## Enabling Research in Rare Hematologic Disorders
### American Society of Hematology (ASH) Ancillary Meeting

**Friday, December 5, 2014, 9:00 am to 1:00 pm**
**San Francisco Marriott Marquis, Conference Room Pacific J**
**780 Mission Street, San Francisco, CA • Phone: (415) 896-1600**

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<td>9:00 AM – 9:30 AM</td>
<td>Nigel Key. <em>Enabling Research in Rare Hematologic Disorders</em></td>
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<td>9:30 AM – 10:10 AM</td>
<td>Cindy Leissinger and Neil Goldenberg. <em>How I Did It: From Pilots to Clinical Trials – Two Examples of the Path from Pilot Funding to NHLBI Supported Clinical Research in Rare Hematologic Disorders</em></td>
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<td>10:10 AM – 10:30 AM</td>
<td>Break &amp; Refreshments</td>
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<td>10:30 AM – 11:00 AM</td>
<td>Diane Catellier. <em>The Clinical Trials Development Resource for Hematological Disorders</em></td>
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<td>11:00 AM – 12:00 AM</td>
<td>Joseph Ibrahim and Don Brambilla. <em>Using Bayesian Clinical Trial Design for Rare Diseases</em></td>
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<td>12:00 AM – 12:20 PM</td>
<td>Sreelatha Meleth. <em>Recruitment &amp; Retention in Clinical Trials for Rare Diseases</em></td>
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<td>12:20 PM – 1:00 PM</td>
<td>Nigel Key, James Bussel, Margaret Ragni, and Marilyn Manco-Johnson. <em>Panel Discussion: Planning And Development Of Feasible And Well-Designed Clinical Trials Focused On Rare Hematologic Disorders</em></td>
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Donald Brambilla, PhD, Senior Statistician, RTI International

Dr. Brambilla has over 27 years of experience collaborating with clinical investigators on the design and execution of multicenter clinical trials, including trials in various aspects of sickle cell anemia, reduction of inhibitors of Factor VIII in hemophilia, and treatments for thrombocytopenia (ITP, ITP). He has helped develop strategies for screening, recruitment, and retention- and proposed adjustments to sample sizes to compensate for losses and crossovers that resulted in successful completion of trials in rare disorders.

James Bussett, MD, Professor of Pediatrics, Medicine, and Obstetrics at the Weill Medical College of Cornell University in New York City

Dr. Bussett’s research interests center around diagnosis and especially management of adults and children with idiopathic thrombocytopenic purpura (ITP), HIV infected patients with thrombocytopenia, and fetuses affected by autoimmune and alloimmune thrombocytopenia. He has worked with IV Ig, IV anti-D, rituximab, and most recently the thrombopoietic agents. Dr. Bussett is currently developing a clinical trial that is being supported through the NHLBI Clinical Trials Development Resource for Hematologic Disorders initiative.

Diane Catellier, DrPH, Senior Statistician, RTI International

Dr. Catellier has served as Coordinating Center (CC) PI for five NIH-sponsored Phase III clinical trials, and has first-hand knowledge of the process steps that are needed to launch a large clinical trial protocol and the barriers that exist in conducting trials. Dr. Catellier has contributed to a compendium of best practices for CCs that support large clinical trials that will serve as a resource to National Heart, Lung and Blood Institute (NHLBI) program staff for crafting application and review criteria for RFAs/RFPs and when evaluating CCs as part of a large clinical trial program.

Neil Goldenberg, MD, PhD, is Associate Professor of Pediatrics and Medicine at the Johns Hopkins University School of Medicine. HE is Director of Research and Director of the Clinical and Translational Research Organization at All Children’s Hospital Johns Hopkins Medicine (ACH JHM). He has previously served as Director of the Children’s Clinical Research Organization and Co-Director of the Pediatric Thrombosis and Stroke Programs at Children’s Hospital Colorado, and as Associate Center Director and Director of Clinical Research for the Mountain States Regional Hemophilia and Thrombosis Center. Dr. Goldenberg completed a five-year Career Development Award from the NHLBI for clinical investigation in pediatric thrombosis and has held additional research grants from the American Society of Hematology (ASH), the Hemophilia and Thrombosis Research Society and the National Hemophilia Foundation. Dr. Goldenberg is the Principal Investigator, Steering Committee Chair, or Data and Safety Monitoring Committee chair for several NIH- and industry-sponsored multicenter clinical trials, and has co-authored international clinical and clinical research guidelines.

