S. Krajewski, P.J. Burke, S.D. Gore, and J.C. Reed. Elevated expression of the apoptotic regulator Mcl-1 at the time of leukemic relapse. *Blood*, 91(3):991–1000, 1998b.

N. Keiding, C. Hols, and A. Green. Retrospective estimation of diabetes incidence from information in a current prevalent population and historical mortality. *Am J Epidemiol*, 130:588–600, 1989.

S. Keleş, M.J. van der Laan, and S. Dudoit. Asymptotically optimal model selection method for regression on censored outcomes. Technical Report 124, Division of Biostatistics, University of California, Berkeley, 2002.

D. Kibler, D.W. Aha, and M.K. Albert. Instance-based prediction of real-valued attributes. *Comput Intell*, 5:51, 1989.

I. Kirsch, B.J. Deacon, T.B. Huedo-Medina, A. Scoboria, T.J. Moore, and B.T. Johnson. Initial severity and antidepressant benefits: A meta-analysis of data submitted to the Food and Drug Administration. *PLoS Med*, 5(2):e45. doi:10.1371/journal.pmed.0050045, 2008.

L. Kish. Weighting for unequal p_i. *J Off Stat*, 8:183–200, 1992.

G.G. Koch, C.M. Tangen, J.W. Jung, and I.A. Amara. Issues for covariance analysis of dichotomous and ordered categorical data from randomized clinical trials and nonparametric strategies for addressing them. *Stat Med*, 17:1863–1892, 1998.

I. Komuro, T. Yasuda, A. Iwamoto, and K.S. Akagawa. Catalase plays a critical role in the CSF-independent survival of human macrophages via regulation of the expression of BCL-2 family. *J Biol Chem*, 280(50):41137–45, 2005.

C. Kooperberg. *polspline: Polynomial spline routines*, 2009. URL http://CRAN.R-project.org/package=polspline. R package version 1.1.4.

C. Kooperberg, C.J. Stone, and Y.K. Truong. Hazard regression. *J Am Stat Assoc*, 90(429):78–94, 1995.

J. Kos and T.T. Lah. Cysteine proteinases and their endogenous inhibitors: Target proteins for prognosis, diagnosis and therapy in cancer (review). *Oncol Rep*, 5 (6):1349–61, 1998.

H. Koul, V. Susarla, and J. van Ryzin. Regression analysis with randomly right censored data. *Ann Stat*, 9:1276–88, 1981.

K. Kozar, M.A. Ciemerych, V.I. Rebel, H. Shigematsu, A. Zagozdzon, E. Sicinska, Y. Geng, Q. Yu, S. Bhattacharya, R.T. Bronson, K. Akashi, and P. Sicinski. Mouse development and cell proliferation in the absence of D-cyclins. *Cell*, 118:477–491, 2004.

L.L. Kupper, J.M. Karon, D.G. Kleinbaum, H. Morgenstern, and D.K. Lewis. Matching in epidemiologic studies: Validity and efficiency considerations. *Biometrics*, 37:271–291, 1981.

R.J. LaLonde. Evaluating the econometric evaluations of training programs with experimental data. *Am Econ Rev*, 76:604–620, 1986.

E.S. Lander and D. Botstein. Mapping Mendelian factors underlying quantitative traits using RFLP linkage maps. *Genetics*, 1989.

A.A. Lane and T.J. Ley. Neutrophil elastase cleaves PML-RAR-alpha and is important for the development of acute promyelocytic leukemia in mice. *Cell*, 115 (305-318), 2003.

M. LeBlanc and J. Crowley. Relative risk trees for censored data. *Biometrics*, 48: 411–425, 1992.

M. LeBlanc and R.J. Tibshirani. Combining estimates in regression and classification. *J Am Stat Assoc*, 91:1641–1650, 1996.

S.S.F. Lee, L. Sun, R. Kustra, and S.B. Bull. EM-random forest and new measures of variable importance for multi-locus quantitative trait linkage analysis. *Bioinformatics*, 2008.

E.L. Lehmann. *Testing Statistical Hypotheses*. Springer, Berlin Heidelberg New York, 2nd edition, 1986.

S. Leon, A.A. Tsiatis, and M. Davidian. Semiparametric estimation of treatment effect in a pretest-posttest study. *Biometrics*, 59:1046–1055, 2003.

G. Leone, R. Sears, E. Huang, R. Rempel, F. Nuckolls, C.H. Park, P. Giangrande, L. Wu, H.I. Saavedra, S.J. Field, M.A. Thompson, H. Yang, Y. Fujiwara, M.E. Greenberg, S. Orkin, C. Smith, and J.R. Nevins. Myc requires distinct E2F activities to induce S phase and apoptosis. *Mol Cell*, 8:105–113, 2001.

A. Liaw and M. Wiener. Classification and regression by randomforest. *R News*, 2 (3):18–22, 2002. URL http://CRAN.R-project.org/package=randomForest.

D.V. Lindley. *Introduction to Probability and Statistics from a Bayesian Point of View, Part 2*. Cambridge, Cambridge, MA, 1980.

R.J.A. Little and Donald B. Rubin. *Statistical Analysis with Missing Data*. Wiley, Hoboken, 2nd edition, 2002.

Z. Liu and T. Stengos. Nonlinearities in cross country growth regressions: A semiparametric approach. *J of Appl Econom*, 14:527–538, 1999.

