SCT Newsletter Vol. 7

December, 2016

President's Message

The SCT train keeps moving along. Can you believe it is already the end of 2016?

Thankfully, with excellent support from all our volunteers, the plans for the Liverpool meeting are moving along nicely. We had a RECORD-BREAKING number of abstracts submitted! Contributed abstract submissions surpassed 600, three times the number submitted for the 2015 Washington DC area meeting, and about 2 ½ times the number for the 2016 Montreal meeting.

We have our 3 keynote speakers lined up (learn more next month) and the Scientific Programme and Education committees have completed their reviews for all the submissions. As I write this, both groups are busily putting all the pieces together for scheduling of the workshops, invited sessions, contributed sessions and contributed posters. I have already made my airline reservations to take advantage of some very nice fares from Chicago to London Heathrow (I'm spending a few days sightseeing around London before I take the train to Liverpool to do even more sightseeing and get ready for the meeting).

Oh, and by the way, meeting registration is open! Register by February 10, 2017 to take advantage of the early-bird discount. And don't forget to sign up for the Tuesday evening banquet, which replaces the SCT mixer for this year. Fine dining, camaraderie, music, and dancing.

Are you interested in serving for a leadership position in SCT? Nominations are now open for SCT president and three Board of Directors positions. See the article from KyungMann Kim, past president of SCT and Nominating Committee Chair.

The SCT/QSPI conference last month was a big success. I really enjoyed all the talks and getting to meet some of you. Conference Co-Chairs Janelle Charles and Josh Chen have an article summarizing the meeting in this newsletter. Thanks so much to Janelle and Josh (and of course, Mike Hale, who leads QSPI, and the rest of the program committee) for putting together such a great program.

Get ready for the 10th Annual University of Pennsylvania Conference on Statistical Issues in Clinical Trials! See the Save the Date notice in this newsletter. (And by the way, one of the organizers is one of our keynotes for the Liverpool meeting).

However you celebrate this time of year, I want to wish you a happy holiday season and hope that your 2017 is your best year yet. Thank you for your continued support of the Society for Clinical Trials.

If you have an article you would like us to consider for the next edition, please send it to sct@fernley.com by January 9.

Domenic J. Reda
SCT President

Registration Is Now Open: SCT's 38th Annual Meeting in Liverpool, UK

For the first time, the Annual Meeting of the Society for Clinical Trials is being jointly held with the International Clinical Trials Methodology Conference, providing one of the largest international platforms for those working in clinical trials to collaborate and discuss new ideas.

This event will bring together international colleagues working in clinical trials, including trialists, methodologists, clinicians and allied health professionals. The programme will include three Keynote lectures, and a range of sessions designed to stimulate discussion and engagement during this unique event.

View The Preliminary Programme

Register To Attend The Annual Meeting

Co-chair Reflections:
2016 SCT-QSPI/FDA Fall Innovation Workshop

By Joshua Chen, PhD and Janelle K. Charles, PhD

It has been our pleasure to serve as co-chairs for the 2016 SCT-QSPI/FDA Fall Innovation Workshop which was held on November 14-15, 2016, at The Universities at Shady Grove Conference Center, Rockville, Maryland, USA!

Together with the Program Organizing Committee, we selected the theme, *New Approaches in Clinical Science for Developing Evidence*, for this year's workshop. While not intended to be a large-scale conference, such as the SCT Annual Meeting, the workshop was designed to provide a forum fostering open and constructive dialogue for professionals in industry, academia, government, among others, who have an active interest in advancing the science and practice of clinical trials and improving healthcare and the practice of medicine. In keeping with this vision of an interactive workshop, the program comprised four half-day sessions focused on timely, relevant, and provocative topics, which were carefully selected by the Committee. Of course this was not a simple task, given the many topics related to recent advances in clinical research that would have also been of interest our target audience. In the end, three topics were selected, one of which was split into two half-day sessions on the second day of the workshop. Each session featured brief talks by exceptional speakers and panelists, and allotted substantial time (up to an hour!) for audience engagement.

