Enhancing Patient Engagement at FDA

Andrea Furia-Helms, MPH
Director, Patient Affairs Staff
Office of Medical Programs and Tobacco
Office of the Commissioner

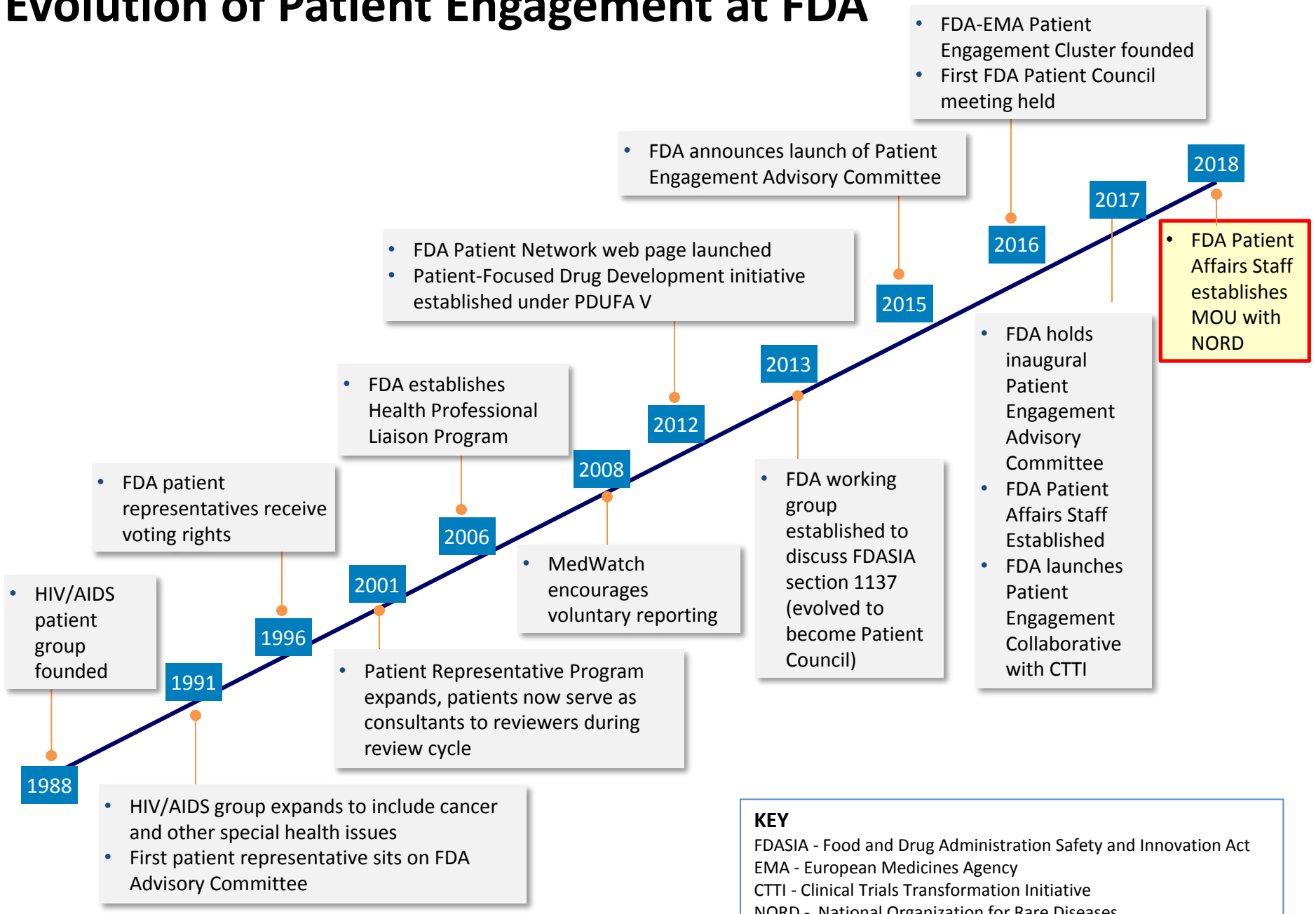
Global Patients Congress
International Alliance of Patients' Organizations
25 May, 2018

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 and diverse opinions/experiences
- Varied perspectives on risk tolerance and potential benefit
- The human element (judgment vs. empirical data)