C.L. Loprinzi, J.A. Laurie, H.S. Wieand, J.E. Krook, P.J. Novotny, J.W. Kugler, J. Bartel, M. Law, M. Bateman, and N.E. Klatt. Prospective evaluation of prognostic variables from patient-completed questionnaires, North Central Cancer Treatment Group. *J Clin Oncol*, 12(3):601–607, 1994.

X. Lu and A.A Tsiatis. Improving the efficiency of the log-rank test using auxiliary covariates. *Biometrika*, 95(3):679–694, 2008.

D. MacKinnon. *Introduction to Statistical Mediation Analysis*. Erlbaum, New York, 2008.

D. MacKinnon, C. Lockwood, C. Brown, W. Wang, and J. Hoffman. The intermediate endpoint effect in logistic and probit regression. *Clin Trials*, 4:499–513, 2007.

R. Mansson, M.M. Joffe, W. Sun, and S. Hennessy. On the estimation and use of propensity scores in case-control and case-cohort studies. *Am J Epidemiol*, 166 (3):332–339, 2007.

J. Marschak. Studies in econometric method. In W. C. Hood and T. C. Koopmans, editors, *Economic Measurements for Policy and Prediction*. Wiley, New York, 1953.

J.I. Martin-Subero, R. Ibbotson, W. Klapper, L. Michaux, E. Callet-Bauchu, F. Berger, M.J. Calasanz, C. De Wolf-Peeters, M.J. Dyer, P. Felman, A. Gardiner, R.D. Gascoyne, S. Gesk, L. Harder, D.E. Horsman, M. Kneba, R. Kuppers, A. Majid, N. Parry-Jones, M. Ritgen, M. Salido, F. Sole, G. Thiel, H.H. Wacker, D. Oscier, I. Wlodarska, and R. Siebert. A comprehensive genetic and histopathologic analysis identifies two subgroups of B-cell malignancies carrying a t(14;19)(q32;q13) or variant BCL3-translocation. *Leukemia*, 21(7):1532–1544, 2007.

G.L. Masinde, X. Li, W. Gu, H. Davidson, S. Mohan, and D.J. Baylink. Identification of wound healing/regeneration quantitative trait loci (QTL) at multiple time points that explain seventy percent of variance in (MRL/MpJ and SJL/J) mice F2 population. *Genome Res*, 2001.

P. McCullagh. Quasi-likelihood functions. *Ann Stat*, 11:59–67, 1983.

P. McCullagh and J.A. Nelder. *Generalized Linear Models*. Chapman & Hall, Boca Raton, 2nd edition, 1989.

S.M. McKinlay. Pair-matching – a reappraisal of a popular technique. *Biometrics*, 33(4):725–735, 1977.

K.L. Moore and M.J. van der Laan. Covariate adjustment in randomized trials with binary outcomes. Technical Report 215, Division of Biostatistics, University of California, Berkeley, April 2007.

K.L. Moore and M.J. van der Laan. Application of time-to-event methods in the assessment of safety in clinical trials. In Karl E. Peace, editor, *Design, Summarization, Analysis & Interpretation of Clinical Trials with Time-to-Event Endpoints*, Boca Raton, 2009a. Chapman & Hall.

K.L. Moore and M.J. van der Laan. Covariate adjustment in randomized trials with binary outcomes: Targeted maximum likelihood estimation. *Stat Med*, 28(1):39–64, 2009b.

K.L. Moore and M.J. van der Laan. Increasing power in randomized trials with right censored outcomes through covariate adjustment. *J Biopharm Stat*, 19(6): 1099–1131, 2009c.

K.L. Moore, R.S. Neugebauer, M.J. van der Laan, and I.B. Tager. Causal inference in epidemiological studies with strong confounding. Technical Report 255, Division of Biostatistics, University of California, Berkeley, 2009.

S.L. Morgan and D.J. Harding. Matching estimators of causal effects: Prospects and pitfalls in theory and practice. *Sociol Meth Res*, 35(1):3–60, 2006.

A.P. Morise, G.A. Diamon, R. Detrano, M. Bobbio, and Erdogan Gunel. The effect of disease-prevalence adjustments on the accuracy of a logistic prediction model. *Med Decis Making*, 16:133–142, 1996.

K.M. Mortimer, R. Neugebauer, M.J. van der Laan, and I.B. Tager. An application of model-fitting procedures for marginal structural models. *Am J Epidemiol*, 162 (4):382–388, 2005.

A.R. Moss, J.A. Hahn, S. Perry, E.D. Charlebois, D. Guzman, R.A. Clark, and D.R. Bangsberg. Adherence to highly active antiretroviral therapy in the homeless population in San Francisco: A prospective study. *Clin Infect Dis*, 39(8):1190–1198, 2004.

K. Nebral, H.H. Schmidt, O.A. Haas, and S. Strehl. NUP98 is fused to topoisomerase (DNA) IIbeta 180 kDa (TOP2B) in a patient with acute myeloid leukemia with a new t(3;11)(p24;p15). *Clin Cancer Res*, 11(18):6489–6494, 2005.

R. Neugebauer and J. Bullard. *DSA: Data-adaptive estimation with cross-validation and the D/S/A algorithm*, 2009. URL http://www.stat.berkeley.edu/ laan/Software/. R package version 3.1.3.

R. Neugebauer and M. J. van der Laan. Nonparametric causal effects based on marginal structural models. *J Stat Plan Infer*, 137(2):419–434, 2007.

