RADIOLOGICAL SOCIETY OF NORTH AMERICA

CLINICAL TRIALS METHODOLOGY WORKSHOP
January 2023

SUPPLEMENTARY BIBLIOGRAPHY

Notes: *This bibliography corresponds roughly to the topics of the didactic portion of the workshop. The list of references included here is meant to provide broad coverage of each topic. It is not a required reading list for the workshop. The references in each section are listed in reverse chronological order of publication*

A. Diagnostic Imaging Evaluation: General

1. Fryback DG, Thornbury JR. Efficacy of diagnostic imaging. Med Decis Making 1991;11:88–94.

2. Hillman, B , Gatsonis C. When is the right time to conduct a clinical trial of a diagnostic imaging technology? Radiology 2008, 248: 12-15.

3. The Evidence Base of Clinical Diagnosis: Theory and Methods of Diagnostic Research, 2nd Edition, A Knotterus and F. Buntinx (Eds), BMJ Books, 2008.

B. Introduction to Clinical Trials

1. Friedman LM, Furberg CD, DeMets DL. *Fundamentals of Clinical Trials.* 3 ed. St. Louis: Mosby, 1998.

2. Johnson, J. Williams G, Pazdur R. End Points and United States Food and Drug Administration Approval of Oncology Drugs. J Clin Oncol 2005; 21:1404-1411.

3. Stolberg HO, Norman G, Trop I. Randomized controlled trials. AJR 2004; 183: 1539-1544.

C. Introduction to Biostatistics

1. Dawson B, Trapp RG. *Basic & Clinical Biostatistics.* 4th ed. Lange Medical Books-McGraw-Hill, 2004

2. Pagano M, Gavreau K. *Principles of Biostatistics* 2nd Ed, Duxbury Press, 2000.

3. The "Statistical Concepts Series" published in Radiology in 2002-2004. (www2.rsna.org/re/CTMW2023/index.htm).

D. Statistical methods for diagnostic test evaluation: General

Textbooks, Chapters, Tutorials

1. Articles in the series: *Fundamentals of Clinical Research for Radiologists* Am J Roentgenol. 2001-2006. www2.rsna.org/re/CTMW2023/index.htm

2. Toledano AY. Cancer diagnostics: Statistical methods. In *Biostatistical Applications in Cancer Research.* Beam C (Ed.) Kluwer Norwell: Academic Publishers, 2002, 183-218.

3. Pepe M. *The Statistical Evaluation of Medical Tests for Classification and Prediction.* New York: Oxford Press, 2003.

4. Zhou X-H, Obuchowski N, McClish D. *Statistical Methods in Diagnostic Medicine.* New York: Wiley, 2011.

5. Obuchowski, N. ROC analysis. AJR 2005 ;184:364-72.

E. Statistical Methods for Diagnostic Test Evaluation: Special topics

E1. Biases and Their Control

1. Begg CB, McNeil BJ. Assessment of radiologic tests, control of bias, and other design considerations. Radiology 1988; 167:565-569.

2. Ransohoff DJ, Feinstein AR. Problems of spectrum and bias in evaluating the efficacy of diagnostic tests. NEJM 1978; 299:926-930.

3. Obuchowski N. Special Topics III: Bias. Radiology 2003; 229: 617-621.

E2. Predictive Value of Tests

1. Sargent D., Conley B., Allegra C., Collette L. Clinical Trial Designs for Predictive Marker Validation in Cancer Treatment Trials .J Clin Oncol 2005;23: 2020-2027.

2. McShane, Altman DG, Sauerbrei W, et al. Reporting recommendations for tumor marker prognostic studies (REMARK). J Natl Canc Inst 2005; 97:1180-84.

3. Fleming, T.R. Surrogate Endpoints and FDA's Accelerated Approval Process: The challenges are greater than they seem. Health Affairs 2005 ;24:67-78.

E3. Meta Analysis of Diagnostic Accuracy Studies

1. Gatsonis C, Paliwal P. Meta-analysis of diagnostic and screening test accuracy evaluations: A methodologic primer. AJR 2006; 187:271-281

2. Leeflang MM, Deeks JJ, Gatsonis C, Bossuyt PM; Cochrane Diagnostic Test Accuracy Working Group. Systematic reviews of diagnostic test accuracy. Ann Intern Med. 2008; 149(12):889-97

F. Quality of Life

1. Brazier J, Deverill M, Green C. A review of the use of health status measures in economic evaluation. J Health Serv Res Policy 1999;4:174-184.

2. Spilker B (Ed.). *Quality of Life and Pharmacoeconomics in Clinical Trials*. New York: Raven Press, 1996.

3. Calvert M, Blazeby J, Revicki D, Moher D, Brundage M. Reporting Quality of Life in Clinical Trials: A CONSORT Extension. The Lancet 2011; 378: 1684-85

4. Torrance G, Furlong W, Feeny D, Boyle M. Multi-attribute preference functions: Health Utilities Index. Pharmacoeconomics 1995;7:503-520.

