Rare Diseases Forum 1

Wednesday, October 17, 2018
Carnegie Endowment for International Peace
8:00 AM - 7:00 PM

DRAFT AGENDA

8:00 AM	**Registration & Breakfast**	
8:30 AM	**Session 1: Introductions & Project Overview**	**Moderator:** Veronica Miller, *Forum for Collaborative Research*
	Welcoming Remarks & Introductions	**Presenters:** Veronica Miller, *Forum for Collaborative Research* Marshall Summar, *Academic Co-Chair* John Crowley, *Industry Co-Chair* Janet Woodcock, *FDA/ CDER* Frank Sasinowski, *Hyman, Phelps & McNamara, P.C.* Dragos Roman, *FDA/ CDER/ DGIEP*
9:20 AM	**Session 2: Regulatory Considerations**	**Moderator:** Frank Sasinowski, *Hyman, Phelps & McNamara, P.C.*
		Presenters: Erica Lyons, *FDA/ CDER/ DGIEP* Rachel Witten, *FDA/ CBER/ OTAT*
9:50 AM	**Break**	
10:05 AM	**Session 3: What Has Worked, What Has Not?**	**Moderator:** Marshall Summar, *Children's National Health System*
	Case Study 1: Palynziq	**Presenters:** Holly Weng, *BioMarin Pharmaceutical* Patroula Smpokou, *FDA/ CDER/ DGIEP*
	Case Study 2: Brineura	David Jacoby, *BioMarin Pharmaceutical* Elizabeth Hart, *FDA/ CDER/ DGIEP*
	Case Study 3: Mepsevii	Qais Abu Ali, *Ultragenyx* Dina Zand, *FDA/ CDER/ DGIEP*
	Panel Discussion on Case Studies	**Discussants:** Margie Frazier, *Patient Representative* Chester Whitley, *University of Minnesota* Jeffrey D Marrazzo, *Spark Therapeutics* Ken Mills, *Regenxbio, Inc.*
12:05 PM	**Networking Lunch**	
12:50 PM	**Session 4: Identifying Gaps & Needs Panel**	**Moderator:** Scott Steele, *University of Rochester Medical Center*
		Panelists: Caroline Loewy, *Patient Advocate* John F Crowley, *Amicus Therapeutics* Ilan Ganot, *Solid Biosciences* Philip John Brooks, *NIH/ NCATS* Dragos Roman, *FDA/ CDER/ DGIEP* Barry Byrne, *University of Florida*
1:50 PM	**Session 5: Overcoming Barriers/ Practical Approaches**	**Moderator:** John F. Crowley, *Amicus Therapeutics*
	Ensuring Regulatory Flexibility: Need for Quality Data	**Presenters:** Kathleen Donohue, *FDA/ DGIEP*
	Ethical Considerations for Pediatric Studies in Rare Diseases	Donna Snyder, *FDA/ OC / OPT*

Rare Diseases Forum 1

Wednesday, October 17, 2018
Carnegie Endowment for International Peace
8:00 AM - 7:00 PM

DRAFT AGENDA, CONTINUED

2:20 PM	**Break**	
2:30 PM	Patient Focused Drug Development, Direct Benefit for Pediatrics, and 21st Century Cures Implementation	**Panelists:** Dina Zand, *FDA/ DGIEP* Pam Gavin, *NORD* Kristin Stephenson, *Muscular Dystrophy Association* Josh Lehrer, *Global Blood Therapeutic* s Matt Wilsey, *Grace Science Foundation*
3:30 PM	**Session 6: Putting it All Together**	**Moderators**: Veronica Miller, *Forum for Collaborative Research* Marshall Summar, *Children's National Health System* John Crowley, *Amicus Therapeutics* Frank Sasinowski, *Hyman, Phelps & McNamara, P.C.*
3:30 PM	Quick Summary	**Rapporteur:** *Sandra Lehrman, Patient Advocate*
3:40 PM	Working Group Proposals & Mandates	All
4:40 PM	Summary & Next Steps	Veronica Miller, *Forum for Collaborative Research*
5:00 PM	**Networking Reception**	
7:00 PM	**Adjourn**	