ASA 20TH ANNUAL CT CHAPTER MINI-CONFERENCE

VIRTUAL EVENT

Friday, April 29, 2022

9:00 am – 4:00 pm EDT

REGISTRATION

[Registration Link]

Registration Fee: $20*

*Student and postdocs can request this fee be waived

WEBCAST

Zoom meeting link will be sent in a confirmation email after registration.

Questions? Contact pkohli@conncoll.edu

SCHEDULE

9:00-9:10 *Opening Remarks*, **Priya Kohli, PhD**, Associate Professor of Statistics, Assistant Chair of the Mathematics and Statistics Department, Connecticut College

9:10-9:45 *"U.S. Regulatory Efforts in Complex Innovative Trial Design"*, **John Scott, PhD**, Director, Division of Biostatistics, Center for Biologics Evaluation and Research, US FDA

9:45-11:00 *"Complex Innovative Trial Designs to Expedite the Development of Drug Combination Therapies"*, **Ying Yuan, PhD**, Bettyann Asche Murray Distinguished Professor & Deputy Chair, Department of Biostatistics, The University of Texas MD Anderson Cancer Center

11:00-11:15 *Break*

11:15-12:30 *"A Complex Innovative Design of Systemic Lupus erythematosus"*, **Tony Jiang, PhD**, Director Statistical Modeling and Simulation, Center for Design and Analysis, Amgen

12:30-13:30 *Lunch Break*

13:30-14:15 *"Label-enabling dynamic borrowing with external control for OS – FDA Complex Innovative Designs Pilot Program"*, **Jiawen Zhu, PhD**, Senior Principal Statistical Scientist, Genentech, and **Herb Pang, PhD**, Expert Statistical Scientist, PD Data Sciences, Genentech

14:15-14:30 *Break*

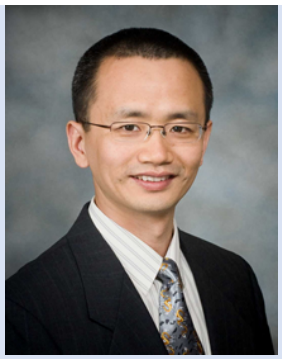
14:30-15:30 *"Hybrid control arms with electronic health record data for trials in oncology"*, **Katherine Tan, PhD**, Senior Data Scientist, Flatiron Health

15:30-16:30 *Q&A, Discussions and Closing Remarks*, **Yihua Zhao, PhD**, Director Quantitative Sciences, Flatiron Health

John Scott, **PhD**, Director, Division of Biostatistics, Center for Biologics Evaluation and Research, US FDA

Title: "U.S. Regulatory Efforts in Complex Innovative Trial Design"

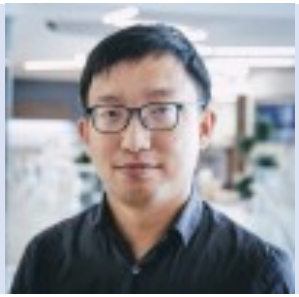
Abstract: Clinical trials provide gold standard evidence of drug safety and effectiveness and have been the backbone of pre-market regulatory decision-making for decades. Increasing costs and complexity of scientific questions have put a strain on the traditional clinical trial enterprise, sparking innovation in design, statistical methodologies, and logistics. In response to the demand for new clinical trial approaches and supported by Congress' passage of the 21st Century Cures Act in 2016, the U.S. Food and Drug Administration launched a pilot program for the review of complex innovative trial designs (CID). In this talk, I will discuss the background and structure of FDA's CID pilot program, outline relevant FDA policy on CID and adaptive designs, and describe several examples of CID proposals reviewed under the program. Finally, I will touch on next steps for FDA's CID program and for CIDs in general.



Ying Yuan, **PhD**, Bettyann Asche Murray Distinguished Professor & Deputy Chair, Department of Biostatistics, The University of Texas MD Anderson Cancer Center

Title: "Complex Innovative Trial Designs to Expedite the Development of Drug Combination Therapies"

Abstract: Combining different treatment regimens provides an effective way to induce a synergistic treatment effect and overcome resistance to monotherapy. Development of combination therapy, however, is challenging due to the large number of possible combinations, and new potentially more efficacious compounds may become available any time during drug development. In this talk, I will introduce several master-protocol-based platform trial designs that allow efficient identification of effective combinations by adaptively borrowing information across combinations using Bayesian methods. The designs are highly flexible and allow adding new combinations during the course of the trial and early graduate effective combinations.



Tony Jiang, PhD, Director Statistical Modeling and Simulation, Center for Design and Analysis, Amgen

Title: "A COMPLEX INNOVATIVE DESIGN of SYSTEMIC LUPUS ERYTHEMATOSUS"

Abstract: Complex innovative designs (CID) can potentially reduce cost, shorten cycle time and increase probability of success in drug development so new medicines can be delivered to patients in a more expedited way. PDUFA VI and 21st Century Cures Act provide exciting opportunities for industry to collaborate with regulatory agencies in promoting the use of CID. In this presentation, a case study of CID of systemic lupus erythematosus accepted by the FDA CID pilot program will be described. Experiences and considerations from this case study regarding innovative design elements, rationale for the design, design evaluation through simulation, and visualization tools will be discussed.

Jiawen Zhu, **PhD**, Senior Principal Statistical Scientist, Genentech

Herb Pang, **PhD**, Expert Statistical Scientist, PD Data Sciences, Genentech

Title: "Label-enabling dynamic borrowing with external control for OS – FDA Complex Innovative Designs Pilot Program"

Abstract: In this talk, we will present a hybrid control trial design proposal in DLBCL which went through the FDA CID pilot program. The design involves both internal and external control data to support the analysis of secondary endpoint overall survival. The background, study design, method, simulation plan, discussions during the meetings and lessons learned will be shared in the talk.

Katherine Tan, **PhD**, Senior Data Scientist, Flatiron Health

Title: "Hybrid control arms with electronic health record data for trials in oncology"

Abstract: Hybrid control arms augment the control arm of a randomized controlled trial (RCT) with external data, and aim to improve the efficiency and feasibility of trials while maintaining some randomization. Trials leveraging hybrid controls are a middle ground between full RCTs and single-arm trials with external comparators and have some qualities of each design. This talk will give an overview of hybrid-controlled trials using retrospective electronic health record (EHR)-derived external data for trials in oncology. I will give an overview of EHR-derived control arms, and analytic considerations for using EHR data as part of hybrid control arms. Then I will discuss considerations for selecting appropriate statistical borrowing methods to construct hybrid control arms. Finally, I will share some lessons learned from simulations and applications of hybrid control arms.