Overcoming Barriers to International Collaboration in Rare Cancer Clinical Trials

Summary

The ASCO/NCI meeting on Overcoming Clinical Barriers to International Collaboration in Rare Cancer Clinical Trials meeting was held on December 10, 2010 in Bethesda, Maryland, following a meeting the previous day on Clinical Trials in Small Populations held at the ASCO Headquarters in Alexandria, Virginia. The NCI Cancer Bulletin published an overview of the meeting, and a more formal meeting report was prepared by the NCI. A research paper based on the meeting is now in preparation and will be submitted to a peer-reviewed journal. The meeting was sponsored by the NIH Office of Rare Disease Research, the NCI Cancer Therapy Evaluation Program, and the American Society for Clinical Oncology.

This meeting brought together international stakeholders including representatives of the NIH and the NCI-sponsored Clinical Trials Cooperative Group Program, to consider when international trials might be appropriate for studying rare cancers, develop a systematic approach to prioritize such trials, and to work on practical issues that would enable such collaborations to be conducted in a speedy and cost-efficient manner. In addition to U.S. Government (NIH, Food and Drug Administration, Office for Human Research Protections) and cooperative group representatives, other attendees included investigators from Canada, France, Japan, Korea, Singapore, the UK, the European Organisation for Research and Treatment of Cancer (EORTC), representatives from patient advocacy organizations, and industry.

This meeting’s agenda built upon prior meetings that have examined more general aspects of international collaboration in clinical trials research and on design-oriented discussions of performing trials in rare populations. This meeting focused on those instances where it would not be practical to perform a phase II or III trial in North America alone. To help make the meeting more concrete, after general presentations, break-out sessions focused on five specific research areas identified previously in international meetings: rare pediatric cancers, adult brain cancers, sarcoma, cholangiocarcinoma, and rare gynecological malignancies.
Presentations

Except as noted, most of these presentations are in Microsoft powerpoint format. Please do not re-use these slides without the permission of the author.

- **Meeting Introduction**, Jeffrey Abrams, Associate Director, Cancer Therapeutic Evaluation Program, National Cancer Institute
- **Overview**, Jack Welch, Cancer Therapy Evaluation Program, National Cancer Institute
- **Rare Trials Research in the International Setting**, Stephen Groft, Office of Rare Diseases Research, National Institutes of Health
- **Engaging Patient Advocacy Organizations for Rare Cancers**, Shannon Bell, Office of Advocacy Relations, National Cancer Institute
- **Alternative Approaches to Institutional Review Board Review**, Ed Bartlett, Office for Human Research Protections
- **A Regulatory Perspective on Rare Cancers and Orphan Drugs**, Tim Coté, Food and Drug Administration
- **The CTSU model in Rare Tumors**, Mike Montello, Cancer Therapy Evaluation Program, National Cancer Institute
- **US Cooperative Groups in International Clinical Trials**, Ted Trimble, Cancer Therapy Evaluation Program, National Cancer Institute
- **Experience of the Japanese Clinical Oncology Group: Overcoming International Barriers**, Eriko Aotani, Kitasato University Research Center for Clinical Pharmacology
- **European and American Osteosarcoma Study Group**, Jeremy Whelan, University College London Hospital
- **International Brain Studies**, Bhupinder Mann, Cancer Therapy Evaluation Program, National Cancer Institute
- **EORTC and European Perspective on Trans?Atlantic Collaboration**, Anastassia Negrouk, Head, EORTC Intergroup Office
- **Summary of the American Society of Clinical Oncology Panel on Clinical Trial Designs in Small Populations Workshop**, Samir Khleif, Chief, Vaccine Section, National Cancer Institute
- **Expediting Protocol Review and Activation**, Gareth Griffiths, Scientific Director, Wales Cancer Trials Unit (pdf)
- **The Role of Epidemiology in International Clinical Trials**, Ann Hsing, National Institutes of Health
- **Rare Cancer Registries and Biorepositories**, Yaffa Rubinstein, Office of Rare Disease Research, National Institutes of Health
- **Cancer Clinical Trials at the Korean National Cancer Institute**, Byung?Ho Nam, National Cancer Center, Republic of Korea
- **Building International Partnerships**, Matt Seymour, Director, UK National Cancer Research Network
- **The UK NCRN and NCRI**, Matt Seymour, Director, UK National Cancer Research Network
- **International Collaboration: Kitasato Institute Experience**, Eriko Aotani, Kitasato University Research Center for Clinical Pharmacology
- **INCA Initiatives on Innovative Drugs in Cancers**, Christian Cailiot, Early Drug Development, Institut du Cancer, France
- **Coordinating the Patient Voice in UK Cancer Research**, David Ardron, Chair, Consumer Liaison Group, UK National Cancer Research Network
- **Rare Paediatric Cancers**, Pamela Kearns, Deputy Clinical Director, Cancer Research UK Clinical Trials Unit
• **International Clinical Trials in Pediatric Cancers**, Greg Reaman, Chairman, Children’s Oncology Group

