

Key Multiplicity Issues in Clinical Trials (Part II)

Online training course by Alex Dmitrienko available at www.sprmm.com

Papers

Alosh, M., Bretz, F., Huque, M. (2014). Advanced multiplicity adjustment methods in clinical trials. *Statistics in Medicine*. 33, 693-713.

Barnes, P.J. et al (2010). Integrating indacaterol dose selection in a clinical study in COPD using an adaptive seamless design. *Pulmonary Pharmacology and Therapeutics* 23, 165-171.

Bauer, P., Kohne, F. (1994). Evaluation of experiments with adaptive interim analyses. *Biometrics*. 50, 1029-1041.

Benda, N., Branson, M., Maurer, W., Friede, T. (2010). Aspects of modernizing drug development using clinical scenario planning and evaluation. *Drug Information Journal*. 44, 299-315.

Brechenmacher, T., Xu, J., Dmitrienko, A., Tamhane, A.C. (2011). A mixture gatekeeping procedure based on the Hommel test for clinical trial applications. *Journal of Biopharmaceutical Statistics*. 21, 748-767.

Brechenmacher, T., Dmitrienko, A. (2017). Multiplicity adjustment methods. *Analysis of Clinical Trials Using SAS (Second Edition)*. Dmitrienko, A., Koch, G. (editors). SAS Press, Cary, NC.

Bretz, F., Maurer, W., Brannath, W., Posch, M. (2009). A graphical approach to sequentially rejective multiple test procedures. *Statistics in Medicine*. 28, 586-604.

Burman, C.F., Sonesson, C., Guilbaud, O. (2009). A recycling framework for the construction of Bonferroni-based multiple tests. *Statistics in Medicine*. 28, 739-761.

Cummings S.R. et al. (2009). Denosumab for prevention of fractures in postmenopausal women with osteoporosis. *New England Journal of Medicine*. 361, 756-765.

Dmitrienko, A., Tamhane, A., Wiens, B. (2008). General multistage gatekeeping procedures. *Biometrical Journal*. 50, 667-677.

Dmitrienko, A., Tamhane, A.C. (2011). Mixtures of multiple testing procedures for gatekeeping applications in clinical trials. *Statistics in Medicine*. 30, 1473-1488.

Dmitrienko, A., Millen, B.A., Brechenmacher, T., Paux, G. (2011). Development of gatekeeping strategies in confirmatory clinical trials. *Biometrical Journal*. 53, 875-893.

Dmitrienko, A., D'Agostino, R.B., Huque, M.F. (2013). Key multiplicity issues in clinical drug development. *Statistics in Medicine*. 32, 1079-1111.

Dmitrienko, A., Tamhane, A.C. (2013). General theory of mixture procedures for gatekeeping. *Biometrical Journal*. 55, 402-419.

Dmitrienko, A., D'Agostino, R.B. (2013). Tutorial in Biostatistics: Traditional Multiplicity Adjustment Methods in Clinical Trials. *Statistics in Medicine*. 32, 5172-5218.

Dmitrienko, A., Paux, G., Brechenmacher, T. (2015). Power calculations in clinical trials with complex clinical objectives. *Journal of the Japanese Society of Computational Statistics*. 28, 15-50.

Dmitrienko, A., Kordzakhia, G., Brechenmacher, T. (2016). Mixture-based gatekeeping procedures for multiplicity problems with multiple sequences of hypotheses. *Journal of Biopharmaceutical Statistics*. 26, 758-780.

Dmitrienko, A., Paux, G., Pulkstenis, E., Zhang, J. (2016). Tradeoff-based optimization criteria in clinical trials with multiple objectives and adaptive designs. *Journal of Biopharmaceutical Statistics*. 26, 120-140.

Dmitrienko, A., Offen, W., Westfall, P.H. (2003). Gatekeeping strategies for clinical trials that do not require all primary effects to be significant. *Statistics in Medicine*. 22, 2387-2400.

Dmitrienko, A., Tamhane, A.C. (2009). Gatekeeping procedures in clinical trials. *Multiple Testing Problems in Pharmaceutical Statistics*. Dmitrienko, A., Tamhane, A.C., Bretz, F. (editors). Chapman and Hall/CRC Press, New York.

Downing, A.M. et al. (2014). A double-blind, placebo-controlled comparator study of LY2140023 monohydrate in patients with schizophrenia. *BMC Psychiatry*. 14, 351.

Frederich, R. et al. (2012). The efficacy and safety of the dipeptidyl peptidase-4 inhibitor saxagliptin in treatment-naïve patients with Type 2 diabetes mellitus: A randomized controlled trial. *Diabetology and Metabolic Syndrome*. 4:36.

Friede, T., Nicholas, R., Stallard, N., Todd, S., Parsons, N. R., Valdes-Marquez, E., Chataway, J. (2010). Refinement of the clinical scenario evaluation framework for assessment of competing development strategies with an application to multiple sclerosis. *Drug Information Journal*. 44, 713-718.

Glimm E, Maurer W, Bretz F. (2009). Hierarchical testing of multiple endpoints in group-sequential trials. *Statistics in Medicine*. 29, 219-228.

Herring, W.J. et al. (2016). Suvorexant in patients with insomnia: Results from two 3-month randomized controlled clinical trials. *Biological Psychiatry*. 79, 136-148.

Hung, H.M.J., Wang, S.J., O'Neill, R. (2007). Statistical considerations for testing multiple endpoints in group sequential or adaptive clinical trials. *Journal of Biopharmaceutical Statistics*. 17, 1201-1210.

