AGENDA — DAY 1
Monday, October 18

12:00 p.m.  Registration

1:00 p.m.  Welcoming Remarks
Janet Woodcock, M.D. — Director, Center for Drug Evaluation and Research (CDER), Food and Drug Administration (FDA)

1:30 p.m.  Session 1 — Introduction to Orphan Drug Development
Session Chair: Stephen C. Groft, Pharm.D. — Office of Rare Diseases Research (ORDR), National Institutes of Health (NIH)

1.  **Historical perspective of orphan drug development – the first 25 years (1:30 p.m. – 2:00 p.m.)**
   Marlene Haffner, M.D., M.P.H. — Haffner Associates

2.  **Orphan Drug Act: The next 25 years – challenges and mandate for the future (2:00 p.m. – 2:30 p.m.)**
   Timothy Côté, M.D., M.P.H. — Office of Orphan Products Development, FDA

3.  **Venture capital community perspective on orphan drugs (2:30 p.m. – 3:00 p.m.)**
   Patrick Lee, M.D., M.B.A — Palo Alto Investors

3:00 p.m.  Break (15 minutes)

3:15 p.m.  Session 2 — Regulatory Overview: Entering Clinical Trials – From Bench to Bedside
Session Chair: David Jacobson-Kram, Ph.D. — Office of New Drugs, CDER, FDA

1.  **Demystifying the process and interacting with FDA (3:15 p.m. – 3:45 p.m.)**
   LT Richard (Wes) Ishihara — Office of New Drugs, CDER, FDA

2.  **Chemistry manufacturing and controls: Recommendations to enable first-in-human investigational new drug (IND) applications – drugs and biological products (3:45 p.m. – 4:30 p.m.)**
   Gibbes Johnson, Ph.D. — Office of Biotechnology Products, CDER, FDA
   Rapti Madurawe — Office of New Drug Quality Assessment, CDER, FDA
3. Nonclinical IND requirements to enable first-in-human rare disease trials (4:30 p.m. – 5:15 p.m.)
   Sushanta Chakder, Ph.D. — Office of New Drugs, CDER, FDA
   Lois Freed, Ph.D — Office of New Drugs, CDER, FDA

4. Special considerations for cellular and genetic therapies and blood-derived products (5:15 p.m. – 6:00 p.m.)
   Steven Winitsky, M.D. — Office of Cellular, Tissue, and Gene Therapies: Center for Biologics Evaluation and Research (CBER), FDA
   Nisha Jain, M.D. — Office of Blood Research and Review, CBER, FDA

6:00 p.m.  Adjourn
AGENDA — DAY 2
Tuesday, October 19

8:00 a.m.  Registration and Continental Breakfast

8:30 a.m.  Session 3 — Considerations for Clinical Trial Designs to Support Marketing Applications
Session Chair: Anne Pariser, M.D. — Office of New Drugs, CDER, FDA

1. Standards for clinical trials to support marketing applications (8:30 a.m. – 9:00 a.m.)
   John Jenkins, M.D. — Office of New Drugs, CDER, FDA

2. Implementing trials in rare diseases (9:00 a.m. – 9:30 a.m.)
   Petra Kaufman, M.D. — National Institute of Neurological Disorders and Stroke (NINDS), NIH

3. Clinical trial endpoints: Development and validation of measures to support claims in labeling (9:30 a.m. – 10:00 a.m.)
   CAPT Laurie Burke, R.Ph., M.P.H. — Office of New Drugs, CDER, FDA

10:00 a.m.  Break (30 minutes)

10:30 a.m.  Session 4 — IND Drug Development: Biomarkers – Strategies and Approaches to Rare Disease Drug Development
Session Chair: Dan Tagle, Ph.D. — NINDS, NIH

1. Use of biomarkers and surrogates in clinical trials – FDA perspective (10:30 a.m. – 11:00 a.m.)
   Marc Walton, M.D., Ph.D. — Office of Translational Sciences, CDER, FDA

