

Rare Diseases Forum Workshop 1

Innovation in Trial Designs: Bringing Lessons from the Oncology Experience to Rare Diseases

Monday September 23, 2019
National Press Club, Washington DC

8:00 AM	Registration & Light Breakfast	
8:30 AM	Session I: Introduction & Project Overview	
	Moderator: <i>Veronica Miller, Forum for Collaborative Research</i>	
8:30 AM	Welcoming Remarks & Introductions	<i>Veronica Miller, Forum for Collaborative Research</i> <i>Marshall L. Summar, Academic Co-Chair</i> <i>John F. Crowley, Industry Co-Chair</i> <i>Violeta Stoyanova, European Medicines Agency</i>
8:45 AM	Session II: Lessons from Oncology Master Protocols	
	Moderator: <i>Veronica Miller, Forum for Collaborative Research</i>	
8:45 AM	Panel 1: Platform Trials A. BeatAML B. Pediatric MATCH	Panelists: <i>Amy Burd, Leukemia Lymphoma Society</i> <i>Nita Seibel, NCI,NIH</i> <i>Kathleen Neville, Johnson and Johnson</i> <i>Gwen Nichols, Leukemia Lymphoma Society</i> <i>Gregory Reaman, CDER, FDA</i> <i>Peter Bross, CBER, FDA</i>
10:15 AM	Break	
10:30 AM	Perspectives from FDA Leadership	<i>Janet Woodcock, CDER, FDA</i>
	Moderator: <i>P.J. Brooks, NCATS, NIH</i>	
11:00 AM	Panel 2: Basket Trials	Panelists: <i>Julia Glade Bender, Memorial Sloan Kettering Center</i> <i>David Pearce, Sanford Health</i> <i>Kelley Kidwell, University of Michigan</i> <i>Larissa Higgins, Health Products Regulatory Authority</i> <i>Adnan Jaigirdar, CBER, FDA</i> <i>Jingjing Ye, CDER, FDA</i>
12:30 PM	Lunch	

1:30 PM Session III: Specific Topics Panels

Moderator: *Donna Griebel, Griebel Consulting*

1:30 PM	Panel 3: A. Data Quality in Outcome Assessments	Panelists: <i>Brad Pollock, UC Davis</i> <i>Abby Bronson, Parent Project Muscular Dystrophy</i> <i>Mary Lin, CBER, FDA</i> <i>Rajeshwari Sridhara, CDER, FDA</i> <i>Laura Lee Johnson, CDER, FDA</i>
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Moderator: *Sandi Lehrman, Patient Advocate*

2:30 PM	Panel 4: A. Challenges in Inclusion of Younger Children, Diagnostics and Follow-up Biomarkers	Panelists: <i>Abby Sandler, NCI, NIH</i> <i>Andrew Mulberg, Amicus Therapeutics</i> <i>Janki Veeraraghavan, CDRH, FDA</i> <i>Dina Zand, CDER, FDA</i>
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3:30 PM	Discussion	<i>All</i>
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4:00 PM Break

4:15 PM Session IV: Putting It All Together

Moderators: *Veronica Miller, Forum for Collaborative Research & John F. Crowley, Amicus Therapeutics*

4:15 PM	A. Which Questions Do We Need to Address? B. Working Group Proposals/ Leads C. Meeting Summary and Next Steps
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5:00 PM Networking Reception

7:00 PM **Adjourn**