# State-of-the-Art Small-Sample Analytics

## Now Available

### Medical Applications

**Data analysis for testing treatments for**

- Studies of rare diseases and other small populations
- Treatment development in the early stages
- Situations in which patients cannot be randomized
- Data that are to be collected during normal clinical services or must be collected in the field
- Mechanisms of outcomes heterogeneity as treatment is administered
- Budgets that are limited
- Diseases that are newly evolving and/or spreading

### Nonmedical Applications

**Data analysis for testing treatments for**

- Studies of small samples
- Situations in which test subjects cannot be randomized at reasonable cost
- Collection of data in situ
- Analysis of 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 have developed software tools to support rigorous small-sample experiments, early stage clinical trials, and quantified monitoring of patients over time. These robust statistical tools and techniques are being coupled with within-person experimental designs to form methodologies, called idiographic clinical trials (ICTs), that can be used in many scenarios when RCTs are not feasible.

To make ICT methods more broadly available and easier to employ, RTI has developed patient-centered clinical trial software called PersonAlytics™. PersonAlytics 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 have recently been used in medical arenas such as neurology, organ transplantation, intensive care pharmacology, and cardiac arrest rehabilitation; nonmedical arenas include speech therapy, psychotherapy, health-related behavior change, occupational stress, and biosensor outcomes.

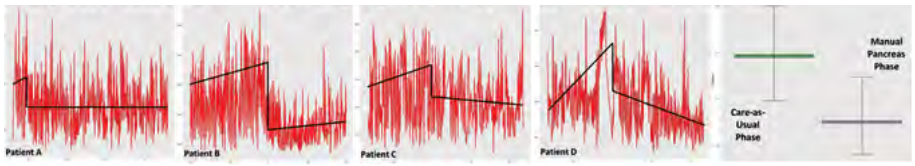
### The Technology

PersonAlytics is a menu-driven statistical program that simplifies analysis for researchers conducting rigorous small sample–size trials or researchers wanting evidence-based, individualized treatment specifically for their patients. Time series data (i.e., many observations over short time periods such as weekly, daily, or shorter intervals 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. PersonAlytics guides users through the analytic process using nonstatistical terms, making ICT methods available 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)
- Gives each participant the experimental treatment and can be provided with individualized outcomes
- Provides efficacy report, giving participants strong incentive to complete trials
- Offers short timelines
- Provides analytics to investigate mechanisms of outcomes as treatment is administered
- Allows for studies to be conducted during usual clinical services
- Uses analysis to provide detailed quantification of heterogeneity in outcomes
- Applies to most types of treatment studies (e.g., medical, social science)
- Provides intuitive, customizable user interface for each user's needs and data patterns
- Allows for many of the more complex statistical decisions to be automated and data-driven

Clinical studies that have employed these methods include an evaluation of converting liver- or kidney-transplant patients from branded to generic tacrolimus; comparison among sedation medications used during intensive care; and pilot testing an individually tailored "manual pancreas" to manage blood glucose levels in diabetic nursing home patients.




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, 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.

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## More Information

To discuss licensing or to speak with the inventors of this patented and patent-pending invention, please contact **Ginger Rothrock** at **919.541.6025** or **licensing@rti.org**.



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