R. Neugebauer and M.J. van der Laan. Why prefer double robust estimates. *J Stat Plan Infer*, 129(1-2):405–26, 2005.

R. Neugebauer, M.J. Silverberg, and M.J. van der Laan. Observational study and individualized antiretroviral therapy initiation rules for reducing cancer incidence in HIV-infected patients. 272, Division of Biostatistics, University of California, Berkeley, 2010.

D.J. Newman, S. Hettich, C.L. Blake, and C.J. Merz. UCI Repository of Machine Learning Databases, 1998.

S. Newman. Causal analysis of case-control data. *Epid Persp Innov*, 3:2, 2006.

J. Neyman. Sur les applications de la theorie des probabilites aux experiences agricoles: Essai des principes (In Polish). English translation by D.M. Dabrowska and T.P. Speed (1990). *Stat Sci*, 5:465–480, 1923.

T. Palomero, D.T. Odom, J. O'Neil, A.A. Ferrando, A. Margolin, D.S. Neuberg, S.S. Winter, R.S. Larson, W. Li, X.S. Liu, R.A. Young, and A.T. Look. Transcriptional regulatory networks downstream of TAL1/SCL in T-cell acute lymphoblastic leukemia. *Blood*, 108(3):986–992, 2006.

M. Pavlic and M.J. van der Laan. Fitting of mixtures with unspecified number of components using cross validation distance estimate. *Comput Stat Data An*, 41: 413–428, 2003.

J. Pearl. Causal diagrams for empirical research. *Biometrika*, 82:669–710, 1995.

J. Pearl. Direct and indirect effects. In *Uncertainty in Artificial Intelligence, Proceedings of the 17th Conference*, San Francisco, 2001. Morgan Kaufmann.

J. Pearl. *Causality: Models, Reasoning, and Inference*. Cambridge, New York, 2nd edition, 2009.

J. Pearl. On a class of bias-amplifying variables that endanger effect estimates. *Proceedings of Uncertainty in Artificial Intelligence*, 2010a.

J. Pearl. An introduction to causal inference. *Int J Biostat*, 6(2):Article 7, 2010b.

J. Pearl. The mediation formula: A guide to the assessment of causal pathways in nonlinear models. In C. Berzuini, P. Dawid, and L. Bernardinelli, editors, *Statistical Causality*. 2011.

K. Pearson. *Grammar of Science*. Black, London, 3rd edition, 1911.

K. Penrose, A. Nelson, and A. Fisher. Generalized body composition prediction equation for men using simple measurement techniques. *Med Sci Sport Exer*, 17: 189, 1985.

A. Peters and T. Hothorn. *ipred: Improved Predictors*, 2009. URL http://CRAN.R-project.org/package=ipred. R package version 0.8-8.

M.L Petersen, S.G. Deeks, J.N. Martin, and M.J. van der Laan. History-adjusted marginal structural models to estimate time-varying effect modification. *Am J Epidemiol*, 166(9):985–993, 2007a.

M.L. Petersen, S.G. Deeks, and M.J. van der Laan. Individualized treatment rules: Generating candidate clinical trials. *Stat Med*, 26(25):4578–601, 2007b.

M.L. Petersen, M.J. van der Laan, S. Napravnik, J.J. Eron, R.D. Moore, and S.G. Deeks. Long-term consequences of the delay between virologic failure of highly active antiretroviral therapy and regimen modification. *AIDS*, 22(16):2097–106, 2008.

M.L. Petersen, K.E. Porter, S. Gruber, Y. Wang, and M.J. van der Laan. Diagnosing and responding to violations in the positivity assumption. *Stat Meth Med Res*, published online 28 Oct (doi: 10.1177/0962280210386207), 2010.

S.J. Pocock, S.E. Assmann, L.E. Enos, and L.E. Kasten. Subgroup analysis, covariate adjustment, and baseline comparisons in clinical trial reporting: Current practice and problems. *Stat Med*, 21:2917–2930, 2002.

E.C. Polley and M.J. van der Laan. Predicting optimal treatment assignment based on prognostic factors in cancer patients. In K.E. Peace, editor, *Design, Summarization, Analysis & Interpretation of Clinical Trials with Time-to-Event Endpoints*, Boca Raton, 2009. Chapman & Hall.

E.C. Polley and M.J. van der Laan. Super learner in prediction. Technical Report 266, Division of Biostatistics, University of California, Berkeley, 2010.

R.L. Prentice and N.E. Breslow. Retrospective studies and failure time models. *Biometrika*, 65(1):153–158, 1978.

R.L. Prentice and R. Pyke. Logistic disease incidence models and case-control studies. *Biometrika*, 66:403–411, 1979.

R. Puri, A. Tousson, L. Chen, and S.S. Kakar. Molecular cloning of pituitary tumor transforming gene 1 from ovarian tumors and its expression in tumors. *Cancer Lett*, 163:131–139, 2001.

R Development Core Team. *R: A language and environment for statistical computing*. R Foundation for Statistical Computing, Vienna, 2010. URL http://www.R-project.org.

S. Ramaswamy, K.N. Ross, E.S. Lander, and T.R. Golub. A molecular signature of metastasis in primary solid tumors. *Nat Genet*, 33:49–54, 2003.

L.E. Ramsay, I.U. Haq, P.R. Jackson, and W.W. Yeo. Sheffield risk and treatment table for cholesterol lowering for primary prevention of coronary heart disease. *Lancet*, 346(8988):1467–1471, 1995.

L.E. Ramsay, I.U. Haq, P.R. Jackson, and W.W. Yeo. The Sheffield table for primary prevention of coronary heart disease: Corrected. *Lancet*, 348(9036):1251, 1996.

