



RARE PATIENT ADVOCACY SYMPOSIUM

A partnership of
Penn Medicine Orphan Disease Center
and Global Genes



Sheraton University
City Hotel

Saturday, May 19, 2018

7:00 am - 4:30 pm

Who's Who at Regulatory Agencies



Andrea Furia-Helms, MPH

Acting Director, Patient Affairs Staff, Office of Medical Products and Tobacco, Office of Commissioner

U.S. Food and Drug Administration



Megan Lenz

Director of Marketing and Communications

Cure SMA

#PennMedMDBR2018

#GGPennRareSymposium



Who's Who at Regulatory Agencies



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*Acting Director, Patient Affairs Staff,
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Enhancing Patient Engagement at FDA

Andrea Furia-Helms, MPH

Director, Patient Affairs Staff
Office of Medical Programs and Tobacco
Office of the Commissioner

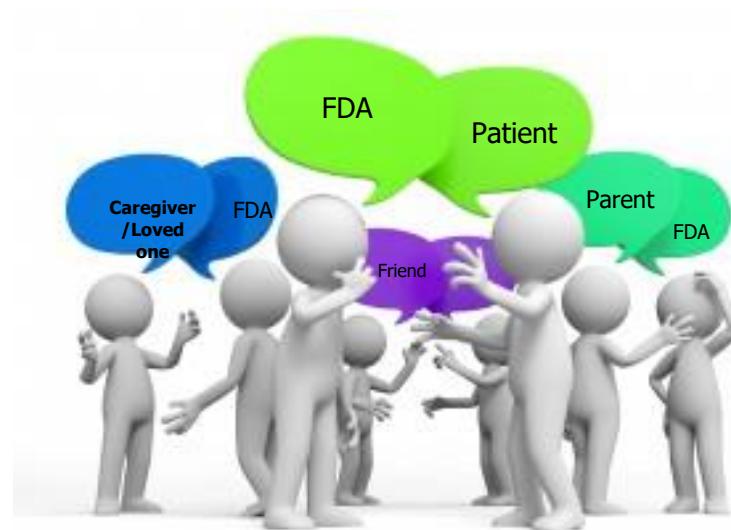
Rare Patient Advocacy Symposium
Penn Medicine Orphan Disease Center and Global Genes
May 19, 2018

Why is the Patient Voice Important?

- 👉 Provide insight on issues, problems, and/or questions that are important to patients and family members
- 👉 Patients have a vested interest diversity of opinions
- 👉 Varied perspectives, both in terms of risk tolerance and potential benefit
- 👉 The human element (judgment vs. empirical data)

Ultimately, patients are the focus of all of FDA's activities





Engage Listen Advocate

Early and iterative engagement can improve clinical and regulatory understanding of diseases and conditions, provide a common understanding of the most urgent patient needs, and inform drug development programs.

Commissioner Scott Gottlieb, M.D.
FDA Voice, February 26, 2018

Patient Affairs Staff (PAS)

Office of the Commissioner

- Established late 2017
- Works closely with the medical product centers and other offices to support and complement patient engagement efforts
- Reports into the Principal Deputy Commissioner for Medical Products and Tobacco

Patient Engagement Collaborative (PEC)



The FDA and the Clinical Trials Transformation Initiative (CTTI) are establishing an external group of patient organization and individual representatives to discuss topics about enhancing patient engagement in medical product development and regulatory discussions at FDA.

Patient Experience Listening Sessions

Rare Diseases Pilot

- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- To enhance the incorporation of patient experience information into regulatory discussions
- Inform FDA review division staff what is important to patient communities (e.g., disease burden, risk tolerance, impacts on daily activities and QOL)
- Assess value added to possibly expand

