SCT Newsletter Vol. 8

January, 2017

President's Message

Happy New Year! Since our December 2016 newsletter went out, we have notified people who submitted abstracts for the Liverpool meeting whether they will be on the program. As mentioned then, we had a record-breaking number of submissions and had to make some difficult decisions what could get on the program. If you have not been notified yet, please e-mail our management office sct@Fernley.com .

In this newsletter we provide more information about our three keynote speakers for the conference as well as the pre-conference workshops. And of course, meeting registration is open. **Take advantage of the early bird registration rates, which are good until February 20.**

We also have a new column in this newsletter which I am sure you will enjoy. Periodically, we will feature an interview with someone who has been a prominent member of the Society. This month we feature Dr. Barbara Hawkins from Johns Hopkins University. Barbara has been an active member of the Society since its inception, a regular attendee/presenter at virtually every annual meeting and an inspiration to many. I am sure you will enjoy learning more about her.

We are now accepting nominations for the David Sackett Trial of the Year Award. See the article in this newsletter and send us your nomination! The winner will be announced at our annual meeting, and hopefully we will have the principal investigator attend and give a talk about the trial.

Finally, registration is now open for the 10th Annual UPenn Conference on Statistical Issues in Clinical Trials, which is co-sponsored by the Society for Clinical Trials. The theme for the conference is **Current Issues Regarding Data and Safety Monitoring Committees in Clinical Trials**. It will be held April 19, 2017 in Philadelphia, PA.

Keynote Speakers: SCT's 38th Annual Meeting in Liverpool, UK

We are delighted to announce the Keynote Speakers for the 38th Annual Meeting of The Society for Clinical Trials, and the 4th International Clinical Trials Methodology Conference.

Professor Hywel C. Williams DSc, FMedSci, NIHR Senior Investigator

Hywel became Director of the UK NIHR Health Technology Assessment Programme in 2016, having chaired the HTA commissioning board from 2010-15. The NIHR HTA Programme is the largest UK funder of pragmatic clinical trials and other studies that can directly influence clinical practice in the UK National Health Service.

The HTA Programme is over 20 years old and manages a portfolio of over 400 active studies with an annual spend of around £76 million. About half of HTA work is commissioned following extensive topic identification and prioritisation, and half is researcher-led. Hywel cares passionately about research that is directly relevant to the NHS and has been a champion of public and patient involvement in research.

His own research interests include conducting independent clinical trials and systematic reviews of interventions to prevent and treat skin diseases. Hywel co-directs the Centre of Evidence-Based Dermatology at the University of Nottingham and is consultant paediatric dermatologist at Nottingham University Hospitals NHS Trust. Hywel is an NIHR senior investigator and Fellow of the Academy of Medical Sciences.

Professor Williams will be speaking on Wednesday 10th May.

Dr. Susan Ellenberg, PhD., Professor of Biostatistics

Dr. Susan Ellenberg is Professor of Biostatistics, Department of Biostatistics and Epidemiology, Perelman School of Medicine at the University of Pennsylvania. Her research has focused on practical problems and ethical issues in designing, conducting and analyzing data from clinical trials, including surrogate endpoints, data monitoring committees, clinical trial designs, adverse event monitoring, vaccine safety and special issues in cancer and AIDS trials. At Penn, in addition to her teaching and administrative duties she serves as senior statistician for several multicenter clinical trials and directs the Biostatistics Core of the Penn Center for AIDS Research and chairs the organizing committee for the annual Penn conference on statistical issues in clinical trials.

Prior to her appointment at Penn, Dr. Ellenberg held positions of increasing responsibility in the federal government, including service as Director of the Office of Biostatistics and Epidemiology in the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration, and as the first Chief of the Biostatistics Research Branch in the Division of AIDS, National Institute of Allergy and Infectious Diseases.

Dr. Ellenberg is a Fellow of the American Statistical Association, the Society for Clinical Trials and the American Association for the Advancement of Science, and is an elected member of the International Statistical Institute. A second edition of her book, *Data Monitoring Committees in Clinical Trials: A Practical Perspective*, co-authored with Drs. Thomas Fleming and David DeMets, is in preparation.