S.J. Reynolds, G. Nakigozi, K. Newell, A. Ndyanabo, R. Galiwongo, I. Boaz, T.C. Quinn, R. Gray, M. Wawer, and D. Serwadda. Failure of immunologic criteria to appropriately identify antiretroviral treatment failure in Uganda. *AIDS*, 23(6): 697–700, 2009.

G. Ridgeway. *gbm: Generalized boosted regression models*, 2007. R package version 1.6-3.

B.D. Ripley. *Pattern Recognition and Neural Networks*. Cambridge, New York, 1996.

J. Rissanen. Modelling by shortest data description. *Automatica*, 14:465–471, 1978.

P.C. Robert. *The Bayesian Choice: From Decision-Theoretic Foundations to Computational Implementation*. Springer, Berlin Heidelberg New York, 2007.

J.M. Robins. A new approach to causal inference in mortality studies with sustained exposure periods–application to control of the healthy worker survivor effect. *Math Mod*, 7:1393–1512, 1986.

J.M. Robins. Addendum to: "A new approach to causal inference in mortality studies with a sustained exposure period—application to control of the healthy worker survivor effect". *Comput Math Appl*, 14(9–12):923–945, 1987a.

J.M. Robins. A graphical approach to the identification and estimation of causal parameters in mortality studies with sustained exposure periods. *J Chron Dis (40, Supplement)*, 2:139s–161s, 1987b.

J.M. Robins. Information recovery and bias adjustment in proportional hazards regression analysis of randomized trials using surrogate markers. In *Proceedings of the Biopharmaceutical Section*. American Statistical Association, 1993.

J.M. Robins. Correcting for noncompliance in randomized trials using structural nested mean models. *Commun Stat*, 23:2379–2412, 1994.

J.M. Robins. Marginal structural models. *1997 Proceedings of the American Statistical Association. Section on Bayesian Statistical Science*, pages 1–10, 1998.

J.M. Robins. [Choice as an alternative to control in observational studies]: Comment. *Stat Sci*, 14(3):281–293, 1999a.

J.M. Robins. Marginal structural models versus structural nested models as tools for causal inference. In *Statistical Models in Epidemiology: The Environment and Clinical Trials*. Springer, Berlin Heidelberg New York, 1999b.

J.M. Robins. Robust estimation in sequentially ignorable missing data and causal inference models. In *Proceedings of the American Statistical Association*, 2000.

J.M. Robins and S. Greenland. Identifiability and exchangeability for direct and indirect effects. *Epidemiol*, 3:143–155, 1992.

J.M. Robins and A. Rotnitzky. Recovery of information and adjustment for dependent censoring using surrogate markers. In *AIDS Epidemiology*. Birkhäuser, Basel, 1992.

J.M. Robins and A. Rotnitzky. Semiparametric efficiency in multivariate regression models with missing data. *J Am Stat Assoc*, 90:122–129, 1995.

J.M. Robins and A. Rotnitzky. Comment on the Bickel and Kwon article, "Inference for semiparametric models: Some questions and an answer". *Stat Sinica*, 11(4): 920–936, 2001.

J.M. Robins and N. Wang. Inference for imputation estimators. *Biometrika*, 87: 113–124, 2000.

J.M. Robins, S.D. Mark, and W.K. Newey. Estimating exposure effects by modelling the expectation of exposure conditional on confounders. *Biometrics*, 48(479–495), 1992.

J.M. Robins, A. Rotnitzky, and L.P. Zhao. Estimation of regression coefficients when some regressors are not always observed. *J Am Stat Assoc*, 89(427):846–866, 1994.

J.M. Robins, A. Rotnitzky, and L.P. Zhao. Analysis of semiparametric regression models for repeated outcomes in the presence of missing data. *J Am Stat Assoc*, 90:106–121, 1995.

J.M. Robins, M.A. Hernan, and B. Brumback. Marginal structural models and causal inference in epidemiology. *Epidemiol*, 11(5):550–560, 2000a.

J.M. Robins, A. Rotnitzky, and M.J. van der Laan. Comment on "On profile likelihood". *J Am Stat Assoc*, 450:431–435, 2000b.

J.M. Robins, M.A. Hernan, and A. Rotnitzky. Effect modification by time-varying covariates. *Am J Epidemiol*, 166(9):994–1002, 2007a.

J.M. Robins, M. Sued, Q. Lei-Gomez, and A. Rotnitzky. Comment: Performance of double-robust estimators when "inverse probability" weights are highly variable. *Stat Sci*, 22:544–559, 2007b.

J.M. Robins, L. Orellana, and A. Rotnitzky. Estimation and extrapolation of optimal treatment and testing strategies. *Stat Med*, 27:4678–4721, 2008.

A.J. Rodger, Z. Fox, J.D. Lundgren, J.L. Kuller, C. Boesecke, D. Gey, A. Skoutelis, M.B. Goetz, A.N. Phillips, and INSIGHT Strategies for Management of Antiretroviral Therapy (SMART) Study Group. Activation and coagulation biomarkers are independent predictors of the development of opportunistic disease in patients with HIV infection. *J Infect Dis*, 200(6):973–83, 2009.

S. Rose and M.J. van der Laan. Simple optimal weighting of cases and controls in case-control studies. *Int J Biostat*, 4(1):Article 19, 2008.

S. Rose and M.J. van der Laan. Why match? Investigating matched case-control study designs with causal effect estimation. *Int J Biostat*, 5(1):Article 1, 2009.

