U.S. FOOD & DRUG ADMINISTRATION

FDA White Oak Campus
Building 31 Great Room
Silver Spring, MD

AGENDA

8:00 – 9:00 a.m.	Registration
9:00 – 9:30 a.m.	Welcome and Introductions
9:00 – 9:10 a.m.	**Welcome and Introductions:** *Francis Kalush, Ph.D.*
9:10 – 9:20 a.m.	**Global Genes Introduction:** *Meredith Cagle, M.P.H.*
9:20 – 9:30 a.m.	**FDA Opening Remarks:** *Douglas Throckmorton, M.D.*
9:30 – 10:25 a.m.	What Is the FDA and Who Is Involved with Rare Diseases Engagement?
	Moderator: *Francis Kalush, Ph.D.*
9:30 – 9:40 a.m.	**Introduction to FDA:** Heidi Marchand, Pharm. D.
9:40 – 9:50 a.m.	**FDA Orphan Medical Product Designation Program:** *Gayatri Rao, M.D., J.D.*
9:50 – 10:00 a.m.	**CDER Divisions Working with Rare Diseases:** *Jonathan Goldsmith, M.D.*
10:00 – 10:10 a.m.	**Professional Affairs and Stakeholder Engagement within CDER:** *John Whyte, M.D., M.P.H.*
10:10 – 10:25 a.m.	**Q & A**
10:25 – 10:45 a.m.	Break
10:45am–12:00pm	Types of Patient Engagement with CDER at FDA
	Moderator: *Francis Kalush, Ph.D.*
10:45 – 11:00 a.m.	**Overview of CDER Patient Engagement and Interactions:** *Douglas Throckmorton, M.D.*
11:00 – 11:15 a.m.	**Externally-led Patient-Focused Drug Developed Meetings:** *Meghana Chalasani*

11:15 – 11:35 a.m.	**CureSMA Early Engagement and PFDD Meeting with FDA:** *Rosangel Cruz, M.A.*
11:35 – 11:45 a.m.	**Experience with Patient Engagement in Neurology:** *Billy Dunn, M.D.*
11:45 – 12:00 p.m.	***Q & A***

12:00 – 1:00 p.m. Lunch

1:00 – 2:15 p.m. Case Studies: The Importance of Historical Controls Patient Data and Regulatory Flexibility When Engaging with CDER

Moderator: *Francis Kalush, Ph.D.*

1:00 – 1:20 p.m.	**Case Study 1 – TSAlliance:** *Steve Roberds, Ph.D.*
1:20 – 1:40 p.m.	**Case Study 2- Amyloidosis Research Consortium:** *Isabelle Lousada*
1:40 – 2:00 p.m.	**External Controls Patient Data and CDER Flexibility for Rare Disease Drug Approval:** *Dragos Roman, M.D.*
2:00 – 2:15 p.m.	**Importance of Controlled Trials and Natural History Studies – Bridging the Gap Between Impressions and Data:** *Henrietta Hyatt-Knorr, M.A.*

2:15 – 2:30 p.m. Break

2:30 – 3:00 p.m. So, You Want to Meet with CDER? Developing an Effective Engagement Strategy

Moderator: *Kendall Davis, M.P.H.*

2:30 p.m. – 2:45 p.m.	**CDER Expert Perspective – Best Practices:** *Laurie Muldowney, M.D.*
2:45 p.m. – 3:00 p.m.	**Patient Advocate Perspective – Best Practices:** *James Valentine, J.D., M.P.H.*

3:00 – 3:45 p.m. Panel Discussion: Determining Your Next Steps

Moderator: *Meredith Cagle, M.P.H.*

Panelists:

Jonathan Goldsmith, M.D.	*John Whyte, M.D., M.P.H.*
Billy Dunn, M.D.	*Rosangel Cruz, M.A.*
Isabelle Lousada	*James Valentine, J.D., M.P.H.*
Steve Roberds, Ph.D.	*Henrietta Hyatt-Knorr, M.A.*

3:45 – 4:00 p.m. Closing Remarks

Meredith Cagle, M.P.H.
Francis Kalush, Ph.D.

Speaker Information

Meredith Cagle, M.P.H.
Patient Engagement Director, Global Genes

Meghana Chalasani
Operations Research Analyst, Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER)

Rosangel Cruz, M.A.
Associate Research Director, CureSMA

Kendall Davis, M.P.H.
Sr. Manager, Strategic Alliances, Global Genes

Billy Dunn, M.D.
Director, Division of Neurology Products (DPP), CDER

Jonathan Goldsmith, M.D.
Associate Director, Rare Diseases Program, CDER

Henrietta Hyatt-Knorr, M.A.
Senior Program Policy Analyst, Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

Francis Kalush, Ph.D.
Health Programs Coordinator, Professional Affairs and Stakeholder Engagement (PASE), CDER

Isabelle Lousada
President and CEO, Amyloidosis Research Consortium

Heidi Marchand, Pharm.D.
Assistant Commissioner, Office of Health and Constituents Affairs (OHCA)

Laurie Muldowney, M.D.
Associate Director for Medical Policy, Office of Translational Sciences (OTS), CDER

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Chief Scientific Officer, TS Alliance

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