



# Rare Disease Day

27 February 2015

Patients and Researchers, Partners for Life!

## Agenda

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

8:30 a.m. First Morning Session

- **Opening Remarks**  
John I. Gallin, M.D. – Director, Clinical Center, National Institutes of Health (NIH)
- **How The Children’s Inn Supports Rare Disease Research**  
Jennie Lucca – Chief Executive Officer, The Children’s Inn at NIH
- **Congressional Remarks**  
Congressman Leonard Lance – Representative (R – NJ 7<sup>th</sup> District)
- **New Treatment for a Rare Metabolic Disorder**  
William H. Theodore, M.D. – Chief, Clinical Epilepsy Section, National Institute of Neurological Disorders and Stroke (NINDS), NIH
- **Food and Drug Administration (FDA) Updates**  
Gayatri Rao, M.D., J.D. – Director, Office of Orphan Product Development, FDA
- **National Organization for Rare Disorders (NORD) Updates**  
Diane Dorman – Vice President, Public Policy, NORD

10:00 a.m. Morning Break

10:15 a.m. Second Morning Session

### Team and Community Building Discussion Panel

- **Academic Team Model**  
Maureen Hoatlin, Ph.D., M.B.A. – Associate Professor, Oregon Health & Science University
- **Patient Advocacy Group Team Model**  
Jill Wood – Co-Founder and Chief Financial Officer, Jonah’s Just Begun
- **Patient Advocacy Group Community Building Model**  
Matt Might, Ph.D. – President, NGLY1.org
- **Foundation-Directed Team Model**  
Barbara L. Handelin, Ph.D. – Chief Executive Officer, BioPontis Alliance

### Genetic Alliance Updates

James O’Leary, M.B.A. – Chief Innovation Officer, Genetic Alliance

12:00 p.m. Lunch and Poster Session / Clinical Center Tours



1:30 p.m. First Afternoon Session

**NIH Director Remarks**

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

**Technology Hand-Off Discussion Panel**

- **Technology Transfer at the National Center for Advancing Translational Sciences (NCATS)**  
Lili M. Portilla, M.P.A. – Director, Office of Strategic Alliances, NCATS
- **Experiences of an Inventor: From Intramural to Biotech**  
Robert Kotin, Ph.D. – Vice President of Production, Voyager Therapeutics, Inc.
- **Technology Transfer: A Success Story**  
Stephen Seiler – Founder, AesRx, LLC

**Global Genes Updates**

Barbara Lavery – Vice President of Strategic Alliances, Global Genes

**Uplifting Athletes Updates**

Scott Shirley – Executive Director, Uplifting Athletes

3:00 p.m. Afternoon break

3:15 p.m. Second afternoon session

**NCATS Director Remarks**

Christopher P. Austin, M.D. – Director, NCATS

**Rare Diseases Clinical Research Network (RDCRN)**

- **RDCRN Update**  
Rashmi Gopal-Srivastava, Ph.D. – Program Director, NCATS
- **RDCRN Patient Advocate**  
Charles A. Mohan, Jr. – Chair, Coalition of Patient Advocacy Groups
- **RDCRN Program Officer**  
Wendy R. Galpern, M.D., Ph.D. – Program Director, Clinical Trials, NINDS
- **RDCRN Principal Investigator**  
Peter A. Merkel, M.D, M.P.H. – Chief, Division of Rheumatology, University of Pennsylvania

4:45 p.m. Wrap Up

5:00 p.m. Adjourn