Here are a few highlights from the 2016 workshop …

On **Day 1**, Dr. Lyric A. Jorgenson, Deputy Executive Director of the Cancer Moonshot Task Force in the Office of the Vice President, opened the workshop with her keynote address titled, *Sustaining Momentum under the Cancer Moonshot: Strengthening the Clinical Research Enterprise*. Dr. Jorgenson provided an overview of the Vice President's strategic vision for transforming cancer research and care under the Cancer Moonshot, and the Task Force's roadmap for achieving these aims. She emphasized the role of clinical research under the Cancer Moonshot, and how the effort is working to strengthen the clinical research enterprise. Drs. Thomas Birkner (CDER/FDA) and Marc Walton (Janssen R&D) co-chaired the morning session titled, *Estimands – Clinical Objectives, Statistical Methods, and Missing Data*. After three presentations by Drs. Estelle Russek-Cohen (CBER/FDA), Marc Walton (Janssen R&D) and David Ohlssen (Novartis), a panel of experts that included the speakers and additional panelists, Robin A. Elliott (Parkinson's Disease Foundation & World Parkinson Congress), Steven A. Geller (Centennial Medical Group) and Thomas Permutt (CDER/FDA), answered questions from the audience and discussed challenges relating to what are relevant questions clinical trials might be desired to answer, and how that might be done from multiple perspectives (clinical trialist, patient, practicing clinician, statistician, and regulator). The afternoon session was titled, *Challenges and Opportunities in the Design and Implementation of Basket and Platform Trials*, and co-organized by Dr. Susan Halabi (Duke University) and Dr. Lisa LaVange. Drs. Richard Simon (National Cancer Institute), Ben Saville (Berry Consultants) and Richard Schilsky (American Society of Clinical Oncology) presented their research on the statistical challenges in the design and logistical implementation of innovative basket and platform trials. Dr. Lisa LaVange (CDER/FDA) was a discussant and shared her views on these presentations.

On **Day 2**, Dr. Robert M. Califf (FDA Commissioner) welcomed participants and opened the meeting in a pre-recorded video message. In this video, Dr. Califf emphasized the importance of innovative approaches to generate clinical evidence for decision making. The morning session was titled, *Real World Evidence – Sources and Applications*, and co-chaired by Drs. Olga Marchenko (Quintiles) and John Scott (CBER/FDA). Drs. Benjamin Eloff (CDRH/FDA), Jonathan Jarow (CDER/FDA) and Hector Izurietta (CBER/FDA) presented potential regulatory applications of real world data in CDRH, CDER and CBER, respectively. Drs. Ted Lystig (Medtronic) and Ryan Ferguson (US Department of Veterans Affairs) presented CTTI update on registry trials and VA's Point of Care Clinical Trial, respectively. Drs. Michael Hale (Shire) and Yves Rosenberg (NHLBI, NIH) co-organized the afternoon session with focus on *Transforming Real World Data into Evidence*. Greg Powell (GlaxoSmithKline), Sally Okun (PatientsLikeMe) and Carol Pamer (CDER/FDA) spoke about listening social media for drug safety, and Michael S. Lauer (National Institutes of Health) and Alyson Karesh (CDER/FDA) presented pragmatic clinical trials. Dr. Mark Levenson (CDER/FDA) joined the speakers for an interactive panel discussion with the audience that followed the presentations.

There were approximately 100 registrants for this year's workshop and we have received much positive feedback from attendees. Such a well-received workshop would not have been possible without the dedicated efforts and timely contributions of the Program Organizing Committee (listed below). We also extend special thanks to Drs. Michael Hale and John Scott for sharing their experiences and insights from co-chairing the 2014 SCT-QSPI/FDA workshop, Nicolette Pelbano and Kate Ho from Fernley & Fernley who provided administrative support in organizing Committee meetings and logistics for the workshop, and Dr. Domenic Reda, President of the SCT, who actively participated in planning meetings and provided guidance on key issues.

