



# FDA Rare Disease Patient Advocacy Day

## March 1, 2012

White Oak Campus • Building 31 • Great Room • 10903 New Hampshire Ave. • Silver Spring, MD 20993

### **PLENARY SESSION (interactive webcast)**

- 8:30-8:40 AM Introductions and Opening Remarks – Gayatri Rao, MD, JD
- 8:40-8:50 AM Patient Representative Perspective – Tiffany House
- 8:50-9:15 AM Office of Orphan Products Development (OOPD)  
*Presenter:* Gayatri Rao, MD, JD  
*Topic:* Overview of the Food and Drug Administration (FDA) & the Office of Orphan Products Development (OOPD)
- 9:15-9:30 AM Center for Drug Evaluation and Research (CDER)  
*Presenter:* Anne Pariser, MD  
*Topic:* Rare Diseases Drug Development
- 9:30-9:45 AM FDA Commissioner Margaret Hamburg, MD
- 9:45-10:00 AM Center for Biologics Evaluation and Research (CBER)  
*Presenter:* Nisha Jain, MD  
*Topic:* CBER Perspective on Products for Rare Diseases
- 10:00-10:15 AM Center for Devices and Radiological Health (CDRH)  
*Presenter:* Markham Luke, MD, PhD  
*Topic:* Rare Diseases from a Medical Device Perspective
- 10:15-10:35 AM Panel Discussion  
*On-site and webcast participants to present questions to panel members concerning above presentations*
- 10:35-10:55 AM Break (snacks to be offered)
- 10:55-11:10 AM CDER Drug Shortage Program  
*Presenter:* CDR Emily Thakur, RPh  
*Topic:* Introduction to U.S. Drug Shortages
- 11:10-11:25 AM Office of Special Health Issues (OSHI)  
*Presenter:* Richard Klein  
*Topic:* Patient Advocacy
- 11:25-11:40 AM National Institutes of Health (NIH)  
*Presenter:* Steve Groft, PharmD  
*Topic:* NIH Rare Diseases Research
- 11:40 AM-12:00 PM Panel Discussion  
*On-site and webcast participants to present questions to panel members concerning above presentations*

**ROUNDTABLE LUNCH SESSION (not webcasted)**

12:00-1:30 PM Roundtable Lunch Session  
*Informal setting to allow audience members to have lunch with and to engage with a variety of designated experts both within and outside of the FDA*

**KEYNOTE ADDRESS (non-interactive webcast)**

1:30-2:00 PM Delivered by Stephen Spielberg, MD, PhD  
Deputy Commissioner for Medical Products and Tobacco

2:00-2:30 PM Break (snacks to be offered)

**BREAKOUT SESSIONS (not webcasted)**

*The format may consist of a short presentation followed by an open discussion*

2:30-3:15 PM Breakout Sessions

<i>Presenter</i>	<i>Topic</i>
OOPD	Grants Program
CDER	Regulatory Science
CDRH	Devices, Diagnostics, Humanitarian Use Devices (HUDs)
OSHI	Expanded Access, Advocate Training Program
NORD	Current Patient Advocacy Initiatives & NORD Analysis of Effectiveness Evidence in Orphan Drugs

3:15-3:25 PM Break (snacks to be offered)

3:25-4:10 PM Breakout Sessions

<i>Presenter</i>	<i>Topic</i>
OOPD	Designation Program
NIH	NIH and Rare Diseases
CDER	Drug Shortage Program
Center for Food Safety and Applied Nutrition (CFSAN)	Regulation of Medical Foods
Genetic Alliance	Advocates and FDA – Working Together

4:10-4:30 PM Break (snacks to be offered)

**SENIOR TOWNHALL (interactive webcast) and CLOSING REMARKS**

4:30-5:00 PM Senior Townhall Panel/Closing Remarks  
*On-site and webcast participants to present questions to panel members during Senior Townhall session*

