Questions and Answers (Q&As)

Mind the Gap — Power Analyses to Plan Idiographic Clinical Trials, Illustrated for Prevention and Rare Diseases

Ty Ridenour, Ph.D., M.A. and Stephen Tueller, Ph.D., M.S.

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Q: Can PersonAlytics be used with wearable/biometric data?

A: Yes, and we are currently adapting PersonAlytics specifically for use with wearable/biometric data. Our current focus is on specifying data formatting and appropriate levels of aggregation that balance reasonable model fitting run times against desired levels of granularity. We also are exploring automated translation of data that are output from wearables into spreadsheets for statistical analysis.

Q: Have these methods been used in ecological momentary assessment studies?

A: Yes, and we have upcoming studies that plan to use PersonAlytics with EMA, including one funded by the NIH/NIMH. Below are two references that could be cited as examples.

- *Matson P.A., Ridenour T.A., Chung S.E., Adhia A., Grieb S.D., Poole E., Huettner S., Rothman E.F., Bair-Merritt M.H. Adolescent and Young Women's Daily Reports of Emotional Context and Episodes of Dating Violence. Journal of Family Violence. 2020; 1-9. https://doi.org/10.1007/s10896-020-00151-7*
- *Murray, D.W., Ridenour, T.A., Swingler, M.M., Morgan, A., Hegarty-Craver, M. (In press). Feasibility of Combining Biosensor and Ecological Momentary Assessment to Measure Stress Experiences among Economically Disadvantaged Adolescents. Stress and Health. https://doi.org/10.1002/smi.3211*

Q: How comparable are efficacy estimates from traditional randomized controlled trials to outcomes estimates from idiographic clinical trials?

A: Computation of efficacy estimates for RCTs that use hierarchical modeling are directly comparable to hierarchically modelled estimates from ICTs. However, estimates from RCTs are typically based on fewer than five waves of observations that are spread over months or years whereas estimates from ICTs are typically modelled from dozens of observations that are collected over weeks or perhaps a few months. The measures used in each type of design also are typically different even when measuring the same theoretical construct.

Q: What design factors from your power analysis simulation study were found to improve power to detect intervention effects?

A: The design factors that improved power and are nominally under researcher control included

- *More time points overall;*
- *More participants; and*
- *More baseline/preintervention timepoints and more specifically a more equal balance in the number of preintervention and postintervention time points*

The design factors that improved power and are not nominally under researcher control included

- *Lower error variance;*
- *Higher effect size; and*
- *A phase jump effect size had greater power to detect than a comparable slope effect size*

Q: How do idiographic clinical trial methods differ from or overlap with just-in-time adaptive interventions?

A: These are two entirely different methods with JITAIs being an iteratively personalized intervention approach whereas an ICT utilizes a research design and analytic approach to test the outcomes/impact of an intervention.

Q: What are the other R packages utilized by PersonAlytics?

A: The R packages that do the model fitting are nlme, gamlls, and stats (for ARIMA models). Other packages that PersonAlytics and PersonAlyticsPower require are listed in the Appendix.

Q: Do acceptability and feasibility studies have different methods of power analysis?

A: Acceptability and feasibility studies usually only consist of one to a few waves of data collection with longer time spans between them than is common for an ICT. So, for those studies, more traditional power analysis is recommended. If, on the other hand, their study design and frequency of data collection more closely resembles and ICT, the power analysis methods that were demonstrated during the webinar could be used.

Q: How do the approaches and software tools you shared with us today compare with the micro-randomized trials approach of Susan Murphy and the newer dynamic SEM (DSEM) approaches implemented in the Mplus latent variable modeling program?

A: While there are some conceptual and methodological parallels between the hierarchical model approaches that were presented for ICTs and DSEM, we've found the two approaches better address different types of research questions. For example, the ICT methods that were discussed during the webinar are better attuned for testing outcomes of interventions while DSEM is more adept for investigating how characteristics/factors impact each other within persons over time. Also see the response to #5 regarding JITAIs.

Q: In the slide that shows the equation for intensive HLM, can you walk through again the difference between time and phase? Also, can you describe the interaction term? Finally, as for the USEM, can we have more than one pair of variables for both IVs and DVs?

A: Please see the first paper listed below for detailed delineation of time, time points, phases, and use of related interaction terms. For USEM/DSEM, it is possible to use more than 2 variables in the analyses. The second paper listed below provides one example of a 3-variate model.

- *Tueller, S., Ramirez, D., Cance, J.D., Ye, A., Wheeler, A.C., Fan, Z., Hornik, C., Ridenour, T.A. (2022). Power Analysis for Idiographic (Within-Subject) Clinical Trials: Implications for Treatments of Rare Conditions and Precision Medicine. Behavior Research Methods. https://doi.org/10.3758/s13428-022-02012-1*
- *Ridenour TA, Chen SHK, Liu HY, Hill K, Bobashev, G., Cooper R. The clinical trials mosaic: Toward a range of clinical trials designs to optimize evidence-based treatment. Journal of Person Oriented Research 2017; 3: 28-48. doi: 10.17505/jpor.2017.03*

Q: What is the FDA's view of ICTs and acceptance of its use for interventional trials in rare diseases?

A: Of course, we cannot speak on behalf of FDA. That said, we know of at least one FDA-approved study to test a novel treatment for a rare disease in childhood that uses a non-inferiority design and the hierarchical modeling ICT approach.