

ADVANCING RARE DISEASES RESEARCH THROUGH NETWORKS AND COLLABORATION

July 16, 2009

NIH Natcher Conference Center • Bethesda, MD

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. Graft, Pharm.D. - Office of Rare Diseases Research, NIH
Barbara Alving, M.D. - National Center for Research Resources, NIH

8:15 a.m. Keynote
Francis Collins, M.D., Ph.D. - Former Director, National Human Genome Research Institute, NIH (Invited)

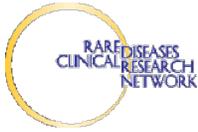
9:00 a.m. Challenges and Opportunities in Rare Disease Research
Robert C. Griggs, M.D. - University of Rochester Medical Center

9:20 a.m. Training Professionals in Rare Disease Research Panel
Moderator: *Michael 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

10:00 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
Peter Merkel, M.D., Ph.D. - Boston University School of Medicine

11:30 a.m. LUNCH (ON YOUR OWN) AND POSTER SESSION



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- 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 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 Merkel, M.D., Ph.D.* - Boston University School of Medicine
Avital Cnaan, M.D. - Children's Hospital of Philadelphia
Peter L. Saltonstall - National Organization for Rare Disorders
Ronald J. Sokol, M.D. - 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: *Rashmi Gopal-Srivastava, Ph.D.* - Office of Rare Diseases Research, NIH
Donna J. McCloskey, Ph.D. - National Center for Research Resources, NIH
W. Andrew Faucett, M.S., C.G.C. - Emory University
Joel Moss, M.D. - National Heart, Lung and Blood Institute, NIH
Christopher 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 / Hasbro Children's Hospital
- 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