ARVO EDUCATION COURSE

Saturday, May 4, 2024
1-5pm
Seattle Convention Center
Seattle, WA

The ABCs of randomized controlled trials

Course organizers

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Course description

While researchers and clinicians attain expertise in their respective specialties, they usually do not acquire proficiency in clinical trial techniques as part of their PhD, postdoctoral, residency, or fellowship training. Yet, they often have research questions that would best be answered with a clinical trial, such as efficacy and safety of a new treatment. Grant reviewers may also need to understand and even judge clinical trial applications, as part of not-for-profit grant funding and /or pharmaceutical industry activities. This course provides an overview of the basics of clinical research, primarily randomized controlled trials (RCT), and the key steps to develop a good trial and /or to review grant applications that contain clinical trial activities.

Learning objectives

Attendees will leave this session with the ability to:

- Identify the different types of clinical trials.
- Describe the pyramid of evidence and the role of RCT.
- List methods to minimize bias in design and conduct of clinical trials.
- Explain role of the manual of procedures and its key elements.
- Name resources needed for RCT (clinical sites, coordinating center, reading centers etc.).

Presentations

Presenters and presentations may change.

Time	Topic	Speaker
1 PM	Introduction - overview of topics: ABCs of Clinical Trials	Penny Asbell, MD, FACS, MBA, FARVO, University of Memphis
	Geared to researchers, clinicians, grant reviewers, pharma industry who want to develop a clinical trial and/or review a grant for a clinical trial. Topics to be covered: types of clinical trials, RCT, how to minimize bias, elements of the manual of procedures, introduction to power calculations, resources needed for RCT, role of PI; panel for Q & A.	

Time	Topic	Speaker
1:05 PM	Clinical Trial Designs	Jimmy T. Le, ScD, MA, National Eye Institute
	Clinical trial design refers to the structure and sequence of activities that clinical trialists (investigators) follow to answer a specific research question. The most common clinical trial design uses a parallel group, two-arm, randomized approach, where participants are divided into different intervention groups, such as treatment versus active comparator or placebo. Investigators observe (follow-up) participants for the outcome of interest, and they compare differences in outcomes to determine the effect of the intervention. Other design choices exist, and they hinge on study objectives, resource availability, and ethical consideration. These design choices include altering participant allocation ratios, including multiple arms, or adopting elements such as crossover, factorial, or cluster-randomized designs. An overview of clinical trial designs will cover their impact on study methods, analysis, and interpretation; and key considerations such as power calculations and sample size.	
1:40 PM	Key steps for a successful clinical trial: start with a good question.	Penny Asbell, MD, FACS, MBA, FARVO, University of Memphis
	An overview of types of clinical trials will be reviewed and why randomized controlled trials (RCT) typically offer the best evidence on efficacy and safety of new treatments. Key decisions needed to get started and the importance of clearly stating the question to be answered by the clinical trial. Defining your specific aims and the key ingredients needed for a clinical trial. List of the 20 steps to be addressed for clinical trial development and successful completion of the trial.	
2:10 PM	Overview of concept for power calculations for RCTs	Mae O. Gordon, PhD, Washington University School of Medicine
	"How big a sample do I need for statistical significance?" is the most frequent question received by statisticians. This session will highlight how to answer this question when talking to a statistician and/or when using on-line calculators.	
2:30 PM	Break	
2:45 PM	How to minimize bias?	Mae O. Gordon, PhD, Washington University School of Medicine
	We strive to achieve rigorous and reproducible preclinical and clinical research. Data corrupted by bias/error cannot be rescued by statistics! This session will present smart study designs to mitigate bias/error including: Randomization to balance study groups; Masking to reduce placebo effects; Independent graders to assess outcome; and Granular measurement units to increase	

Time	Topic	Speaker
	statistical precision.	
3:20 PM	Trial protocol, manual of procedures, and data and safety monitoring	Maureen G. Maguire, PhD, FARVO, University of Pennsylvania
	The protocol for a clinical trial provides key information to the investigative team, the monitoring board, and external parties of the trial's objectives, design, organization, and administration. Multi-center clinical trials benefit by also developing a manual of operations that details the methods and procedures that will take place during the trial. This is especially useful for those involved in enrolling and following trial participants. Oversight of trials is frequently provided by a data and safety monitoring committee, whose members are charged with ensuring the safety of trial participants and the integrity of the trial's conduct.	
3:50 PM	When and which approvals and regulations are needed in RCTs? (e.g., IRB application, consent, registration of trials, and data sharing)	Tianjing Li, MD, MHS, PhD, University of Colorado Anschutz Medical Campus
	In this workshop session, participants delve into the crucial aspects of approvals and regulations in conducting randomized controlled trials (RCTs). The focus is on introducing the landscape of ethical and regulatory considerations surrounding RCTs. Additionally, the workshop delves into the significance of trial registration and the growing importance of transparent data sharing practices. Through real-world examples and interactive discussions, attendees leave with a good understanding of the ethical framework and regulatory requirements that underpin the successful design and execution of RCTs.	
4:10 PM	Leading the Way: The Role of the Principal Investigator in Randomized Clinical Trials	Susan A. Cotter, OD, MS, Marshall B. Ketchum University
	The principal investigator (PI) of a randomized clinical trial (RCT) is responsible for all aspects of the clinical trial, including its ethical conduct, patient eligibility, and protocol adherence. Other key responsibilities include ensuring adherence to ethical guidelines and regulatory requirements, obtaining necessary IRB approvals, overseeing the implementation of the study, training study personnel, obtaining informed consent from participants, monitoring the trial's progress, managing data collection, ensuring participant safety, and adhering to Good Clinical Practice (GCP) standards.	
4:30 PM – 5 PM	Panel: Open Q&A with presenters	