Program Organizing Committee: Thomas Birkner, CDER/FDA; Ivan S.F. Chan, AbbVie; Li Chen, Amgen; Michael Hale, Shire; Susan Halabi, Duke University; Theodore Karrison, The University of Chicago; Bo Li, CDER/FDA; Olga Marchenko, Quintiles; José Pinheiro, Janssen Research & Development; Renée Rees, CBER/FDA; Yves Rosenberg, NHLBI/National Institutes of Health; John Scott, CBER/FDA; David Stock, Bristol Myers Squibb; Marc Walton, Janssen Research & Development.

Call for Nominations

Nominating Committee
By KyungMann Kim, Chair

SCT's Nominating Committee is soliciting nominees for President-elect and members of the Board of Directors (three).

Please let us know of anyone that you would recommend as a possible candidate. If you, yourself, are interested in serving the Society in either of these roles, please do not hesitate to put your name forward. The Nominating Committee will consider all proposed candidates and, from the list of willing nominees, select a slate of candidates for the ballot. This year, the Nominating Committee consists of Domenic Reda (President), Ted Karrison (President-elect), Elizabeth Wright (Board member), Li Chen (Board member), Dixie Eklund (Member at large), Jane Blazeby (Member at large), and KyungMann Kim (Member at large appointed as Chair by President).

To qualify as a candidate, the person should be:

1. a member of the Society, preferably one who is or has recently been an active participant in SCT activities and/or committees, and

2. someone who is well respected in their area of clinical trials, whether it be in information technology, patient care, statistics, project management, regulatory affairs, epidemiology, laboratory sciences, or any of the other disciplines that are important to the planning, conduct, analysis, interpretation and reporting of good clinical trials.

Please contact KyungMann Kim (kyungmann.kim@wisc.edu) with your nominations by January 15, 2017.

Center for Clinical Epidemiology & Biostatistics (CCEB)

Perelman School of Medicine
University of Pennsylvania

10th Annual Clinical Trials Conference

SAVE THE DATE!

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

Registration to open January 4, 2016

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

Faculty & Provisional Talks:

Tom 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

Jim 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, PhD, University of Texas
Kay Dickersin, PhD, Johns Hopkins University
Dennis Dixon, PhD, NIAID (retired)
Rick Ferris, MD, NEI
Judy Goldberg, ScD, New York University
David Kerr, MS, Axio Research
Steve 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.

News From Clinical Trials Transformation Initiative

JAMA Publishes CTTI's Recommendations for Data Monitoring Committees

An article describing key points from CTTI's Recommendations for Data Monitoring Committees (DMCs) appears in this week's edition of *JAMA*. As independent bodies able to review accumulating data for ongoing clinical trials, DMCs fulfill a unique and vital role in ensuring the scientific integrity and safety of clinical trials. (The article can be found here: http://jamanetwork.com/journals/jama/article-abstract/2592508)

The article details the following:

- The unequivocal need for DMCs to review unmasked data
- The importance of DMCs reviewing safety and efficacy data together
- Appropriate qualifications for DMC members

With the growing use of DMCs for trial oversight, these evidence-based best practices for DMC establishment and conduct can help ensure proper DMC functioning to fulfill their mission. CTTI is pleased to see these recommendations reach a broad audience and is encouraging widespread implementation.

View CTTI's complete recommendations for additional consensus-driven best practices for DMCs - https://www.ctti-clinicaltrials.org/files/recommendations/dmc-recommendations.pdf

Learn more about CTTI's DMCs Project - https://www.ctti-clinicaltrials.org/projects/data-monitoring-committees-dmcs.

New Insights on Data Monitoring Committees Published in Clinical Trials

CTTI has published a new article in *Clinical Trials*: *Understanding the Functions and Operations of Data Monitoring Committees: Survey and Focus Group Findings*.This article shares insights gathered from research with clinical trial sponsors, data monitoring committee (DMC) members, regulators, and other stakeholders. The results were used in the development of CTTI's official recommendations (https://www.ctti-clinicaltrials.org/files/recommendations/dmc-recommendations.pdf) to improve the functioning of DMCs and quality of trial oversight.