S. Rose and M.J. van der Laan. A targeted maximum likelihood estimator for two-stage designs. *Int J Biostat*, 7(1):Article 17, 2011.

S. Rose, J.M. Snowden, and K.M. Mortimer. Rose et al. respond to "G-computation and standardization in epidemiology". *Am J Epidemiol*, 173(00):000–000, 2011.

P.R. Rosenbaum. *Observational Studies*. Springer, Berlin Heidelberg New York, 2nd edition, 2002.

P.R. Rosenbaum and Donald B. Rubin. The central role of the propensity score in observational studies for causal effects. *Biometrika*, 70:41–55, 1983.

W.F. Rosenberger. New directions in adaptive designs. *Stat Sci*, 11:137–149, 1996.

W.F. Rosenberger, A.N. Vidyashankar, and D.K. Agarwal. Covariate-adjusted response-adaptive designs for binary response. *J Biopharm Stat*, 11(227–236), 2001.

M. Rosenblum and M.J. van der Laan. Using regression models to analyze randomized trials: Asymptotically valid hypothesis tests despite incorrectly specified models. *Biometrics*, 65(3):937–945, 2009a.

M. Rosenblum and M.J. van der Laan. Confidence intervals for the population mean tailored to small sample sizes, with applications to survey sampling. *Int J Biostat*, 1:Article 4, 2009b.

M. Rosenblum and M.J. van der Laan. Targeted maximum likelihood estimation of the parameter of a marginal structural model. *Int J Biostat*, 6(2):Article 19, 2010a.

M. Rosenblum and M.J. van der Laan. Simple, efficient estimators of treatment effects in randomized trials using generalized linear models to leverage baseline variables. *Int J Biostat*, 6(1):Article 13, 2010b.

M. Rosenblum, S.G. Deeks, M.J. van der Laan, and D.R. Bangsberg. The risk of virologic failure decreases with duration of HIV suppression, at greater than 50% adherence to antiretroviral therapy. *PLoS ONE*, 4(9): e7196.doi:10.1371/journal.pone.0007196, 2009.

B. Rosner. *Fundamentals of Biostatistics*. Duxbury, Pacific Grove, 5th edition, 1999.

K.J. Rothman and S. Greenland. *Modern Epidemiology*. Lippincott, Williams & Wilkins, Philadelphia, 2nd edition, 1998.

Daniel B. Rubin and M.J. van der Laan. Empirical efficiency maximization: Improved locally efficient covariate adjustment in randomized experiments and survival analysis. *Int J Biostat*, 4(1):Article 5, 2008.

Donald B. Rubin. Estimating causal effects of treatments in randomized and nonrandomized studies. *J Educ Psychol*, 66:688–701, 1974.

Donald B. Rubin. Bayesian inference for causality: The importance of randomization. In *The Proceedings of the Social Statistics Section of the American Statistical Association*. American Statistical Association, Alexandria, 1975.

Donald B. Rubin. Multivariate matching methods that are equal percent bias reducing, II: Maximums on bias reduction for fixed sample sizes. *Biometrics*, 32(1): 121–132, 1976.

Donald B. Rubin. Bayesian inference for causal effects: The role of randomization. *Ann Stat*, 6:34–58, 1978.

Donald B. Rubin. Comment: Neyman (1923) and causal inference in experiments and observational studies. *Stat Sci*, 5(4):472–480, 1990.

Donald B. Rubin. Estimating causal effects from large data sets using propensity scores. *Ann of Intern Med*, 127(8S):757–763, 1997.

Donald B. Rubin. Using propensity scores to help design observational studies: Application to the tobacco litigation. *Health Serv Outcome Res Meth*, 2(1):169–188, 2002.

Donald B. Rubin. *Matched Sampling for Causal Effects.* Cambridge, Cambridge, MA, 2006.

Donald B. Rubin. For objective causal inference, design trumps analysis. *Ann of Appl Stat*, 2(3):808–840, 2008.

I. Ruczinski, C. Kooperberg, and M. LeBlanc. Logic regression – methods and software. In D. Denison, M. Hansen, C. Holmes, B. Mallick, and B. Yu, editors, *Proceedings of the MSRI workshop on Nonlinear Estimation and Classification*, pages 333–344, 2002.

K. Rudser, M. LeBlanc, and S.S. Emerson. Estimation for arbitrary functionals of survival. Technical Report 335, Department of Biostatistics, University of Washington, 2008.

J.M. Satagopan, B.S. Yandell, M.A. Newton, and T.C. Osborn. A Bayesian approach to detect quantitative trait loci using Markov chain Monte Carlo. *Genetics*, 1996.

K. Sax. The association of size difference with seed-coat pattern and pigmentation in *Phaseolus vulgaris*. *Genetics*, 1923.

J.L. Schafer. *Analysis of Incomplete Multivariate Data*. Chapman & Hall, London, 1997.

D.O. Scharfstein, A. Rotnitzky, and J.M. Robins. Adjusting for nonignorable drop-out using semiparametric nonresponse models, (with discussion and rejoinder). *J Am Stat Assoc*, 94:1096–1120 (1121–1146), 1999.

J.J. Schlesselman. *Case-Control Studies: Design, Conduct, Analysis*. Oxford, Oxford, 1982.

G. Schwartz. Estimating the dimension of a model. *Ann Stat*, 6:461–464, 1978.

M.R. Segal. Regression trees for censored data. *Biometrics*, 44:35–47, 1988.

J.S. Sekhon. Alternative balance metrics for bias reduction in matching methods for causal inference. Technical Report, University of California, Berkeley, 2006.