Hung, H.M.J., Wang, S.J. (2009). Some controversial multiple testing problems in regulatory

- applications. *Journal of Biopharmaceutical Statistics*. 19, 1-11.
- Huque, M.F., Dmitrienko, A., D'Agostino, R.B. (2013). Multiplicity issues in clinical trials with multiple objectives. *Statistics in Biopharmaceutical Research*. 5, 321-337.
- Huque, M.F. (2016). Validity of the Hochberg procedure revisited for clinical trial applications. *Statistics in Medicine*. 35, 5-20.
- Kordzakhia, G., Brechenmacher, T., Ishida, E., Dmitrienko, A., Zheng, W., Li, D. (2018). An enhanced mixture method for constructing gatekeeping procedures in clinical trials. *Journal of Biopharmaceutical Statistics*.
- Kordzakhia, G., Dmitrienko, A., Ishida, E. (2018). Mixture-based gatekeeping procedures in adaptive clinical trials. *Journal of Biopharmaceutical Statistics*.
- Magirr, D., Jaki, T., Koenig, F., Posch, M. (2016). Sample size reassessment and hypothesis testing in adaptive survival trials. *PLoS ONE*. 11, e0146465.
- Marcus, R., Peritz, E., Gabriel, K.R. (1976). On closed testing procedures with special reference to ordered analysis of variance. *Biometrika*. 63, 655-660.
- Maurer, W., Bretz, F. (2013). Memory and other properties of multiple test procedures generated by entangled graphs. *Statistics in Medicine*. 32, 1739-1753.
- Maurer, W., Bretz, F. (2013). Multiple testing in group sequential trials using graphical approaches. *Statistics in Biopharmaceutical Research*. 5, 311-320.
- Meltzer, H.Y. et al. (2011). Lurasidone in the treatment of schizophrenia: a randomized, double-blind, placebo- and olanzapine-controlled study. *American Journal of Psychiatry*. 168, 957-967.
- Nasrallah, H.A. et al. (2013). Lurasidone for the treatment of acutely psychotic patients with schizophrenia: A 6-week, randomized, placebo-controlled study. *Journal of Psychiatric Research*. 47, 670-677.
- Rosenstock, J., Aguilar-Salinas, C., Klein, E., Nepal, S., List, J., Chen, R. (2009). Effect of saxagliptin monotherapy in treatment-naïve patients with type 2 diabetes. *Current Medical Research And Opinion*. 25, 2401-2411.
- Sarkar, S., Chang, C.K. (1997). Simes' method for multiple hypothesis testing with positively dependent test statistics. *Journal of the American Statistical Association*. 92, 1601-1608.
- Sarkar, S.K. (1998). Some probability inequalities for censored MTP2 random variables: A proof of the Simes conjecture. *The Annals of Statistics*. 26, 494-504.
- Sarkar, S.K. (2008). On the Simes inequality and its generalization. *Beyond Parametrics in Interdisciplinary Research: Festschrift in Honor of Professor Pranab K. Sen*. Balakrishnan, N., Pena, E.A., Silvapulle, M.J. (editors). Institute of Mathematical Statistics, Beachwood, Ohio, 231-242.

Sartor, O. et al. (2014). Effect of radium-223 dichloride on symptomatic skeletal events in patients with castration-resistant prostate cancer and bone metastases: results from a Phase 3, double-blind, randomised trial. *The Lancet*. 15, 738-746.

Simes, R.J. (1986). An improved Bonferroni procedure for multiple tests of significance. *Biometrika*. 63, 655-660.

Sternberg, C.N. et al. (2009). Multinational, double-blind, phase iii study of prednisone and either satraplatin or placebo in patients with castrate-refractory prostate cancer progressing after prior chemotherapy: The SPARC trial. *Journal Of Clinical Oncology*. 27, 5431-5438.

Sugitani, T., Bretz, F., Maurer, W. (2016). A simple and flexible graphical approach for adaptive group-sequential clinical trials. *Journal of Biopharmaceutical Statistics*. 26, 202-216.

Turk, D.C. (2008). Analyzing multiple endpoints in clinical trials of pain treatments: IMMPACT recommendations. *Initiative on Methods, Measurement, and Pain Assessment in Clinical Trials*. Pain. 139, 485-493.

Wainwright, C.E. et al. (2015). Lumacaftor-ivacaftor in patients with cystic fibrosis homozygous for Phe508del CFTR. *New England Journal of Medicine*. 373, 220-231.

Wang, S., O'Neill, R., Hung, H. (2007). Approaches to evaluation of treatment effect in randomized clinical trials with genomic subset. *Pharmaceutical Statistics*. 6, 227-244.

Wang, S.J., Bretz, F., Dmitrienko, A., Hsu, J., Hung, J., Huque, M., Koch, G. (2013). Panel forum on multiple comparison procedures: a commentary from a complex trial design and analysis plan. *Biometrical Journal*. 55, 275-293.

Westfall, P.H., Krishen, A. (2001). Optimally weighted, fixed sequence, and gatekeeping multiple testing procedures. *Journal of Statistical Planning and Inference* 99, 25-40.

Books

Dmitrienko, A., Tamhane, A.C., Bretz, F. (editors). (2009). *Multiple Testing Problems in Pharmaceutical Statistics*. Chapman and Hall/CRC Press, New York.

Dmitrienko, A., Pulkstenis, E. (editors). (2017). *Clinical Trial Optimization Using R*. Chapman and Hall/CRC Press, New York.

Dmitrienko, A., Koch, G. (editors). (2017). *Analysis of Clinical Trials Using SAS (Second Edition)*. SAS Press, Cary, NC.