

# Reading List for Adaptive Intervention & SMART Experimental Designs

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## Books

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- Kosorok, M. R., & Moodie, E. E. (2015). Adaptive treatment strategies in practice: Planning trials and analyzing data for personalized medicine. Philadelphia, PA: SIAM.
- Chakraborty, B., & Moodie, E. E. (2013). Statistical methods for dynamic treatment regimes (pp. 31-52). Springer.
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## Introduction to Adaptive Intervention Designs (aka Adaptive Treatment Strategies)

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- Wallace, M. P. and Moodie, E. E. M. (2014), Personalizing medicine: A review of adaptive treatment strategies. *Pharmacoepidemiol Drug Saf*, 23: 580–585.
- Murphy, S. A. & Almirall, D. (2009). Dynamic treatment regimes. In M. W. Kattan (Ed.), *Encyclopedia of medical decision making* (pp. 419-422). Thousand Oaks, CA: Sage.
- Murphy, S. A., Collins, L. M., & Rush, A. J. (2007). Customizing treatment to the patient: Adaptive treatment strategies (Editorial). *Drug and Alcohol Dependence*, 88(2), S1-S72.
- \* Collins, L. M., Murphy, S. A., & Bierman, K. A. (2004). A conceptual framework for adaptive preventive interventions. *Prevention Science*, 5, 185-196.
- \* Murphy, S. A. & McKay, J. R. (2004). Adaptive treatment strategies: An emerging approach for improving treatment effectiveness. *Clinical Science* (Newsletter of the American Psychological Association Division 12, Section III: The Society for the Science of Clinical Psychology). Winter 2003/Spring 2004.
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## Introduction to SMART Experimental Designs

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- \* Wallace, M.P., Moodie, E.E.M. & Stephens, D.A. (2016). SMART Thinking: A Review of Recent Developments in Sequential Multiple Assignment Randomized Trials. *Curr Epidemiol Rep*, 3:225.
- Kidwell, K.M., Hyde W. (2016) Adaptive Intervention and SMART Designs: Application to Child Behavior Research in a Community Setting. *American Journal of Evaluation*, 7 (3).
- \* Kidwell, K.M. (2014). SMART designs in cancer research: Past, present, and future. *Clinical Trials*, 11(4), 445-456.
- \* Almirall, D., Nahum-Shani, I., Sherwood, N. E., & Murphy, S. A. (2014), Introduction to SMART designs for the development of adaptive interventions: With application to weight loss research. *Translational Behavioral Medicine*, 4(3), 260-274.
- \* Lei, H., Nahum-Shani, I., Lynch, K., Oslin, D., & Murphy, S. A. (2012). A "SMART" design for building individualized treatment sequences. *Annual Review of Clinical Psychology*, 8, 14.1 - 14.28.
- Nahum-Shani, I., Qian, M., Almirall, D., Pelham, W., Gnagy, B., Fabiano, G., ... Murphy, S. A. (2012). Experimental design and primary data analysis methods for comparing adaptive interventions. *Psychological Methods*, 17, 457-77.
- Murphy, S. A., Lynch, K. G., McKay, J. R., Oslin, D., & TenHave, T. (2007). Developing adaptive treatment strategies in substance abuse research. *Drug and Alcohol Dependence*, 88(2), S24-S30.
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## SMART Design Considerations, including Sample Size/Power Considerations

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- Almirall D., Nahum-Shani, I., Wang, L., Kasari, C. (submitted). Experimental Designs for Research on Adaptive Interventions: Singly- and Sequentially-Randomized Trials. **[Draft Available Upon Request]**
- \* Oetting, A. I., Levy, J. A., Weiss, R. D. & Murphy, S. A., (2011). Statistical methodology for a SMART design in the development of adaptive treatment strategies. In P.E. Shrout (Ed.), *Causality and psychopathology: Finding the determinants of disorders and their cures* (pp.179-205). Arlington, VA: American Psychiatric Publishing.
- Murphy, S. A., (2005). An experimental design for the development of adaptive treatment strategies. *Statistics in Medicine*, 24(10), 1455–1481.
- Collins, L. M., Nahum-Shani, I., & Almirall, D. (2014). Optimization of behavioral dynamic treatment regimens based on the sequential, multiple assignment, randomized trial (SMART). *Clinical Trials*, 11, 426-434.
- Almirall, D., Lizotte, D., & Murphy, S. (2012). Comment: SMART design issues and the consideration of opposing outcomes: Discussion of “Evaluation of viable dynamic treatment regimes in a sequentially randomized trial of advanced prostate cancer” by Wang, Rotnitzky, Lin, Millikan, and Thall. *Journal of the American Statistical Association*, 107, 509-512.
- Almirall, D., Compton, S. N., Gunlicks-Stoessel, M., Duan, N., & Murphy, S. A. (2012). Designing a pilot sequential multiple assignment randomized trial for developing an adaptive treatment strategy. *Statistics in Medicine*, 31(17), 1887-1902.

Li, Z., & Murphy, S. A. (2011). Sample size formulae for two-stage randomized trials with survival outcomes. *Biometrika*, 98, 503–518.

Murphy, S. A., & Bingham, D. (2009). Screening experiments for developing dynamic treatment regimes. *Journal of the American Statistical Association*, 104(458), 391-408.

