



AGENDA

Purpose

1. Provide forum to discuss lessons learned from the Rare Diseases Clinical Research Network.
2. Highlight successful collaborations and research advances to improve rare diseases research.
3. Highlight the involvement and essential role of patient advocacy groups to facilitate research and accrual of patients.
4. Discuss role of best practices and standards across research consortia and research networks for translation of basic discoveries to clinical practice in rare diseases.

7:30 a.m. Registration and Poster Set-up

Continental Breakfast

8:00 a.m. Welcome

Stephen C. Groft, Pharm.D. — Office of Rare Diseases Research, NIH
Barbara Alving, M.D. — National Center for Research Resources, NIH

8:15 a.m. Keynote — An NCI Experience: Multi-Center Collaborations for Clinical Trials in Rare Cancers

Margaret Mooney, M.D., M.B.A. — Chief of the Clinical Investigations Branch, CTEP, NCI

9:15 a.m. Challenges and Opportunities in Rare Disease Research

Robert C. Griggs, M.D., F.A.A.N. — University of Rochester School of Medicine and Dentistry

9:35 a.m. Training Professionals in Rare Disease Research Panel

Moderator: *Michael R. Knowles, M.D.* — University of North Carolina at Chapel Hill

Stephanie Davis, M.D. — University of North Carolina at Chapel Hill

Joanna Jen, M.D., Ph.D. — Ronald Reagan UCLA Medical Center

10:15 a.m. BREAK

10:30 a.m. Participant Recruitment, Outreach, and Dissemination Panel

Moderator: *Mark Batshaw, M.D.* — Children's National Medical Center

Christina Cornell, CPAG / RDCRN — Representative / Vasculitis Foundation, Vasculitis Clinical Research Consortium

Peter A. Merkel, M.D., M.P.H. — Boston University School of Medicine

- 10:30 a.m. Participant Recruitment, Outreach, and Dissemination Panel**
Moderator: *Mark Batshaw, M.D.* — Children’s National Medical Center
Christina Cornell, CPAG / RDCRN — Representative / Vasculitis Foundation, Vasculitis Clinical Research Consortium
Peter A. Merkel, M.D., M.P.H. — Boston University School of Medicine
- 11:30 a.m. LUNCH (on your own) and Poster Session**
- 1:00 p.m. Strategies in Forming Effective Teams or Networks Panel**
Moderator: *Alan K. Percy, M.D.* — University of Alabama at Birmingham
John W. Walsh — Alpha-1 Foundation
Robert D. Steiner, M.D. — Oregon Health & Science University
Barbara E. Herr — University of Rochester Medical Center
Discussion Question: What are barriers and facilitators to effective rare disease research?
- 1:45 p.m. Sharing Across Diseases Panel**
Moderator: *Peter A. Merkel, M.D., M.P.H.* — Boston University School of Medicine
Avital Cnaan, Ph.D. — Children’s National Medical Center
Patrick F. Terry, GRAND Therapeutics Foundation and GRAND Rx Collaborative
Ronald J. Sokol, M.D. The Children’s Hospital and University of Colorado Denver School of Medicine
Discussion Question: How do rare disease researchers share best practices?
- 2:30 p.m. BREAK**
- 2:45 p.m. Basic Research to Clinical Research Translation Panel**
Moderators: *Donna J. McCloskey, Ph.D.* — National Center for Research Resources, NIH and *W. Andrew Faucett, M.S., C.G.C.* — Emory University
Joel Moss, M.D., Ph.D. — National Heart, Lung and Blood Institute, NIH
Christopher P. Austin, M.D. — National Human Genome Research Institute, NIH
Discussion Question: What are barriers or facilitators to communication and collaboration between basic and clinical researchers?
- 3:30 p.m. Clinical Research to Clinical Practice Translation Panel**
Moderator: *Jeffrey P. Krischer, Ph.D.* — University of South Florida
Mark Batshaw, M.D. — Children’s National Medical Center
Leslie B. Gordon, M.D., Ph.D. — Progeria Research Foundation / Alpert School of Medicine, Brown University
Chris Garabedian, Celgene Corporation
Discussion Question: How can we best disseminate rare disease advances, standards of care, and diagnostics to clinicians, practitioners, and patients?
- 4:15 p.m. Wrap Up**
Alan E. Guttmacher, M.D. — National Human Genome Research Institute, NIH
- 4:30 p.m. Adjourn**
Stephen C. Groft, Pharm.D. — Office of Rare Diseases Research, NIH