RARE DISEASE DAY at NIH
Feb. 27, 2017 | #RDDNIH

Final Agenda
As of Feb. 24, 2017

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

8:30 a.m. First Morning Session

Welcome
Christopher P. Austin, M.D., Director, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH); Chair, International Rare Diseases Research Consortium (IRDiRC)

NIH Clinical Center (CC) Leadership Remarks
James K. Gilman, M.D., Chief Executive Officer, CC, NIH
John I. Gallin, M.D., Associate Director for Clinical Research, NIH; Chief Scientific Officer, CC, NIH

NIH NCATS Director Remarks
Christopher P. Austin, M.D., Director, NCATS, NIH; Chair, IRDiRC

NIH NCATS Office of Rare Diseases Research (ORDR) Leadership Remarks
Petra Kaufmann, M.D., M.Sc., FAAN, Director, ORDR, NCATS, NIH
Anne R. Pariser, M.D., Deputy Director, ORDR, NCATS, NIH
Charles A. Mohan, Jr., Chair, Rare Diseases Clinical Research Network Coalition of Patient Advocacy Groups

Undiagnosed Diseases Network Update
Anastasia L. Wise, Ph.D., Program Director, National Human Genome Research Institute (NHGRI), NIH

National Organization for Rare Disorders (NORD) Update
Martha L. Rinker, J.D., Vice President of Public Policy, NORD

10:00 a.m. Break

10:20 a.m. Second Morning Session

Zika: A Pandemic in Progress
Anthony S. Fauci, M.D., Director, National Institute of Allergy and Infectious Diseases, NIH

Validating and Modeling Rare Human Diseases in Zebrafish Using CRISPR/Cas9
Shawn Burgess, Ph.D., Senior Investigator, Translational and Functional Genomics Branch, NHGRI, NIH

Giant Axonal Neuropathy: The Role of Natural History Studies in Clinical Trial Readiness for Rare Diseases
Diana X. Bharucha-Goebel, M.D., Assistant Professor of Neurology, Children's National Health System; Clinical Collaborator, Neuromuscular and Neurogenetic Disorders of Childhood Section, Neurogenetics Branch, National Institute of Neurological Disorders and Stroke, NIH

Albinism: Can It Become a Treatable Disease?
Brian P. Brooks, M.D., Ph.D., Principal Investigator, Unit on Pediatric, Developmental, and Genetic Ophthalmology, National Eye Institute, NIH; Adjunct Faculty, NHGRI, NIH

Precision Health for All: The *All of Us* Research Program
Stephanie Devaney, Ph.D., Deputy Director, *All of Us* Research Program, NIH

Food and Drug Administration (FDA) Update
Jonathan Goldsmith, M.D., FACP, Associate Director, Rare Diseases Program, Office of New Drugs, Center for Drug Evaluation and Research, FDA

Noon — Lunch and Poster Session/Clinical Center Tours

1:30 p.m. — Afternoon Session

EveryLife Foundation for Rare Diseases Update
Max G. Bronstein, M.P.P., Chief Advocacy & Science Policy Officer, EveryLife Foundation for Rare Diseases

Support for Patients as Partners Through the Drug Development Lifecycle
Wendy White, Chairman of the Board of Directors, Global Genes® — Allies in Rare Disease; Principal, Wendy White Consulting

The Children's Inn at NIH: A Rare Disease Patient Story
Jennie Lucca, CEO, The Children's Inn at NIH
Chris Petty, rare disease patient

2:15 p.m. — Break

FasterCures Update
Margaret Anderson, M.S., Executive Director, FasterCures

Uplifting Athletes: One Athlete's Rare Disease Story
Rob Long, Director of Strategic Development, Uplifting Athletes

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

Genetic Alliance Update
Sharon F. Terry, M.A., President and CEO, Genetic Alliance

Congressional Remarks
Rep. Leonard Lance, Co-Chair, Rare Disease Congressional Caucus

NIH Director Remarks
Francis S. Collins, M.D., Ph.D., Director, NIH

Closing Remarks
Christopher P. Austin, M.D., Director, NCATS, NIH; Chair, IRDiRC

4:00 p.m. Adjournment