FDA Patient Representative Program

Office of the Commissioner

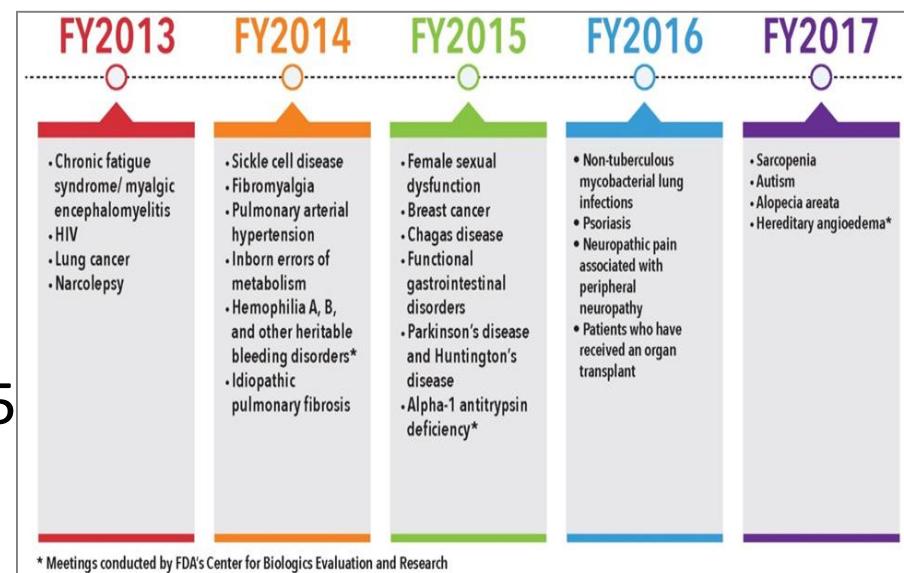
- Began in 1990s
- Patient community voice represented in regulatory discussions
- Special Government Employees (SGEs)
 - Serve in **review division consultation** meetings and on Advisory Committees
- Access to confidential background information
- Over 200 patients and caregivers, representing over 300 diseases and conditions



Patient Focused Drug Development

Center for Drug Evaluation and Research (CDER)/
Center for Biologics Evaluation and Research (CBER)

- A systematic of gathering patient perspectives on their condition to strengthen understanding of disease and treatment burden
- 24 disease specific meetings over 5 years



Externally-led Patient Focused Drug Development

CDER/CBER

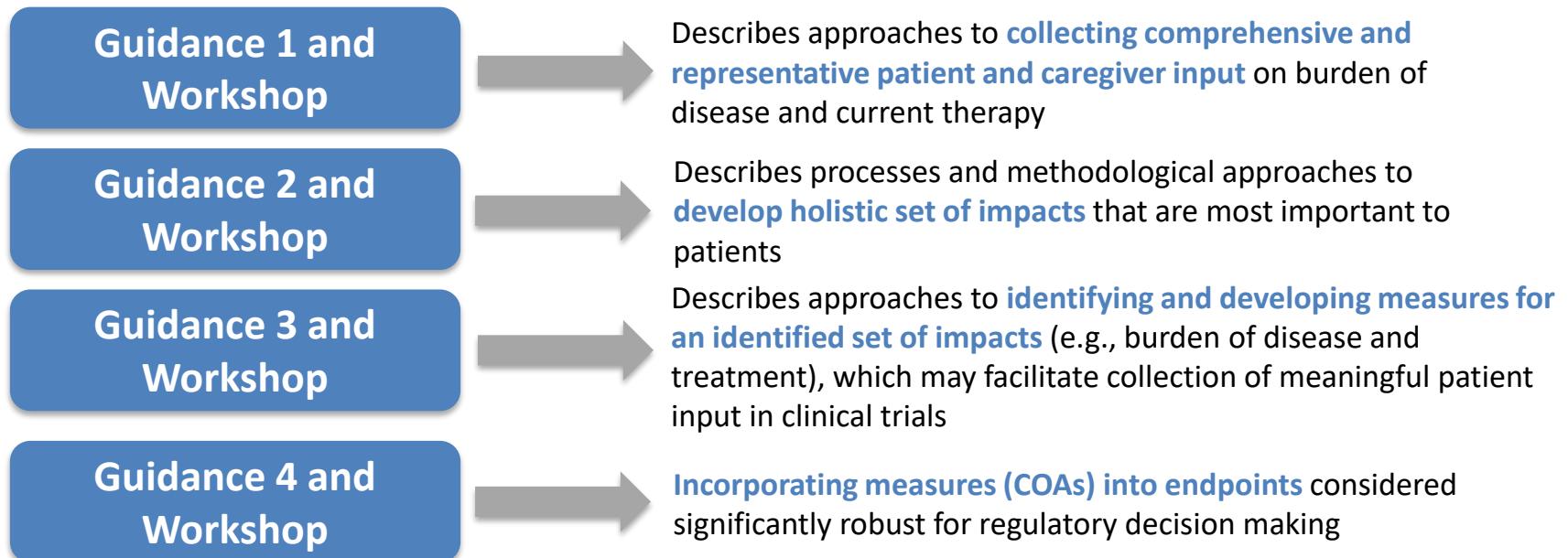
Patient organizations identify and organize patient-focused collaborations to generate public input on specific disease areas