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

Evolution of Patient Engagement at FDA



Source: FDA website, client materials

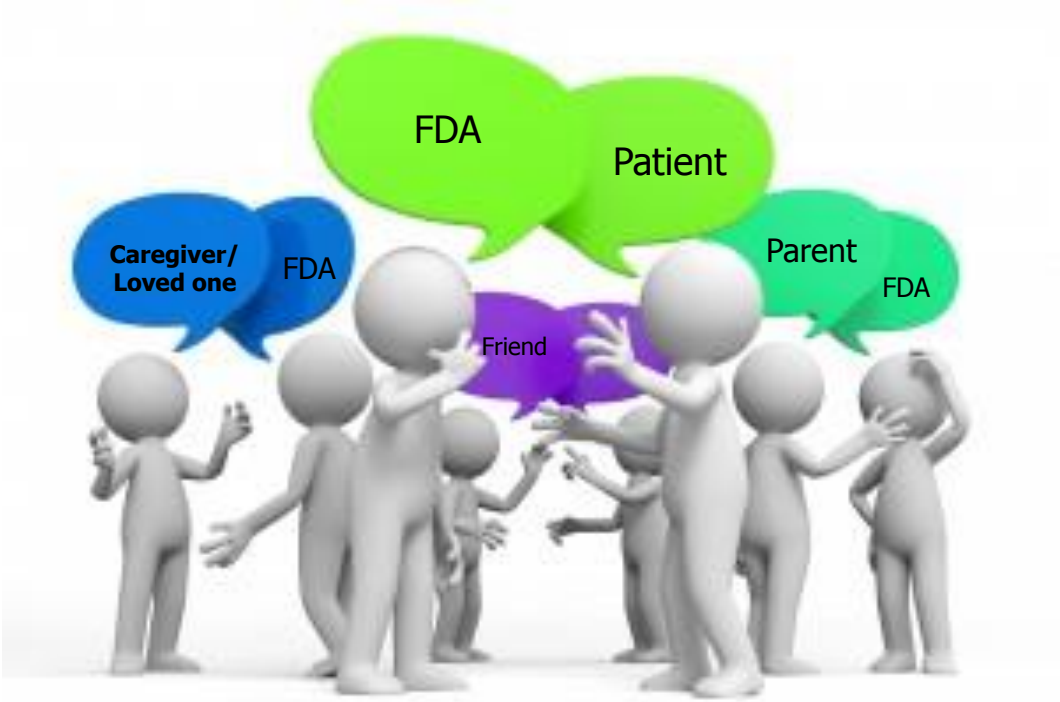
KEY

FDASIA - Food and Drug Administration Safety and Innovation Act
EMA - European Medicines Agency
CTTI - Clinical Trials Transformation Initiative
NORD - National Organization for Rare Diseases

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



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 Engagement Cluster

Mutual exchange on:

- Best practices to further enhance engagement activities, approaches and ideas
- Information on approaches for engaging with and involving patients stakeholders
- High profile topics of mutual interest, especially those with potential high public interest
- Priorities and goals regarding future proposals and collaborations to enhance engagement

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

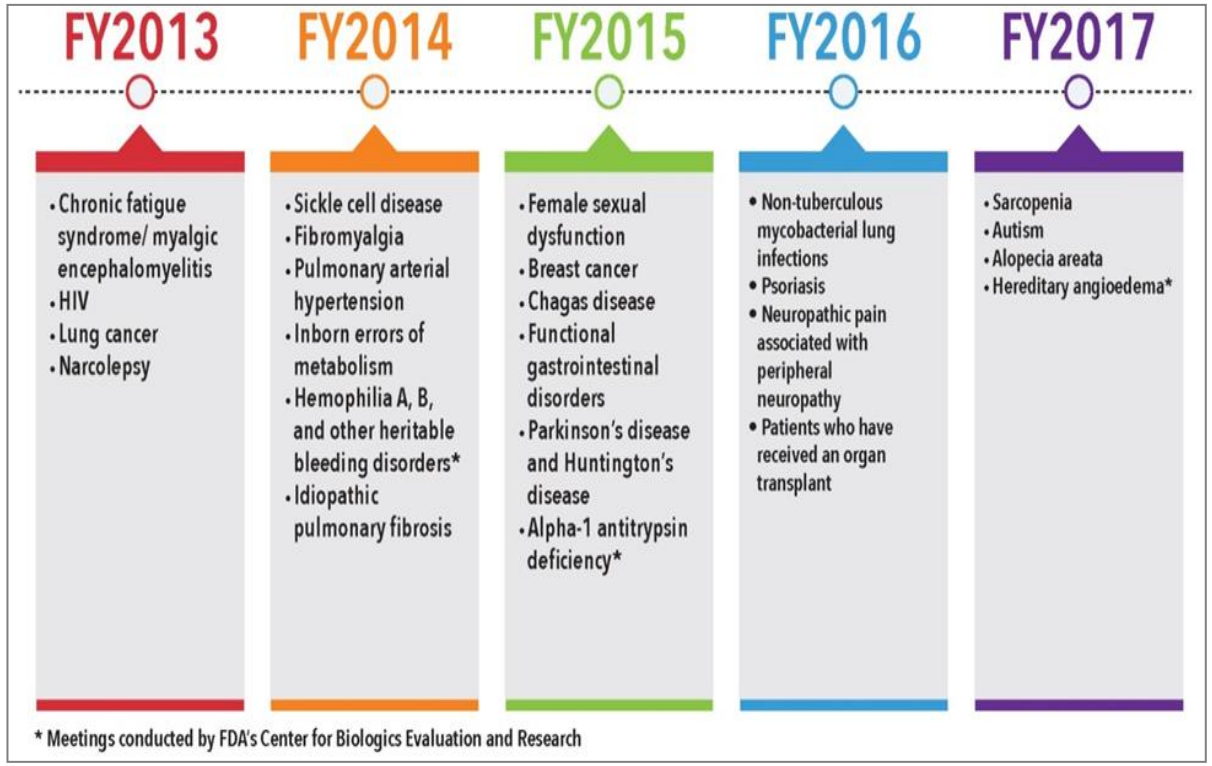
Office of the Commissioner

- 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

Guidance 1 and Workshop	Describes approaches to **collecting comprehensive and representative patient and caregiver input** on burden of disease and current therapy
Guidance 2 and Workshop	Describes processes and methodological approaches to **develop holistic set of impacts** that are most important to patients
Guidance 3 and Workshop	Describes approaches to **identifying and developing measures for an identified set of impacts** (e.g., burden of disease and treatment), which may facilitate collection of meaningful patient input in clinical trials
Guidance 4 and Workshop	**Incorporating measures (COAs) into endpoints** considered significantly robust for regulatory decision making

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