ASA 18TH ANNUAL CT CHAPTER MINI-CONFERENCE

Yale School of Public Health
Winslow Auditorium
60 College Street, New Haven, CT
Wednesday, April 22, 2020

REGISTRATION

ASA CT members: **$75**
Non-members: **$100**
Students: **$25**
Groups of 5+: **$75/person**
(contact ctchapterasa@gmail.com for group rate registration instructions)

Register here:
https://www.123signup.com/register?id=rnvns

Registration closes at **noon** on **Friday, April 17th**

LOCATION

Yale School of Public Health
Winslow Auditorium (Lower Level)
60 College Street
New Haven, CT 06510

Directions to YSPH:
https://publichealth.yale.edu/about/map/

Parking: Air Rights Garage
https://parknewhaven.com/parking/air-rights-garage/

SCHEDULE

8:30-9 am **Registration (Lower level gathering space) & breakfast (Room 108)**

9-10  **Naitee Ting, PhD,** Director, Biostatistics and Data Science, Boehringer-Ingelheim. "*Subgroup analysis in clinical development of new drugs*"

10-10:15 **Break (Room 108)**

10:15-11:45  **John Preisser, PhD,** Professor, Department of Biostatistics, University of North Carolina at Chapel Hill. "*Design and analysis of stepped wedge and other cluster randomized trials based upon generalized estimating equations*"

11:45-1 pm **Lunch (Room 105)**

1-2  **Domenic Reda, PhD,** Director, Hines VA Cooperative Studies Program Coordinating Center; Research Assoc. Professor, U of Illinois at Chicago and Northwestern U. "*Θ1≠ Θ2, p≤.05. SO WHAT! Rethinking clinical trial design*"

2-3  **Max Kuhn, PhD,** Software Engineer, RStudio, PBC. "*What inferential statistics can utilize from predictive models*"

3-3:15 **Break (Room 105)**

3:15-4:15  **Demissie Alemayehu, PhD,** Head, Statistical Research and Data Science Center, Pfizer Inc. "*Trending topics in enhanced clinical research and drug development*"

Naitee Ting, PhD, Director, Biostatistics and Data Science, Boehringer-Ingelheim

"Subgroup analysis in clinical development of new drugs"

Abstract. In clinical development of new medicinal products, it is common to perform subgroup analysis after trial results are available. There are many reasons for performing subgroup analyses. However, most of these analyses are post hoc in nature and hence clinical findings simply from these analyses may not be thought of as confirmatory evidence. In this presentation, a few case studies are used to clarify some of the considerations in performing subgroup analysis

John Preisser, PhD, Professor, Department of Biostatistics, University of North Carolina at Chapel Hill

"Design and analysis of stepped wedge and other cluster randomized trials based upon generalized estimating equations"

Abstract. While mixed effects models for cluster randomized trials (CRTs) are widely used, less attention has been given to population-averaged models for CRTs, particularly stepped wedge (SW) trials and binary outcomes. Starting with a brief review of the primary statistical issues, this talk describes the design and analysis of longitudinal CRTs based on generalized estimating equations (GEE). Topics include (i) accurate formulae for sample size determination; (ii) joint models for the marginal mean outcome, as a function of the treatment indicator and time trend, and within-cluster correlation structure; and (iii) estimation procedures with finite-sample bias adjustments for model parameters and standard errors. The methods are applied to published cross-sectional SW-CRT data for evaluation of the effectiveness of patient-delivered partner therapy to reduce chlamydial infection among heterosexual individuals in 22 local health jurisdictions in Washington State. Ongoing and future work is summarized for the design of incomplete SW-CRTs and computationally fast GEE-type analysis of cluster-period means for SW-CRTs with large clusters.

Domenic Reda, PhD, Director, Hines VA Cooperative Studies Program Coordinating Center; Research Associate Professor, U of Illinois at Chicago; Research Associate Professor, Northwestern University Department of Preventive Medicine

"Θ1≠ Θ2, p≤.05. SO WHAT! Rethinking clinical trial design"

Abstract. Recently, reproducibility of clinical trial results has been called into question. Some have advocated using p≤.005 as the level of evidence needed to declare a significant treatment effect instead of the traditional p≤.05. Others question the reliance on p-values to determine whether a result is meaningful and recommend interpretation of research findings using confidence intervals. We explore the issue and propose several options to consider when designing a clinical trial.

Max Kuhn, PhD, Software Engineer, RStudio, PBC

"What inferential statistics can utilize from predictive models"

Abstract. In practice, the main objective function for models built for inference is only a function of statistical significance. This can lead to problems. Predictive models tend to focus on empirical validation to evaluate models. Can these techniques be applied to inferential techniques and how could they benefit? A few examples are used as illustration.

Demissie Alemayehu, PhD, Head, Statistical Research and Data Science Center, Pfizer Inc.

"Trending topics in enhanced clinical research and drug development"

Abstract. With the growing cost and complexity of drug development, novel approaches for the design and analysis of trials and data sources are increasingly proposed to enhance efficiency and optimize sample size requirements. These approaches may especially be attractive options in situations where traditional randomized controlled trials are impractical or not feasible for operational or ethical reasons. Examples of trending approaches that are specifically intended to optimize subject allocation include use of external control groups and the so-called multi-armed bandit designs. However, the validity of the evidence generated from such trials is dependent on several factors, including research trial objectives, data quality and disease natural history. In this talk, we highlight common issues that arise in the implementation of selected approaches, and provide examples of applications in regulatory settings, where appropriate.