- Amyloidosis
- Friedreich's Ataxia
- Lupus
- Osteoarthritis
- Spinal Muscular Atrophy
- Tuberous Sclerosis Complex
- Hypereosinophilic Syndrome
- Epidermolysis bullosa (EB) and Pachyonychia Congenita (PC)

Patient Focused Drug Development

CDER/CBER

- Upcoming PFDD Methodological Guidance Series



CDER's Professional Affairs and Stakeholder Engagement (PASE)

- Engages with patient and healthcare professional stakeholders on drug specific issues
- External Stakeholder Meeting Requests (ESMR) System
- Navigating CDER Workshops for Advocacy Groups

Center for Devices and Radiological Health

- CDRH Strategic Priority 2016- 2017 (Partner with Patients)
- Patient Engagement Advisory Committee
- Patient Preference Initiative

Contacts

Rare Disease Listening Sessions Pilot and Patient Engagement Collaborative:

Patient Affairs Staff: patientaffairs@fda.hhs.gov

FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov

Patient Focused Drug Development: patientfocused@fda.hhs.gov

CDER's Professional Affairs and Stakeholder Engagement:
CDERPASE@fda.hhs.gov

CDRH's Division of Industry and Consumer Education: DICE@fda.hhs.gov

Thank you



patientaffairs@fda.hhs.gov

Who's Who at Regulatory Agencies



Megan Lenz

*Director of Marketing and
Communications*

Cure SMA

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Effective FDA Engagement

Megan Lenz, Director of Marketing and Communications
Cure SMA

About SMA

- Spinal muscular atrophy is a disease that robs people of strength by affecting the motor nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe.
- Leading genetic cause of death for infants.
- Caused by a deletion or mutation in the survival motor neuron 1 gene.
- Incidence: 1 in 11,000 births.
- Carrier rate: 1 in 50.
- Estimated living population in the US: 10,000 - 12,000.

Treating SMA

- One FDA-approved treatment: Spinraza.
 - Approved December 2016
- Five additional drug programs being tested in clinical trials, including two in Phase 3 trials.
 - Gene therapy (AveXis)
 - Small molecule (Genentech/Roche)

About Cure SMA

- Founded in 1984.
- \$70 million in funding for research
- 4,000 families served annually by support programs.
- Combined patient, researcher and clinician conference is the largest in the world focused on SMA.
- Robust advocacy program, including advocacy with the FDA.

Our FDA Engagement

- Prescription Drug User Fee Act (PDUFA) testimony
- “The Voices of SMA”
- FDA listening session
- Externally-led Patient Focused Drug Development (PFDD) meeting
- Benefit-risk survey

Principles of FDA Advocacy

- Relationship building: get to know staff at the FDA, and become known to them.
- Provide both quantitative and qualitative information.
- Be specific.
- Time it right.

Knowing and Being Known

- Public meetings
 - <https://www.federalregister.gov/>
 - [https://www.fda.gov/ForPatients/Calendar/
default.htm](https://www.fda.gov/ForPatients/Calendar/default.htm)
- Connect with the Office of Health and Constituent Affairs
 - Listening sessions

Quantitative and Qualitative Information

- The Voices of SMA
 - A 24-page booklet sent to key FDA leaders
 - SMA patient stories grouped around three key themes revealed through focus group and survey research
- PFDD meetings
 - Stories from the community
 - *Voice of the Patient Report*
 - Supplemented by a max-diff benefit-risk survey

Be Specific

- Broad labeling
 - Four different disease types with the same genetic cause
- The severity of SMA and what would constitute meaningful change
 - Small changes = big impact
 - Importance of preventing further decline
- Impact of first approved drug on clinical trial design
- Placebo controls

Time it Right

- August 2014: first drug to treat the underlying cause of SMA reaches pivotal trials.
- November 2014: Cure SMA requests PFDD meeting.
- August 2015: Voices of SMA.
- December 2016: FDA listening session.
- December 2016: Spinraza approved.
- April 2017: PFDD meeting.

Questions to Ask Yourself

- Who are our contacts at the FDA? (CDER, CBER, etc.)
- What opportunities already exist to engage with the FDA?
- How can we be data-driven and story-driven?
- Where are we at in the process?
- What are our community's priorities?



Questions?

- Megan Lenz, Director of Marketing and Communications
- megan@curesma.org