Dr. Ellenberg will be speaking on Monday 8th May

Dr. J. Michael Gaziano, MD, MPH

Principal Investigator, Million Veteran Program and *Scientific Director of the Massachusetts Veterans Epidemiology Research and Information Center (MAVERIC),* VA Boston Heathcare System*; Chief, Division of Aging,* Brigham and Women's Hospital*, Professor of Medicine,* Harvard Medical School; all in Boston, Massachusetts

Dr. Gaziano is a preventive cardiologist and internationally recognized chronic disease epidemiologist whose research interests include the epidemiology of chronic diseases using large data sources. He has a particular interest in the lifestyle, metabolic, biochemical and genetic determinants of common chronic disease such as cardiovascular disease and cancer.

A centerpiece of his research involves the conduct of observational studies and trials that are imbedded in a health care system and the curation of electronic health data from many sources. He serves as one of two PIs of the Million Veteran Program (MVP), a project that will enroll one million veterans into a longitudinal cohort with stored biospecimens, self-reported data and the rich electronic clinical and administrative data available in the VA. To date over 450,000 veterans have been enrolled into MVP. He is principal investigator of the Physicians' Health Study, a large-scale trial-based cohort of over 29,000 physicians followed for over 30 years. He has also served as PI, Co-PI or co-investigator on a number of other cohort studies and large-scale trials. He serves on advisory committees for the Precision Medicine Initiative and the UKBiobank.

Dr. Gaziano oversees several fellowship programs and teaches advanced epidemiology at the Harvard School of Public Health. He has published over 500 journal articles, reviews, book chapters and books. He also serves as an Associate Editor for the Journal of the American Medical Association. He is a Fellow of the Royal College of Physicians.

Dr. Gaziano will be speaking on Tuesday 9th May.

SCT Spotlight – Dr. Barbara S. Hawkins
Written By: Leslie Ain McClure

I recently had the pleasure of speaking with Dr. Barbara S. Hawkins, Professor Emeritus of Ophthalmology and Professor of Epidemiology at Johns Hopkins University. Dr. Hawkins was SCT President from 1998-1999, and was involved in the Society in numerous other roles.

Barbara took an atypical path to clinical trials. Her undergraduate degree is from North Carolina State University, where she majored in chemical engineering and minored in mathematics. After graduating, she spent 8 years as a mathematician with an aerophysics research group at the Martin-Marietta Corporation in Baltimore. During her time there, she completed a master's degree in applied mathematics before moving to the University of Maryland School of Medicine in 1970. She began studying for a PhD in mathematical statistics (in 1971); however, the department disbanded at the end of her first year, at which time she transferred to the math department for a year, followed by biostatistics for another year. However, in 1973, the chair of biostatistics (also her advisor) left to spend 2 years in Australia, and she dropped out of the program. After various life-events disrupted her studies, she eventually completed her PhD in Epidemiology at Johns Hopkins in 1992.

Barbara has focused her research on multicenter randomized trials in ophthalmology. After moving to Johns Hopkins, she worked on a large Indonesian field trial of vitamin A supplementation for prevention of xerophthalmia, working in both Indonesia and the US. In 1981, she was asked to take over as the principal investigator of the coordinating center for the Macular Photocoagulation Study, a set of multicenter NIH-sponsored trials, and eventually was named the Director of the Wilmer Clinical Trials and Biometry group. The group was composed of statisticians, epidemiologists, systems analysts, programmers, research coordinators, clinical monitors and other personnel participating in the design, data collection, data management, statistical analysis and reporting, study monitoring and preparation of manuscripts from multicenter trials. The group was disbanded in 2008, when Dr. Hawkins retired from the full-time faculty.

As Professor Emeritus of Ophthalmology, Barbara has been involved with many projects, including preparing data and documentation from completed trials for long-term storage and access; publishing with study groups; serving as a co-investigator of the Cochrane Eyes and Vision U.S., and examining the use of shams in surgery trials. She remains active in the SCT as a reviewer for *Clinical Trials*, and reviews manuscripts for several ophthalmology journals. A major part of her time and effort is devoted to assisting trainees and young faculty to develop and publish their research. She continues to publish her own research in peer-reviewed journals.

