Agenda

Day 1 — Thursday, February 28
Focus on Partner Agencies

8:30 a.m. **Introduction**
Stephen C. Groft, Pharm.D. — Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

8:35 a.m. **Welcoming Address**
Christopher P. Austin, M.D. — Director, NCATS, NIH

8:55 a.m. *Michael Astrue* — Former Commissioner, Social Security Administration

9:25 a.m. **Updates from the Office of Orphan Products Development**
Gayatri Rao, M.D., J.D. — Director, Office of Orphan Products Development (OOPD), Food and Drug Administration (FDA)

9:40 a.m. **FDA Center for Drug Evaluation and Research (CDER) Rare Disease Program: What's New 2012 & 2013**
Anne Pariser, M.D. — Associate Director for Rare Diseases, CDER, FDA

9:55 a.m. **Break**

10:10 a.m. **The Agency for Healthcare Research Quality (AHRQ) Registry of Patient Registries and Related Work**
Elise Berliner, Ph.D. — Director, Technology Assessment Program, AHRQ

10:30 a.m. **Updates on the Global Rare Disease Patient Registry Data Repository (GRDR)**
Yaffa Rubinstein, Ph.D. — Director of Patient Resources for Clinical and Translational Research, ORDR, NCATS, NIH

10:50 a.m. **Updates from the Patient-Centered Outcomes Research Institute (PCORI)**
Adaeze Akamigbo, Ph.D. — Senior Program Officer, PCORI

11:10 a.m. **Newborn Screening, What it is and the Promise Yet to Come**
Sara Copeland, M.D . — Chief, Genetics Services Branch, Division of Services for Children with Special Health Needs (DSCSHN), Maternal and Child Health Bureau (MCHB), Health Resources Service Administration (HRSA)

11:30 a.m. **Lunch and Poster Session**

12:45 p.m. **Breakout Session 1**
FDA — *Anne Pariser, M.D., Gayatri Rao, M.D., J.D.*
HRSA — *Sara Copeland, M.D.*
AHRQ — *Elise Berliner, Ph.D., Scott Smith, Ph.D.*
ORDR — *Stephen C. Groft, Pharm.D.*

1:45 p.m. **Breakout Session 2**
FDA
AHRQ
ORDR

2:45 p.m. **Break**

3:00 p.m. **Healthcare in the Media**
Susannah Cahalan, author — "Brain on Fire: My Month of Madness"
Jessica Wapner, author — "The Philadelphia Chromosome: A Mutant Gene and the Quest to Cure Cancer at the Genetic Level"
Hugh Hempel

4:00 p.m. **Screening of Documentary *Here. Us. Now.***

5:15 p.m. **Adjourn**

Day 2 — Friday, March 1
Focus on Partner NIH Institutes and Centers

8:30 a.m. **Introductions**
David J. Eckstein, Ph.D. — ORDR, NCATS, NIH

8:35 a.m. *John I. Gallin, M.D.* — Director, NIH Clinical Center

8:45 a.m. **NCATS and Department of Defense (DoD) Talks**

Organs on Chips for Drug Screening and Toxicity Assessment
Danilo Tagle, Ph.D. — Associate Director for Special Initiatives, NCATS, NIH

Microphysiological Systems for Drug Efficacy, Toxicity, and Molecular Mechanism
Barry Pallotta, Ph.D. — Program Manager, Defense Advanced Research Projects Agency (DARPA), Defense Sciences Office

Rare Disease Programs at Congressionally Directed Medical Research Programs (CDMRP)
Naba Bora, Ph.D., M.B.A. — Program Manager, CDMRP, U.S. Army Medical Research and Material Command

9:45 a.m. **Registries for All: Reg4All**
Sharon Terry — President & CEO, Genetic Alliance

10:05 a.m. **Legislative Priorities During the 30th Anniversary Year**
Diane Dorman — Vice President of Public Policy, National Organization of Rare Disorders (NORD)

10:20 a.m. **Break**

10:35 a.m. **Rare Diseases and PROMIS: Opportunities**
James Witter, M.D., Ph.D. — Medical Officer, Rheumatic Diseases Clinical Program, National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)

10:55 a.m. **The Developing Evidence to Inform Decisions of Effectiveness (DEcIDE) Program**
Scott R. Smith, Ph.D. — Program Director, Center for Outcomes & Evidence, AHRQ

11:15 a.m. **Updates from the Division of Pre-Clinical Innovation (DPI)**
John McKew, Ph.D. — Acting Director, DPI, NCATS

11:35 a.m. **The Genetic and Rare Diseases Information Center (GARD): Improving Access to Hard-to-Find Genetic and Rare Diseases Information and Resources**
Janine Lewis, M.S., C.G.C. — Senior Project Manager, Genetic and Rare Diseases Information Center (GARD)
Michelle Snyder, M.S., C.G.C — Operations Manager, GARD

11:50 a.m. **Lunch and Poster Session**

1:00 p.m. **Breakout Session 1 — Institutes and Centers (ICs) (with Clinical Center [CC] tour option)**

NCATS
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)
National Heart, Lung, and Blood Institute (NHLBI)
National Cancer Institute (NCI)
The Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)
CC
National Institute of Neurological Disorders and Stroke (NINDS)
National Human Genome Research Institute (NHGRI)
Office of Dietary Supplements (ODS)

2:00 p.m. **Breakout Session 2 — ICs (with CC tour option)**

NCATS
NIDDK
NHLBI
NCI
NICHD
CC
NINDS
NHGRI
National Eye Institute (NEI)

3:00 p.m. **Wrap Up**

3:30 p.m. **Adjourn**