Read this publication for key findings related to:

- The role of DMCs and when they are needed
- Typical DMC composition and methods for identification of members
- Best practices for DMC charters, member contracts, and meetings
- Methods for enhancing DMC communications
- Views on DMC member qualifications and ways to develop training

To learn more about CTTI's DMC Project - https://www.ctti-clinicaltrials.org/projects/data-monitoring-committees-dmcs.

CTTI Publishes Findings on Patient & Physician Perceptions of Streamlined Development

CTTI has published findings on patient and physician attitudes regarding streamlined development approaches for antibacterial drugs in *BMJ Open*. Through a series of interviews and focus group discussions, CTTI found that patients and physicians agreed on the usefulness of streamlined approaches in situations of unmet need, but both groups also emphasized the need for careful oversight, transparency in risk communication, and continuous monitoring and reporting of safety and efficacy post-approval.
(http://bmjopen.bmj.com/content/6/11/e013561.full?keytype=ref&ijkey=lROquFpaDybKTdk)

These findings, which resulted from CTTI's Unmet Need in Antibiotic Development Project can help inform the future use of streamlined drug development approaches and communication with stakeholders. (https://www.ctti-clinicaltrials.org/projects/unmet-need-antibiotic-development)

CTTI gathered input on these findings and their impact in a multi-stakeholder expert meeting; a meeting summary is available for additional context: (https://www.ctti-clinicaltrials.org/sites/www.ctti-clinicaltrials.org/files/abdd_unmetneed_expertmeetingsummary_2016-03-01_final.pdf)

Thank you to the interview and focus group participants, expert meeting attendees, and project team members who contributed to this work. Read the full article here: http://bmjopen.bmj.com/content/6/11/e013561.full?keytype=ref&ijkey=lROquFpaDybKTdk

Recording Now Available: CTTI's DMC Recommendations Presented on NIH Collaboratory Grand Rounds

Did you miss our latest webinar on CTTI's Recommendations for Data Monitoring Committees? View the recording to learn evidence-based best practices for DMC composition, communication, training, and conduct to improve the quality of clinical trial oversight. View recording: https://www.nihcollaboratory.org/Pages/Grand-Rounds-11-11-16.aspx

NIH Requests Comments on Strategies for Data Management, Sharing, and Citation

The NIH has published a Request for Information (RFI) seeking public comments on how data from NIH-funded research should be managed and shared, and how to set standards for data citation. The RFI requests stakeholder feedback to help the NIH prioritize the creation of policies in this area and encourage good data stewardship. Comments will be accepted until December 29, 2016. View RFI: https://grants.nih.gov/grants/guide/notice-files/NOT-OD-17-015.html

Liverpool Trivia Questions and Answers

Last month's Liverpool trivia question:
What is unusual about the two architects who built the Anglican and Catholic cathedrals?

And the answer is:
A Catholic designed the Anglican cathedral, and an Anglican designed the Catholic cathedral.

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

Check the January SCT Newsletter for the answer!

Must See Liverpool Attraction

Tate Liverpool is the home of the National Collection of Modern Art in the north. Located on the Albert Dock within easy walking distance from the City centre, Tate Liverpool is one of the most visited galleries outside of London.

The gallery stages a changing programme of special exhibitions throughout the year, which brings together artworks from all over the world. In recent years artists featured in Tate Liverpool's special exhibitions have included Gustav Klimt, Pablo Picasso, René Magritte and Claude Monet.

The gallery also offers large displays of work from the National Collection free of charge. The Collection features the work of artists including Henri Matisse, Marcel Duchamp, Barbara Hepworth, Henry Moore and Paul Cézanne, making Tate Liverpool the ideal place to visit your favourite artwork or discover something new.

For more details, click **here**