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### Primary Aim Analyses with SMART Data

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NeCamp, T., Kilbourne, A., Almirall, D. (accepted 2017). Cluster-level adaptive interventions and sequential, multiple assignment, randomized trials: Estimation and sample size considerations. *Statistical Methods in Medical Research* [Draft Available Upon Request]

Seewald, N. J., Nahum-Shani, I., McKay, J. R., & Almirall, D. (in progress) Sample Size Considerations for the Analysis of a Continuous Repeated-Measures Outcome in a SMART. [Draft Available Upon Request]

Lu, X., Nahum-Shani, I., Kasari, C., Lynch, K. G., Oslin, D. W., Pelham, W. E., ... & Almirall, D. (2015). Comparing dynamic treatment regimes using repeated-measures outcomes: modeling considerations in SMART studies. *Statistics in Medicine*.

\* Nahum-Shani, I., Qian, M., Almirall, D., Pelham, W., Gnagy, B., Fabiano, G., ... Murphy, S. A. (2012). Experimental design and primary data analysis methods for comparing adaptive interventions. *Psychological Methods*, 17, 457-77.

Ertefaie, A., Wu, T., Lynch, K. G., & Nahum-Shani, I. (2015). Identifying a set that contains the best dynamic treatment regimes. *Biostatistics*, 17, 135-148.

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### Secondary Aim Analyses with SMART Data, or Other Advanced Topics

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Zhang, Y., Laber, E. B., Tsiatis, A., & Davidian, M. (2016). Interpretable Dynamic Treatment Regimes. *arXiv preprint arXiv:1606.01472*.

Lu, X., Lynch, K. G., Oslin, D. W., & Murphy, S. A. (2015). Comparing treatment policies with assistance from the structural nested mean model. *Biometrics* (online).

Laber, E. B., Lizotte, Ferguson, B. (2014). Set-valued Dynamic treatment regimes for Competing Outcomes. *Biometrics*. 70,53-61.

Chakraborty, B., & Murphy, S. A., (2014). Dynamic treatment regimes. *Annual Review of Statistics and its Application*, 1, 447-464.

Shortreed, S. M., Laber, E., Stroup, T. S., Pineau, J., & Murphy, S. A. (2014). A multiple imputation strategy for sequential multiple assignment randomized trials. *Statistics in Medicine*, 33(24), 4202-14.

Laber, E. B., Lizotte, D. J., Qian, M., Pelham, W. E., & Murphy, S. A. (2014). Dynamic treatment regimes: Technical challenges and applications. *Electronic Journal of Statistics*. 8(1),1225-1272.

\* Nahum-Shani, I., Qian, M., Almirall, D., Pelham, W., Gnagy, B., Fabiano, G., ... Murphy, S. A. (2012). Q-learning: A data analysis method for constructing adaptive interventions. *Psychological Methods*, 17, 478-494.

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### Substantive Publications of SMARTs or Other Research on Adaptive Interventions

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\* Gunlicks-Stoessel, M., Mufson, L., Westervelt, A., Almirall, D., & Murphy, S. (2015). A Pilot SMART for developing an adaptive treatment strategy for adolescent depression. *Journal of Clinical Child & Adolescent Psychology*. Advance online publication. doi:10.1080/15374416.2015.1015133, 1-15.

\* Kasari, C., Kaiser, A., Goods, K., Nietfeld, J., Mathy, P., Landa, R., Murphy, S. A., & Almirall, D. (2014). Communication interventions for minimally verbal children with autism: A sequential multiple assignment randomized trial. *Journal of the American Academy of Child and Adolescent Psychiatry*. 53(6), 635-46.

\* Pelham Jr, W. E., Fabiano, G. A., Waxmonsky, J. G., Greiner, A. R., Gnagy, E. M., Pelham III, W. E., ... & Karch, K. (2016). Treatment sequencing for childhood ADHD: A multiple-randomization study of adaptive medication and behavioral interventions. *Journal of Clinical Child & Adolescent Psychology*, 45(4), 396-415.

Almirall, D., DiStefano, C., Chang, Y. C., Shire, S., Kaiser, A., Lu, X., ... & Kasari, C. (2016). Longitudinal effects of adaptive interventions with a speech-generating device in minimally verbal children with ASD. *Journal of Clinical Child & Adolescent Psychology*, 45(4), 442-456.

Almirall, D., & Chronis-Tuscano, A. (2016). Adaptive interventions in child and adolescent mental health. *Journal of Clinical Child & Adolescent Psychology*, 45(4), 383-395.

Kilbourne, A. M., Almirall, D., Eisenberg, D., Waxmonsky, J., Goodrich, D. E., Fortney, J. C., ... Thomas, M. R. (2014). Protocol: Adaptive implementation of effective programs trial (ADEPT): Cluster randomized SMART trial comparing a standard versus enhanced implementation strategy to improve outcomes of a mood disorders program. *Implementation Science*, 9, 132.

- Kilbourne, A. M., Almirall, D., Goodrich, D. E., Lai, Z., Abraham, K. M., Nord, K. M., & Bowersox, N. (2014). Enhancing outreach for persons with serious mental illness: 12-month results from a cluster randomized trial of an adaptive implementation strategy. *Implementation Science*, 9(1) 163.
- \* Wang, L., Rotnitzky, A., Lin, X., Millikan, R. E., & Thall, P. F. (2012). Evaluation of viable dynamic treatment regimes in a sequentially randomized trial of advanced prostate cancer. *Journal of the American Statistical Association*, 107, 493–508.
- \* Marlowe, D. B., Festinger, D. S., Arabia, P. L., Dugosh, K. L., Benasutti, K. M., Croft, J. R., & McKay, J. R. (2008). Adaptive interventions in drug court: A pilot experiment. *Criminal Justice Review*, 33, 343–360.
- Thall PF, Logothetis C, Pagliaro LC, Wen SJ, Brown MA, et al. (2007). Adaptive therapy for androgen-independent prostate cancer: a randomized selection trial of four regimens. *J. Natl. Cancer Inst.* 99: 1613–22