J.S. Sekhon. Multivariate and propensity score matching software with automated balance optimization: The matching package for R. *J Stat Softw*, 2008a.

J.S. Sekhon. The Neyman-Rubin model of causal inference and estimation via matching methods. In J.M. Box-Steffensmeier, H.E. Brady, and D. Collier, editors, *The Oxford Handbook of Political Methodology*. Oxford, New York, 2008b.

J.S. Sekhon. Opiates for the matches: Matching methods for causal inference. *Annu Rev of Polit Sci*, 12:487–508, 2010.

J.V. Selby, D.H. Smith, E.S. Johnson, M.A. Raebel, G.D. Friedman, and B.H. McFarland. Kaiser Permanente medical care program. In B.L. Strom, editor, *Pharmacoepidemiology*. Wiley, 2005.

S. Senturker, B. Karahalil, M. Inal, H. Yilmaz, H. Muslumanoglu, G. Gedikoglu, and M. Dizdaroglu. Oxidative DNA base damage and antioxidant enzyme levels in childhood acute lymphoblastic leukemia. *FEBS Letters*, 416(3):286–290, 1997.

J. Shao, X. Yu, and B. Bob Zhong. A theory for testing hypotheses under covariate-adaptive randomization. *Biometrika*, 2010.

M.J. Sillanpaa and E. Arjas. Bayesian mapping of multiple quantitative trait loci from incomplete inbred line cross data. *Genetics*, 1998.

S.E. Sinisi and M.J. van der Laan. Deletion/Substitution/Addition algorithm in learning with applications in genomics. *Stat Appl Genet Mol*, 3(1), 2004. Article 18.

H.L. Smith. Matching with multiple controls to estimate treatment effects in observational studies. *Sociol Meth*, 27:305–353, 1997.

J.M. Snowden, S. Rose, and K.M. Mortimer. Implementation of g-computation on a simulated data set: Demonstration of a causal inference technique. *Am J Epidemiol*, 173(00):000–000, 2011.

N. Soullier, J. Bouyer, Pouly J-L., J. Guibert, and de la Rochebrochard. Estimating the success of an in vitro fertilization programme using multiple imputation. *Hum Reproduct*, 23:187–192, 2008.

T.P. Speed. Introductory remarks on Neyman (1923). *Stat Sci*, 5(4):463–464, 1990.

O.M. Stitelman and M.J. van der Laan. Collaborative targeted maximum likelihood for time-to-event data. *Int J Biostat*, 6(1):Article 21, 2010.

O.M. Stitelman and M.J. van der Laan. Targeted maximum likelihood estimation of time-to-event parameters with time-dependent covariates. Technical Report, Division of Biostatistics, University of California, Berkeley, 2011a.

O.M. Stitelman and M.J. van der Laan. Targeted maximum likelihood estimation of effect modification parameters in survival analysis. *Int J Biostat*, 7(1), 2011b.

M. Stone. Cross-validatory choice and assessment of statistical predictions. *J R Stat Soc Ser B*, 36(2):111–147, 1974.

M. Stone. Asymptotics for and against cross-validation. *Biometrika*, 64(1):29–35, 1977.

J.S. Stringer, I. Zulu, J. Levy, E.M. Stringer, A. Mwango, B.H. Chi, V. Mtonga, S. Reid, R.A. Cantrell, M. Bulterys, M.S. Saag, R.G. Marlink, A. Mwinga, T.V. Ellerbrock, and M. Sinkala. Rapid scale-up of antiretroviral therapy at primary care sites in Zambia: Feasibility and early outcomes. *J Am Med Asoc*, 296(7): 782–93, 2006.

I. Tager, M. Hollenberg, and W. Satariano. Self-reported leisure-time physical activity and measures of cardiorespiratory fitness in an elderly population. *Am J Epidemiol*, 147:921–931, 1998.

Z. Tan. A distributional approach for causal inference using propensity scores. *J Am Stat Assoc*, 101:1619–1637, 2006.

Z. Tan. Comment: Improved local efficiency and double robustness. *Int J Biostat*, 4 (1):Article 10, 2008.

C.M. Tangen and G.G. Koch. On-parametric analysis of covariance for hypothesis testing with logrank and Wilcoxon scores and survival-rate estimation in a randomized clinical trial. *J Biopharm Stat*, 9(2):307–338, 1999.

G. Taubes. Do we really know what makes us healthy? *The New York Times*, 2007.

P.F. Thall, C. Logothetis, L.C. Pagliaro, S. Wen, M.A. Brown, D. Williams, and R.E. Millikan. Adaptive therapy for androgen-independent prostate cancer: A randomized selection trial of four regimens. *J Natl Cancer Inst*, 99:1613–22, 2007.

T. Therneau and T. Lumley. *survival: Survival analysis, including penalised likelihood*, 2009. URL http://CRAN.R-project.org/package=survival. R package version 2.35-8.

J.M. Thoday. Location of polygenes. *Nature*, 1960.

R. Tibshirani. Regression shrinkage and selection via the lasso. *J R Stat Soc Ser B*, 58(1):267–288, 1996.

R.J. Tibshirani. The lasso method for variable selection in the Cox model. *Stat Med*, 16:385–395, 1997.

R.M. Tombes and G.W. Krystal. Identification of novel human tumor cell-specific CaMK-II variants. *Biochim Biophys Acta*, 1355:281–292, 1997.

A.A. Tsiatis. *Semiparametric Theory and Missing Data*. Springer, Berlin Heidelberg New York, 2006.