I asked Barbara about her life outside of work, and here's what she had to say:

> "In 1992, as a "scratch" beginner, I started studying piano at the Peabody Institute in Baltimore. I was fortunate to have a wonderful teacher during my first 3 years and two more excellent (and patient) teachers after she moved to Colorado. I have enjoyed learning the piano and learning music theory and history; however, the loss of vision in my left eye due to glaucoma has made reading music increasingly difficult. As an adult beginner, I relied on my (learned) ability to read music and did not worry about memorization. Now, I am struggling with memorizing my music. The current "memory" project is J.S. Bach's French Suite V. For the first time, I also am learning to play with figured bass notation.

> Besides learning piano, I am interested in most kinds of music. With my husband (Steve Singer, who once also was active in SCT), I frequently attend recitals, concerts, and other musical events. We also are fond of theater. We subscribe to performances at several theaters in Baltimore; each July we attend the Contemporary American Theater Festival in Shepherdstown, West Virginia.
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> Reading always has been one of my favorite pastimes. As long as I have reading vision, I expect it will remain so. I also like to work outdoors. Some people garden but mostly I weed and prune as there is no room to put in new plantings and beds.
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> My husband and I like to travel, both inside the U.S. and elsewhere. We hope to fly from Liverpool to Singapore next May after the joint meeting to attend an ophthalmology meeting in Singapore. After the latter meeting, we expect to visit Australia and possibly a few Southeast Asian countries. Travel has encouraged me to study several languages in addition to those I learned in high school and college; I enjoy those studies. Our most recent trips were 5 weeks in Peru in November-December 2014, a month-long road trip to Mount Rushmore in summer 2015 (punctuated with stops to visit friends and relatives), and a 3-week road trip through the Canadian Maritime Provinces in May 2016."

Barbara has been an active member of SCT since it was organized. She has served in many roles in the Society and has enjoyed participating in conferences; she recalled the conference in New Orleans as one of her favorites! She appreciates interacting with the community of clinical trialists she finds at the SCT annual meetings, and enjoys learning about new methods and approaches being used in current trials. Her advice to new researchers: "Never stop learning; never stop writing!!"

Thanks to Dr. Hawkins for sharing so much about herself – a true role model for clinical trialists, and a pioneering woman in our field, even if she doesn't see herself through that lens.

Center for Clinical Epidemiology & Biostatistics (CCEB)

Perelman School of Medicine
University of Pennsylvania

10th Annual Clinical Trials Conference

REGISTRATION NOW OPEN

10th Annual University of Pennsylvania Conference on Statistical Issues in Clinical Trials

TOPIC

Current Issues Regarding Data and Safety Monitoring Committees in Clinical Trials

When: Wednesday, April 19, 2017
Time: 8:00 A.M. to 5:00 P.M.
Where: University of Pennsylvania, Philadelphia

For further information please visit:
http://www.med.upenn.edu/cceb/biostat/ClinTrials17_index.shtml

Faculty & Provisional Talks:

Thomas Fleming, PhD, University of Washington
Emerging Challenges in the Practice of Clinical Trial Data Monitoring Committees

David DeMets, PhD, University of Wisconsin
The Independent Statistician Model: How Well is it Working?

Pamela Shaw, PhD, University of Pennsylvania
Choosing Monitoring Boundaries: Balancing Risks and Benefits

James Neaton, PhD, University of Minnesota
How to Construct an Optimal Interim Report: What the DMC Does and Doesn't Need to Know

Panelists:

Barry Davis, MD, PhD, University of Texas
Kay Dickersin, PhD, Johns Hopkins University
Dennis Dixon, PhD, NIAID (retired)
Frederick Ferris, MD, NEI, NIH
Judith Goldberg, ScD, New York University
David Kerr, MS, Axio Research
Stephen Kimmel, MD, MSCE, University of Pennsylvania
John Lachin, ScD, George Washington University
Maureen Maguire, PhD, University of Pennsylvania
Corsee Sanders, PhD, Genentech
Steve Snapinn, PhD, Amgen
Janet Wittes, PhD, Statistics Collaborative, Inc.

Annual Conference: Pre-Conference Workshops

These pre-conference workshops will take place on Sunday 7th May and will be short courses on topical methods and issues relating to clinical trials and will be 4 hours per workshop. The focus will be on education and training and will include hands-on work and plenty of time for questions and discussions.