2. Biomarker use in clinical trials – industry perspective (11:00 a.m. – 11:30 a.m.)
   Joan Keutzer, Ph.D. — Genzyme Corporation

3. Biomarker and assay development (11:30 a.m. – 12:00 p.m.)
   David Millington, Ph.D. — Duke University Medical Center
4. **Drug discovery (12:00 p.m. – 12:30 p.m.)**  
*Christopher Austin, M.D.* — National Human Genome Research Institute, NIH

12:30 p.m. **Working Lunch**  
**Academic perspective on the development of orphan products**  
*Robert Califf, M.D.* — Duke University Medical Center

1:30 p.m. **Session 5 — Biomarker Standardization and Assay and Device Codevelopment**  
Session Chair: *Stephen C. Groft, Pharm.D.* — ORDR, NIH

1. **Standardization for data and reference sets (1:30 p.m. – 2:00 p.m.)**  
*James Osborne, Ph.D.* — Keck Graduate Institute

2. **Device and assay codevelopment (2:00 p.m. – 2:30 p.m.)**  
*Sugato De, M.S.* — Office of Device Evaluation, Center for Devices and Radiological Health, FDA

2:30 p.m. **Break (30 minutes)**

3:00 p.m. **Session 6 — Clinical Trial Statistics**  
Session Chair: *Stephen Wilson, Dr.P.H.* — Office of Biostatistics, CDER, FDA

1. **Level of evidence to support marketing applications (3:00 p.m. – 3:30 p.m.)**  
*Lisa Kammerman, Ph.D.* — Office of Biostatistics, CDER, FDA

2. **Statistical overview for clinical trials (3:30 p.m. – 4:00 p.m.)**  
*Behrang Vali, M.S.* — Office of Biostatistics, CDER, FDA

3. **Adaptive designs and small clinical trials (4:00 p.m. – 4:30 p.m.)**  
*Christopher Coffey, Ph.D.* — University of Iowa

4. **Challenges in rare disease studies (4:30 p.m. – 5:00 p.m.)**  
*Gary Cutter, Ph.D.* — University of Alabama

5:00 p.m. **Adjourn**
AGENDA — DAY 3
Wednesday, October 20

8:00 a.m.  Registration and Continental Breakfast

8:30 a.m.  Session 7 — Case Presentations
Session Chair: Russell Katz, M.D. — Office of New Drugs, CDER, FDA

1. **Introductory remarks: Considerations when assessing marketing applications for orphan products (8:30 a.m. – 9:00 a.m.)**
   Russell Katz, M.D. — Office of New Drugs, CDER, FDA

2. **Pompe disease – the Myozyme story (9:00 a.m. – 9:30 a.m.)**
   Priya Kishnani, M.D. — Duke University Medical Center

3. **Multiple sclerosis – the dalfampridine story (9:30 a.m. – 10:00 a.m.)**
   Eric Bastings, M.D. — Office of New Drugs, CDER, FDA

4. **Hematology case study (10:00 a.m. – 10:30 a.m.)**
   Albert Deisseroth, M.D. — Office of New Drugs, CDER, FDA

5. **Panel discussion (10:30 a.m. – 11:00 a.m.)**
   Drs. Katz, Bastings, Kishnani, and Deisseroth

11:00 a.m.  Break (30 minutes)

11:30 a.m.  Session 8 — Orphan Drug Law and Regulatory Initiatives
Session Chair: Peter Saltonstall — National Organization of Rare Disorders

1. **Orphan drug regulatory initiatives**
   Frank Sasinowski, J.D. — Hyman Phelps & McNamara

2. **Orphan drug legislation**
   Paul Kim, J.D., M.P.P. — Foley Hoag LLP

3. **Panel discussion**
   Mssrs. Saltonstall, Sasinowski, and Kim

12:30 p.m.  Adjourn