A.A. Tsiatis, M. Davidian, M. Zhang, and X. Lu. Covariate adjustment for two-sample treatment comparisons in randomized clinical trials: A principled yet flexible approach. *Stat Med*, 27:4658–4677, 2008.

A.B. Tsybakov. Optimal rates of aggregation. In B. Schölkopf and M.K. Warmuth, editors, *COLT*, volume 2777 of *Lecture Notes in Computer Science*, Berlin Heidelberg New York, 2003. Springer.

C. Tuglus and M.J. van der Laan. Targeted methods for biomarker discovery, the search for a standard. Technical Report 233, Division of Biostatistics, University of California, Berkeley, 2008.

C. Tuglus and M.J. van der Laan. Modified FDR controlling procedure for multi-stage analyses. *Stat Appl Genet Mol*, 8(1):Article 12, 2009.

J. Tyrer, S.W. Duffy, and J. Cuzick. A breast cancer prediction model incorporating familial and personal risk factors. *Stat Med*, 23(7):1111–1130, 2004.

U.S. Preventive Services Task Force. Screening for breast cancer: U.S. Preventive Services Task Force recommendation statement. *Ann Intern Med*, 151(10):716–726, 2009.

M.J. van der Laan. Statistical inference for variable importance. *Int J Biostat*, 2(1): Article 2, 2006.

M.J. van der Laan. Estimation based on case-control designs with known prevalance probability. *Int J Biostat*, 4(1):Article 17, 2008a.

M.J. van der Laan. The construction and analysis of adaptive group sequential designs. Technical Report 232, Division of Biostatistics, University of California, Berkeley, 2008b.

M.J. van der Laan. Targeted maximum likelihood based causal inference: Part I. *Int J Biostat*, 6(2):Article 2, 2010a.

M.J. van der Laan. Targeted maximum likelihood based causal inference: Part II. *Int J Biostat*, 6(2):Article 3, 2010b.

M.J. van der Laan. Estimation of causal effects of community-based interventions. Technical Report 268, Division of Biostatistics, University of California, Berkeley, 2010c.

M.J. van der Laan and S. Dudoit. Unified cross-validation methodology for selection among estimators and a general cross-validated adaptive epsilon-net estimator: Finite sample oracle inequalities and examples. Technical Report 130, Division of Biostatistics, University of California, Berkeley, 2003.

M.J. van der Laan and S. Gruber. Collaborative double robust penalized targeted maximum likelihood estimation. *Int J Biostat*, 6(1):Article 17, 2010.

M.J. van der Laan and M.L. Petersen. Causal effect models for realistic individualized treatment and intention to treat rules. *Int J Biostat*, 3(1):Article 3, 2007a.

M.J. van der Laan and M.L. Petersen. Statistical learning of origin-specific statically optimal individualized treatment rules. *Int J Biostat*, 3(1):Article 6, 2007b.

M.J. van der Laan and M.L. Petersen. Direct effect models. *Int J Biostat*, 4(1): Article 23, 2008.

M.J. van der Laan and J.M. Robins. *Unified Methods for Censored Longitudinal Data and Causality*. Springer, Berlin Heidelberg New York, 2003.

M.J. van der Laan and S. Rose. Statistics ready for a revolution: Next generation of statisticians must build tools for massive data sets. *Amstat News*, 399:38–39, 2010.

M.J. van der Laan and Daniel B. Rubin. Targeted maximum likelihood learning. *Int J Biostat*, 2(1):Article 11, 2006.

M.J. van der Laan and Daniel B. Rubin. A note on targeted maximum likelihood and right-censored data. Technical Report 226, Division of Biostatistics, University of California, Berkeley, 2007.

M.J. van der Laan, S. Dudoit, and S. Keleş. Asymptotic optimality of likelihood-based cross-validation. *Stat Appl Genet Mol*, 3(1):Article 4, 2004.

M.J. van der Laan, M.L. Petersen, and M.M. Joffe. History-adjusted marginal structural models and statically-optimal dynamic treatment regimens. *Int J Biostat*, 1 (1):10–20, 2005.

M.J. van der Laan, S. Dudoit, and A.W. van der Vaart. The cross-validated adaptive epsilon-net estimator. *Stat Decis*, 24(3):373–395, 2006.

M.J. van der Laan, M.L. Petersen, and M.M. Joffe. Response to invited commentary: Petersen et. al. respond to "Effect modification by time-varying covariates". *Am J Epidemiol*, 166(9):1003–1004, 2007a.

M.J. van der Laan, E.C. Polley, and A.E. Hubbard. Super learner. *Stat Appl Genet Mol*, 6(1):Article 25, 2007b.

A.W. van der Vaart. *Asymptotic Statistics*. Cambridge, New York, 1998.

A.W. van der Vaart and J.A. Wellner. *Weak Convergence and Empirical Processes*. Springer, Berlin Heidelberg New York, 1996.

A.W. van der Vaart, S. Dudoit, and M.J. van der Laan. Oracle inequalities for multi-fold cross-validation. *Stat Decis*, 24(3):351–371, 2006.

H.C. van Houwelingen, T. Bruinsma, A.A.M. Hart, L.J. van't Veer, and L.F.A. Wessels. Cross-validated Cox regression on microarray gene expression data. *Stat Med*, 25:3201–3216, 2006.

