



RARE DISEASE DAY at NIH

Feb. 29, 2016 | National Institutes of Health, Bethesda, MD

#RDDNIH

AGENDA

7:30 a.m. Registration and Poster/Exhibit Booth Setup

8:30 a.m. First Morning Session

NIH Director Remarks

Francis S. Collins, M.D., Ph.D. — Director, National Institutes of Health (NIH)

NIH Clinical Center (CC) Remarks

John I. Gallin, M.D. — Director, CC, NIH

Congressional Remarks

Video Remarks from Senator Orrin Hatch (R-UT) — Co-Chair, Rare Disease Congressional Caucus

National Center for Advancing Translational Sciences (NCATS) Director Remarks

Christopher P. Austin, M.D. — Director, NCATS, NIH

Office of Rare Diseases Research (ORDR) Director Remarks

Petra Kaufmann, M.D., M.Sc., FAAN — Director, ORDR, NCATS, NIH

National Organization for Rare Disorders (NORD) Updates

Martha L. Rinker, J.D. — Vice President of Public Policy, NORD

10:00 a.m. Break

10:20 a.m. Second Morning Session

International Collaboration

The 2014–15 Ebola Crisis: The NIH Response

Richard T. Davey, Jr., M.D. — Deputy Clinical Director, National Institute of Allergy and Infectious Diseases, NIH

International Classification of Diseases, 11th revision (ICD-11)

Donna Pickett, M.P.H., R.H.I.A. — Chief, Classifications and Public Health Data Standards, National Center for Health Statistics, Centers for Disease Control and Prevention

B2B Update: Telomeres and Lifestyle in Li-Fraumeni Syndrome Outcomes

Sharon A. Savage, M.D., FAAP — Chief, Clinical Genetics Branch, Division of Cancer Epidemiology and Genetics, National Cancer Institute (NCI), NIH

Undiagnosed Diseases Network (UDN) Update and International Collaborations

Anastasia L. Wise, Ph.D. — Program Director, Division of Genomic Medicine, National Human Genome Research Institute (NHGRI), NIH

William A. Gahl, M.D., Ph.D. — Clinical Director, NHGRI, NIH

Food and Drug Administration (FDA)

FDA Updates

Gayatri Rao, M.D., J.D. — Director, Office of Orphan Products Development, FDA

Jonathan Goldsmith, M.D. — Associate Director, Rare Diseases Program, Office of New Drugs, Center for Drug Evaluation and Research, FDA

12:00 p.m. **Lunch and Poster Session/CC Tours**

1:30 p.m. Afternoon Session

Congressional Remarks

Congressman Leonard Lance (R-NJ 7th District) — Co-Chair, Rare Disease Congressional Caucus

Patient's Voice

Session Moderator: Commander Margaret Bevans, Ph.D., R.N., AOCN®, FAAN — United States Public Health Service, Clinical Nurse Scientist and Program Director, Scientific Resources, CC, NIH

Genetic Alliance Updates

Sharon Terry, M.A. — President and CEO, Genetic Alliance

Using Public Policy to Accelerate Biomedical Innovation for Rare Disease Patients

Max G. Bronstein, M.P.P. — Senior Director, Advocacy and Science Policy, EveryLife Foundation for Rare Diseases

Finding the Help You Need

Suzanne P. Ripley, Ph.D.

Global Genes™ Updates

Kym Kilbourne — Vice President, Patient Advocacy, Global Genes™

NIH CC Patient Advisory Group (PAG)

Beatrice A. Bowie — Facilitator, Sickle Cell Support Group, Adventist HealthCare Shady Grove Medical Center, and Board Member, NIH CC PAG

Uplifting Athletes Update

Scott Shirley — Executive Director, Uplifting Athletes

The Mighty

Mike Porath — Founder and CEO, The Mighty

Caregiver Research: Understanding the Perceived Influence of Childhood Cancer on the Parents' Relationship

Lori Wiener, Ph.D. — Co-Director, Behavioral Health Core, Pediatric Oncology Branch, Center for Cancer Research, NCI, NIH

Session Wrap-up

Commander Margaret Bevans, Ph.D., R.N., AOCN®, FAAN

3:15 p.m. Wrap-Up

3:30 p.m. Adjourn