

Accelerating Therapies FOR RARE DISEASES WORKSHOP

October 18–20, 2010
Rockville, MD
USP Building

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

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

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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