Joseph Ibrahim, PhD, Distinguished Professor of Biostatistics at the University of North Carolina (UNC)

Dr. Ibrahim has over 20 years of experience working in cancer clinical trials. He serves as Director for the UNC Center for Innovative Clinical Trials, directs the Biostatistics and Data Management Core at UNC’s Lineberger Comprehensive Cancer Center, leads the biostatistical core for an NIH Specialized Program of Research Excellence (SPORE) grant in breast cancer, and co-leads an NIH Program Project (P01) center grant for developing statistical methods in cancer clinical trials. He has published two advanced graduate-level books on Bayesian survival analysis and Monte Carlo methods in Bayesian computation.

Nigel Key, MB ChB, FRCP, Distinguished Professor, Chief of the Section of Hematology in the Division of Hematology/Oncology, and Director of the UNC Hemophilia and Thrombosis Center

Dr. Key is an adult hematologist specializing in non-malignant hematologic disorders, particularly those affecting blood coagulation. His clinical interests include the diagnosis and management of bleeding disorders including hemophilia and von Willebrand disease, as well as arterial and venous thromboembolic disorders. He has authored well over 100 peer-reviewed articles, approximately 20 book chapters, and is co-editor of a popular textbook on disorders of bleeding and clotting.

Cindy A. Leissinger, MD, Tulane University New Orleans, LA

Dr. Leissinger is currently Professor of Medicine, and Clinical Professor of Pediatrics at Tulane University. She serves as Chief of the Hematology/Oncology Division, and is also Director of the Louisiana Center for Bleeding and Clotting Disorders. Dr. Leissinger oversees an active clinical research program that receives funding from the National Institutes of Health as well as numerous other federal and industry sources. She participates in several clinical research groups, and has been an active investigator for many research studies related to bleeding disorders, with a particular interest in Factor VIII inhibitor development and management.

Marilyn Manco-Johnson, MD is Professor of Pediatrics at the University of Colorado Denver and Director of the Mountain States Regional Hemophilia and Thrombosis Center. She is a renowned expert on pediatric hemophilia, specifically, joint disease prevention and neonatal and pediatric thrombotic disorders. She was principal investigator of the Joint Outcomes Study (JOS), the first US randomized controlled trial to compare prophylaxis with an enhanced episode-based treatment for structural joint development in young children with hemophilia.

Sreeelatha Meleth, PhD, Senior Statistician, RTI International

Dr. Meleth has more than 17 years of experience collaborating with clinicians in developing and implementing multicenter Phase 1, Phase 2, and Phase 3 clinical trial protocols. She has provided statistical support and guidance for proposals that span the gamut of health research from proteomics and biomarkers to treatment of various cancers, AIDS, cardiovascular disease, tuberculosis, several other disease conditions, and the design and conduct of randomized clinical trials and mixed-study designs that used qualitative and quantitative data collection designs.

Margaret Ragni, MD, is Professor of Medicine and Clinical Translational Science, Division Hematology/Oncology and Director, Hemophilia Center of Western PA. She is PI on a T35 student research grant, one R34 pilot study and two U34 planning grants. She collaborates on an NHLBI genotype-phenotypes von Willebrand study; an NIAID-funded organ transplantation in HIV, an NHLBI-funded hemophilia gene transfer, and an NHLBI-funded Transfusion Medicine/Hemostasis clinical trials network. She is a member of the Medical and Scientific Advisory Board, National Hemophilia Foundation and of the NHLBI Hemostasis & Thrombosis study section. She chaired the Hemophilia/WWD Subcommittee of the NHLBI State of the Science Symposium 2009, developing four clinical trial concepts, three submitted.