G. Imaging as a Measure of Therapeutic Response

1. Eisenhauer EA, Therasse P, Bogaerts J, Schwartz LH, Sargent D, Ford R, Dancey J, Arbuck S, Gwyther S, Mooney M, Rubinstein L, Shankar L, Dodd L, Kaplan R, Lacombe D, Verweij J. New response evaluation criteria in solid tumours: revised RECIST guideline (version 1.1) Eur J Cancer 2009 Jan;45(2):228-47.

2. Wahl RL, Jacene H, Kasamon Y, Lodge MA From RECIST to PERCIST: Evolving Considerations for PET response criteria in solid tumors. J Nucl Med 2009 May;50 Suppl 1:122S-50S

H. Imaging as a Predictor of Therapeutic Response

1. Fernandez FG, Drebin JA, Linehan DC, Dehdashti F, Siegel BA, Strasberg SM. Five-year survival after resection of hepatic metastases from colorectal cancer in patients screened by positron emission tomography with F-18 flurodeoxyglucose (FDG-PET). Ann Surg 2004;240:438-447.

2. Mankoff DA, O'Sullivan F, Barlow WE, Krohn KA. Molecular imaging research in the outcomes era: measuring outcomes for individualized cancer therapy. Acad Radiol. 2007 April; 14(4): 398–405.

3. McShane LM, Cavenagh MM, Lively TG, et al. Criteria for the use of omics-based predictors in clinical trials. Nature. 2013;502(7471):317-320. doi:10.1038/nature12564.

4. Weber WA, Petersen V, Schmidt B, Tyndale-Hines L, Link T, Preschel C, Schwaiger M. Positron emission tomography in non-small-cell lung cancer: Prediction of response to chemotherapy by quantitative assessment of glucose use. J Clin Oncol 2003;2651-2657.

I. Imaging in clinical studies

1. Schnall M, Rosen M. Primer on imaging technologies for cancer. J Clin Oncol 2006;24(20):3225-33.

2. Barkhof F, Filippi M, Miller DH, Tofts P, Kappos L, Thompson AJ. Strategies for optimizing MRI techniques aimed at monitoring disease activity in multiple sclerosis treatment trials. J Neurol 1997;244(2):76-84.

J. Screening Studies: Methods

1. Hillman BJ, Black WC, Hauser B, Smith R. The appropriateness of employing imaging screening technologies: Report of the methods committee of the ACR task force on screening technologies. JACR, 2004;1(11):861-864.

2. Black WC. Overdiagnosis: An underrecognized cause of confusion and harm in cancer screening. J Natl Cancer Inst, 2000;92(16)1280-1282.

3. PM Marcus. Assessment of Cancer Screening: a Primer. November 2019. Available online: https://www.ncbi.nlm.nih.gov/books/NBK550212/

K. Modeling and Cost-Effectiveness Analysis

1. Sanders GD, Neumann PJ, Basu A, Brock DW, Feeny D, Krahn M, Kuntz KM, Meltzer DO, Owens DK, Prosser LA, Salomon JA, Sculpher MJ, Trikalinos TA, Russell LB, Siegel JE, Ganiats TG. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses. JAMA. 2016;316(10):1093-1103.

2. Ramsey S, McIntosh M, Etzioni R, Urban N. Simulation modeling of outcomes and cost effectiveness [Review]. Hematol Oncol Clin North Am, 2000;14(4):925-938.

3. Gazelle GS, McMahon PM, Beinfeld MT, Siebert U. Cost-Effectiveness Analysis in the Assessment of Diagnostic Imaging Technologies. Radiology, 2005; 235: 361-370

4. Knudsen AB, McMahon PM, Gazelle GS. Use of Modeling to Evaluate the Cost-Effectiveness of Cancer Screening Programs. Journal of Clinical Oncology, 2007; 25: 203-208

L. Reporting of Marker and Accuracy Studies

1. McShane LM, Hayes DF. Publication of Tumor Marker Research Results: The Necessity for Complete and Transparent Reporting. Journal of Clinical Oncology. 2012;30(34):4223-4232.

2. Bossuyt PM. Reitsma JB, Bruns DE, Gatsonis CA, Glasziou PP, Irwig L, Lijmer JG, Moher D, Rennie D, de Vet HCW, Kressel HY, Rifai N, Golub RM, Altman DG, Hooft L, Korevaar DA, Cohen JF, for the STARD Group. STARD 2015: An Updated List of Essential Items for Reporting Diagnostic Accuracy Studies. Radiology 2015 277:3, 826-832.

M. Medical Ethics

- The National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research (1979). The Belmont Report: Ethical Principles and Guidelines for the Protection of Human Subjects of Research. Washington, D.C.: Dept. of Health, Education, and Welfare.

N. Statistics

- Seong Ho Park, Kyunghwa Han. Methodologic Guide for Evaluating Clinical Performance and Effect of Artificial Intelligence Technology for Medical Diagnosis and Prediction. pubs.rsna.org/doi/10.1148/radiol.2017171920

O. Resources Available on the Web

- Selected FDA GCP/Clinical Trial Guidance Documents