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 7th 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