State-of-the-Art Small Sample Analytics

Available for Exclusive Licensing

Medical Applications

Data analysis for testing treatments:

- for rare diseases and other small populations
- early phases of treatment development
- when patients cannot be randomized
- when data are to be collected during normal clinical services
- when data must be collected 'in the field'
- mechanisms of outcomes heterogeneity as treatment is administered
- when budgets are limited
- for newly evolving / spreading disease

Non-Medical Applications

Data analysis for testing treatments:

- for studies of small samples
- When test subjects cannot be randomized at reasonable cost
- When data are to be collected in situ
- When analyzing pilot test data
- Program evaluations with fewer than 30 sites

Randomized clinical trials (RCT) are well-established and robust methods for evaluating efficacy of treatments at the population level. However, applying RCT methods requires large sample sizes and is often expensive. These restrictions limit the settings and research questions that RCT methods can address. Two types of studies specifically out of reach of RCT methods are treatment effects on small populations and intensive within-patient effects. Given the opportunities represented by delivering therapies for patients with rare diseases and the insights to be gained from matching within-patient effects with genomic and other personal data, a complementary set of tools is clearly needed.

Researchers at RTI International, a world-leading non-profit research institute, have developed software tools to support rigorous small sample experiments, early stage clinical trials, and quantified monitoring of patients over time. These robust statistical techniques are being coupled to within-person experimental designs to form methodologies, called idiographic clinical trials (ICTs), that are complementary to RCTs.

To make ICT methods more broadly available and easier to employ, RTI has also developed *Patient-Centered Clinical Trials*© (PaCCT©) software. PaCCT simplifies and automates analyses for clinical researchers and clinicians working with small samples, including N=1, thus addressing critical unmet needs, opening access to untapped markets and enabling value-based healthcare. ICT methods running on PaCCT have been used in [medical arenas] neurology, organ transplantation, intensive care pharmacology, cardiac arrest rehabilitation, [non-medical arenas] speech therapy, psychotherapy, health-related behavior change, occupational stress, and biosensor outcomes.

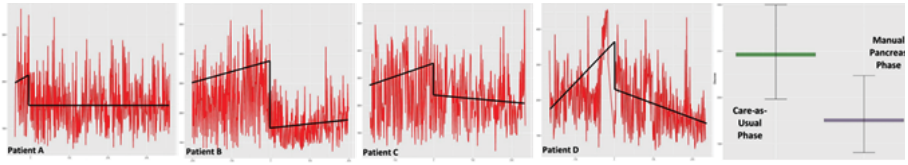
The Technology

PaCCT is a menu-driven statistical program to simplify analysis for researchers to conduct rigorous small sample size trials or researchers wanting evidence-based, individualized treatment specifically for his/her patient. Time series data (many observations over short time periods such as weekly, daily, or shorter intervals such as with biosensors) provide rich study information that cannot be acquired using traditional RCT approaches. Yet, rigorous analysis of time series data requires innovative statistical methods such as multilevel modeling or state-space modeling. PaCCT guides users through the analytic process using non-statistical terms, to avail ICT methods to researchers who are unfamiliar with its statistical approaches for small or N = 1 samples.

Benefits

- Supports very small sample size (1 to 100) trials
- Each participant receives the experimental treatment and can be provided with individualized outcomes
- Efficacy report giving participants strong incentive to complete trials
- Analytics to investigate mechanisms of outcomes as treatment is administered
- Studies can be conducted during usual clinical services
- Analysis provides detailed quantification of heterogeneity in outcomes
- Applicable across most types of treatment studies [e.g., medical, social science]
- User interface is intuitive and customizable to each user's needs and data patterns
- Many of the more complex statistical decisions are automated and data-driven

Some of the clinical studies that have employed these methods include an evaluation of converting liver- or kidney-transplant patients from branded to generic tacrolimus (Momper et al., 2011); comparison among sedation mediations used during intensive care (Benedict et al., 2014; Ridenour et al., 2016); and pilot testing an individually-tailored "manual pancreas" to manage blood glucose levels in nursing home patients with diabetes (Ridenour et al. 2013).



Example of recent individualized and aggregate clinical trial results for glucose control

Technical Team

Dr. Ty Ridenour leads these efforts; he is a senior research scientist with more than 20 years of clinical and research experience on etiology, assessment, and methodology related to disruptive behavior disorders. Before joining RTI, he had academic appointments at the University of Pittsburgh School of Pharmacy, The Pennsylvania State University, and Washington University in St. Louis. Dr. Stephen Tueller is a quantitative psychologist with broad expertise in RCTs, longitudinal dynamic and intensive data analysis, simulation studies, and analytic software programming. Dr. Corina Owens' expertise includes small sample analytics, small sample data simulation, and psychometrics. Christopher Siege is Program Director of RTI's Data Integration, Reporting and Analytics in the Research Computing Division. Dr. Georgiy Bobashev is a Biomathematician and Senior Research Statistician at RTI with long-term interests in predictive modeling, agent-based modeling and systems dynamics.

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More Information

To discuss licensing or to speak with the inventors of this technology, please contact **Kevin Boggs** at **919.248.4140** or **licensing@rti.org**.

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