Time	Stream 1	Stream 2	Stream 3
09:00 - 13:00	Towards Evidence-Based Recruitment and Retention Strategies: Advancing Trial Efficiency Through Embedded Recruitment and Retention Trials	Learning Bayesian Methods and Adaptive Designs: Concepts, Tools, and Applications	Understanding and Interpreting Health Economic Evaluation Alongside Clinical Trials
13:00 - 14:00	Lunch Break (Lunch Not Provided)	Lunch Break (Lunch Not Provided)	Lunch Break (Lunch Not Provided)
14:00 - 18:00	Demystifying Casual Inference: Assessing Efficacy When Patients Depart From Randomised Treatments	Using R for Early-Phase Dose Finding Studies	Data Monitoring Committees (DMCs): Member Responsibilities and Interpretation of DMC Reports for Effective Decision-Making

To read more and register, visit http://www.ictmc2017.com/programme/workshop/

Call For The David Sackett Trial Of The Year Nominations

We Are Accepting Nominations For The Trial Of The Year 2016!

Each year The Society for Clinical Trials presents an award to the randomized clinical trial published (either electronically or in print) in the previous year (2016 in this case) that best fulfills the following standards:

- It improves the lot of humankind.
- It provides the basis for a substantial, beneficial change in health care.
- It reflects expertise in subject matter, excellence in methodology, and concern for study participants.
- It overcame obstacles in implementation.
- The presentation of its design, execution, and results is a model of clarity and intellectual soundness.

Nominations Due By: February 20, 2017

The award will be presented at the 38th SCT Annual Meeting held in conjunction with the 4th International Clinical Trials Methodology Conference (ICTMC), May 7-10, 2017, in Liverpool England.

To nominate the trial that you think best meets these standards, please send the required items below via email to Scott Evans (evans@sdac.harvard.edu), Chair of the David Sackett Trial of The Year Committee:

- The full citation(s)
- A letter outlining how the trial meets each standard
- Other comments regarding why the trial deserves the award.

The trial must be closed to patient follow-up and have been published in print or electronically in the calendar year 2016.

The SCT 2015 Trial of the Year was a Randomized Trial of Peanut Consumption in Infants at Risk for Peanut Allergy. Du Toit et.al. N Engl J Med 2015;372:803-13. DOI: 10.1056/NEJMoa1414850

Click here to read more about the past Trial of the Year recipients.

News From Clinical Trials Transformation Initiative

DMCs Project Paper in JAMA

A new JAMA publication describes key points from CTTI's recommendations for Data Monitoring Committees (DMCs). These recommendations are intended to enhance the functioning of a DMC, beginning with training of members. Other issues addressed by the recommendations include the role and responsibilities of the DMC, composition of members, development of a charter, and communication with the trial sponsor and others. Thank you to co-authors of the paper: Karim Calis (FDA), David DeMets (University of Wisconsin), and Roger Lewis (UCLA Harbor Medical Center).

For more information on best practices for the use of DMCs, see the recording of a CTTI webinar held in June 2016.

New Publication from IND Safety Advancement Project

Findings and recommendations from CTTI's IND Safety Advancement Project were recently published in JMIR Cancer. The recommendations, released earlier this year, describe desired attributes of electronic portals for expedited safety reporting. Implementation of these recommendations can improve efficiency and reduce paperwork burden for sites and sponsors. Thank you to co-authors of the paper: Raymond Perez (University of Kansas), Shanda Finnigan (NIH), Krupa Patel (Merck), Shanell Whitney (Eli Lilly), and Annemarie Forrest (CTTI).

FDA and OHRP Publish Final Guidance on Electronic Informed Consent

The FDA and the OHRP have published a joint final guidance on the use of electronic informed consent. This guidance addresses questions about using electronic systems to obtain informed consent for both Health and Human Services (HHS)-regulated human subject research and FDA-regulated clinical investigations of medical products, including drugs, biologics, and devices.

Liverpool Trivia Questions and Answers

Last month's Liverpool trivia question:
What is the name of the first horse to win the Grand National three times at Liverpool's Aintree racecourse?

And the answer is:
Red Rum

The next question is:
What song gave The Beatles their first US number 1 hit?

Check the February SCT Newsletter for the answer!