J.P. Vandenbrouke, E. von Elm, D.G. Altman, P.C. Gotzsche, C.D. Mulrow, S.J. Pocock, C. Poole, J.J. Schlesselman, and M. Egger for the STROBE Initiative. Strengthening the reporting of observational studies in epidemiology (STROBE): Explanation and elaboration. *PLoS Med*, 4(10):1628–1654, 2007.

L.J. van't Veer, H. Dal, M.J. van de Vijver, Y.D. He, A.A.M. Hart, M. Mao, H.L. Peterse, K. van der Kooy, M.J. Marton, A.T. Witteveen, G.J. Schreiber, R.M. Kerkhoven, C. Roberts, P.S. Linsley, R. Bernards, and S.H. Friend. Gene expression profiling predicts clinical outcome of breast cancer. *Nature*, 415:530–536, 2002.

W.N. Venables and B.D. Ripley. *Modern Applied Statistics with S*. Springer, Berlin Heidelberg New York, 4th edition, 2002.

C. Vitale, C. Romagnani, A. Puccetti, D. Olive, R. Costello, L. Chiossone, A. Pitto, A. Bacigalupo, L. Moretta, and M.C. Mingari. Surface expression and function of p75/AIRM-1 or CD33 in acute myeloid leukemias: Engagement of CD33 induces apoptosis of leukemic cells. *Proc Natl Acad Sci*, 98:5764–5769, 2001.

S. Wacholder. The case-control study as data missing by design: Estimating risk differences. *Epidemiology*, 7(2):144–150, 1996.

H. Wang, S. Rose, and M.J. van der Laan. Finding quantitative trait loci genes with collaborative targeted maximum likelihood learning. *Stat Prob Lett*, published online 11 Nov (doi: 10.1016/j.spl.2010.11.001), 2010.

H. Wang, S. Rose, and M.J. van der Laan. Targeted methods for finding quantitative trait loci. Technical Report, Division of Biostatistics, University of California, Berkeley, 2011.

Y. Wang, M.L. Petersen, D.R. Bangsberg, and M.J. van der Laan. Diagnosing bias in the inverse probability of treatment weighted estimator resulting from violation of experimental treatment assignment. Technical Report 211, Division of Biostatistics, University of California, Berkeley, 2006.

R.W.M. Wedderburn. Quasi-likelihood functions, generalized linear models, and the gauss-newton method. *Biometrika*, 61(3):439–447, 1974.

C.W. Wester, A.M. Thomas, H. Bussmann, S. Moyo, J.M. Gaolathe, V. Novitsky, M. Essex, V. De Gruttola, and R.G. Marlink. Nonnucleoside reverse transcriptase inhibitor outcomes among combination antiretroviral therapy-treated adults in Botswana. *AIDS*, 24:S27–S36, 2010.

P.W.F. Wilson, R.B. D'Agostino, D. Levy, A.M. Belanger, H. Silbershatz, and W.B. Kannel. Prediction of coronary heart disease using risk factor categories. *Circulation*, 97:1837–1847, 1998.

C. Winship and S.L. Morgan. The estimation of causal effects from observational data. *Annu Rev Sociol*, 25:659–707, 1999.

D. H. Wolpert. Stacked generalization. *Neural Networks*, 5:241–259, 1992.

H.P. Wong, L. Yu, E.K. Lam, E.K. Tai, W.K. Wu, and C.H. Cho. Nicotine promotes cell proliferation via alpha7-nicotinic acetylcholine receptor and catecholamine-

synthesizing enzymes-mediated pathway in human colon adenocarcinoma HT-29 cells. *Toxicol Appl Pharm*, 221(3):261–267, 2007.

J. Wooldridge. Should instrumental variables be used as matching variables? Technical Report, Michigan State University, 2009.

World Health Organization. Antiretroviral therapy for HIV infection in adults and adoloescents: Recommendations for a public health approach, 2006.

L. Yang and A.A. Tsiatis. Efficiency study for a treatment effect in a pretest-posttest trial. *Am Stat*, 56:29–38, 2001.

Z. Yu and M.J. van der Laan. Construction of counterfactuals and the g-computation formula. Technical Report 122, Division of Biostatistics, University of California, Berkeley, 2002.

Z. Yu and M.J. van der Laan. Measuring treatment effects using semiparametric models. Technical Report 136, Division of Biostatistics, University of California, Berkeley, 2003.

Z.B. Zeng. Precision mapping of quantitative trait loci. *Genetics*, 1994.

H.H. Zhang and W. Lu. Adaptive lasso for Cox's proportional hazards model. *Biometrika*, 94(3):691–703, 2007.

L.-X. Zhang and F.-F. Hu. A new family of covariate-adjusted response adaptive designs and their properties. *Appl Math J Chinese Univ Ser B*, 24(1):1–13, 2009.

L.-X. Zhang, F.-F. Hu, S.H. Cheung, and W.S. Chan. Asymptotic properties of covariate-adjusted response-adaptive designs. *Ann Stat*, 35(3):1166–1182, 2007.

M. Zhang, A.A. Tsiatis, and M. Davidian. Improving efficiency of inferences in randomized clinical trials using auxiliary covariates. *Biometrics*, 64(3):707–715, 2008.

W. Zheng and M.J. van der Laan. Asymptotic theory for cross-validated targeted maximum likelihood estimation. Technical Report 273, Division of Biostatistics, University of California, Berkeley, 2010.

X. Zhu, Z. Mao, Y. Na, Y. Guo, X. Wang, and D. Xin. Significance of pituitary tumor transforming gene 1 (PTTG1) in prostate cancer. *Anticancer Res*, 26:1253–1259, 2006.

Index

Author Index