• **Sarcoma and Rare Solid Tumors**, Paolo Casali, Head, Adult Sarcoma Medical Oncology Unit, Istituto Nazionale Tumori, Italy

• **Sarcoma Session Overview**, Chandrajit Raut, Assistant Professor, Department of Surgery, Harvard Medical School and Jeremy Whelan, UK National Cancer Research Institute Teen/Young Adult and Sarcoma Clinical Study Groups

• **Biliary Tract Cancers Trials**, John Bridgewater, Senior Lecturer, Medical Oncology, University College London

• **Management of Biliary Tract Cancer Studies**, John Bridgewater, Senior Lecturer, Medical Oncology, University College London

• **Epidemiology of Biliary Tract Cancers**, Ann Hsing, National Cancer Institute
Meeting Output

In addition to the summary documents mentioned above, several action items resulted from this meeting:

- In the year following the meeting, the U.S. National Cancer Institute (NCI), European Organisation for Research and Treatment of Cancer (EORTC), and UK continued to meet by teleconference on a regular basis to develop a rare tumors initiative. Each organization went to their specific disease-oriented groups, queried their interest and capabilities, and prioritized five “first tier” diseases for the initiative to focus on. Diseases that would be difficult or impossible to study outside of this framework (e.g., diseases that could not be studied with accrual from only one country and diseases that otherwise lacked international infrastructure), were intentionally selected. These included: 1) penile cancer; 2) gynecological sarcoma; 3) fibrolamellar hepatocellular carcinoma; 4) rare head and neck tumors (anaplastic thyroid cancer and salivary cancers); and 5) small bowel cancers. The group identified individuals to chair disease-specific meetings at the 2011 American Society of Clinical Oncology (ASCO) conference in Chicago, with the goal of bringing together world leaders in these diseases. The US, EORTC, and UK will work together to prioritize and fund clinical trials and organize investigator infrastructure in these diseases. Although not under this framework, there will also be a meeting on biliary cancer, which was a focus during an earlier rare cancer meeting. If this effort goes well, plans exist to replicate the process at future international meetings like ASCO or the European Society for Medical Oncology (the European equivalent of ASCO).

- The international collaborative working group is developing tools to facilitate joint trials. A web portal for international investigators has been developed, is currently in beta testing, and will be rolled out at ASCO 2011. The portal is designed to provide answers to common questions about how to work with NCI from the perspective of a foreign partner.

- At the Rare Cancers meeting, a break out discussion on operational issues between EORTC and the US cooperative groups was held. The group plans to host a larger follow-up meeting during ASCO 2011, which will involve operations center personnel from the majority of US cooperative groups, the National Cancer Institute of Canada Clinical Trials Group (NCIC CTG), the EORTC, UK, and French Clinical Trial Networks. The results of that meeting will be compiled on this wiki, and the wiki will be extended to capture practical solutions that have arisen in successful collaborations between the groups.

- Since the rare cancer meeting, French cancer centers have successfully competed for phase II trials, including a sarcoma trial.