A Case Study in Creating Networking and Partnering Opportunities:

FDA Patient Affairs Staff and the National Organization for Rare Disorders (NORD)

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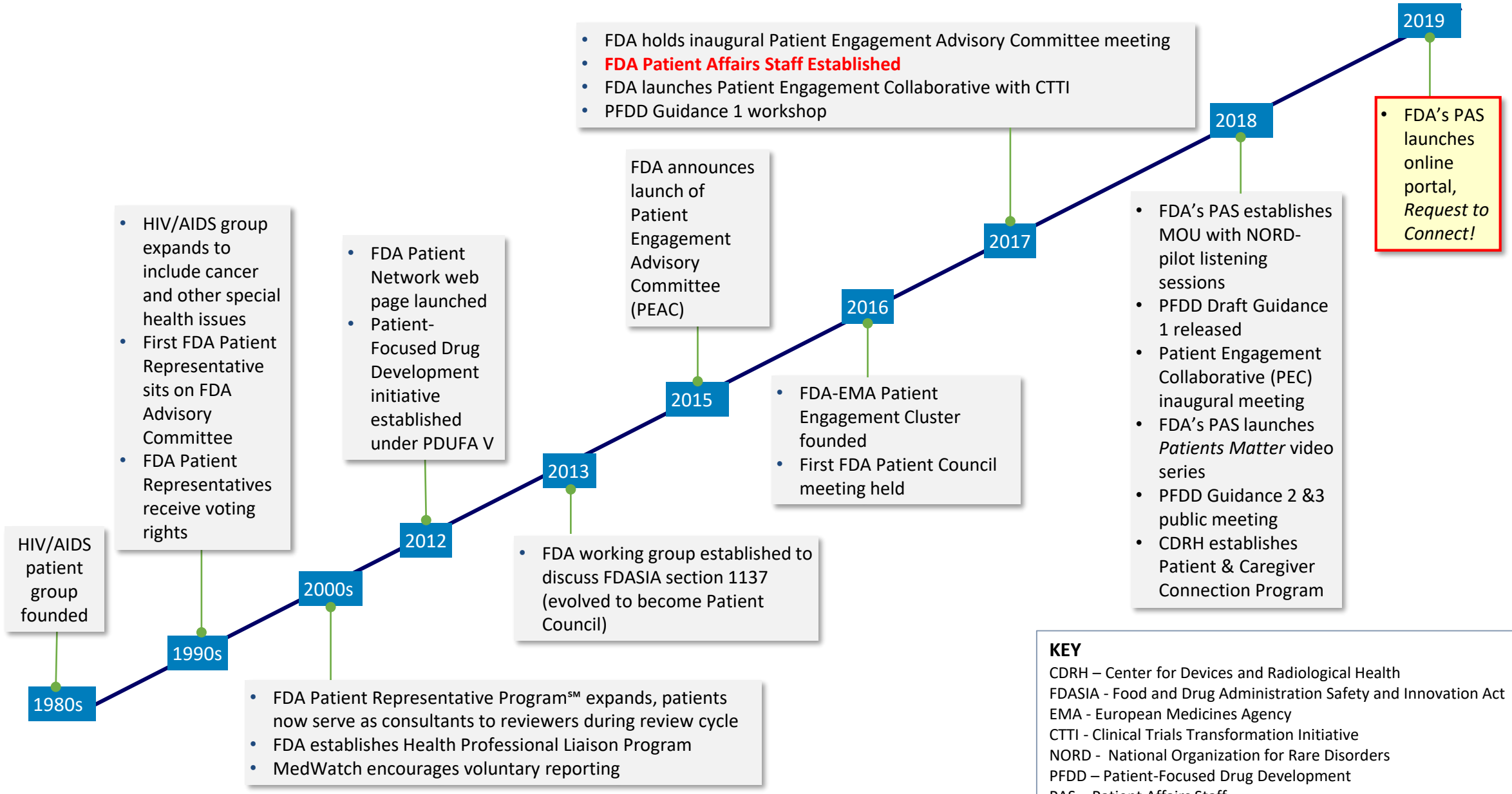
NORD® National Organization for Rare Disorders

Health Research Alliance

MEMBERS MEETING
September 25-26, 2019

Evolution of Patient Engagement at FDA



KEY

CDRH – Center for Devices and Radiological Health
FDASIA - Food and Drug Administration Safety and Innovation Act
EMA - European Medicines Agency
CTTI - Clinical Trials Transformation Initiative
NORD - National Organization for Rare Disorders
PFDD – Patient-Focused Drug Development
PAS – Patient Affairs Staff

Patient Affairs Staff (PAS)

Who we are

Patient Affairs Staff (PAS) in the Office of the Commissioner leads patient engagement activities across the medical product Centers—to allow dialogue and collaboration between patients, their advocates, and the FDA

What we do

- Creating and assisting with public-private collaborations and partnerships
- Lead cross-cutting programs and activities that leverage best practices and enhance patient engagement.
- Enhancing FDA's external communication platforms (e.g., Request to Connect, FDA's *For Patients* webpage, social media, etc.)

Patient Listening Sessions

Rare Diseases

- Collaboration through an MOU with the National Organization for Rare Disorders (NORD)
- Inform regulatory decision-making
- Educate review staff about rare diseases
- Help patients and their advocates understand the FDA's mission and work
- Provide a starting point to inform early stage research & development

Types:

- FDA-requested (specific set of questions to ask of a particular patient sub-population)
- Patient-requested (patient community wants to share their experiences and perspectives with the FDA)

What Are and Are Not Patient Listening Sessions

What are Listening Sessions?

ARE

- Non-public, non-advisory discussions between FDA staff and patients, their caregivers, and/or their advocates
- 1 to 2 hour meetings
- Via phone, in person at FDA in Silver Spring, MD, or a mix of the two
- Meant to facilitate expeditious sharing of patient or advocate perspectives on:
  - Disease burden
  - Treatment burden
  - Impact on daily activities
  - Priorities to consider in medical product development programs

Are NOT

- Open to industry
- Avenues for the endorsement of specific medical products
- Able to guarantee representative or comprehensive perspectives on disease or treatment burden
- Meant to take the place of other patient input and engagement processes, e.g., the FDA Patient Representative Program, Patient-Focused Drug Development (PFDD) Meetings

Patient Listening Sessions

Previously Conducted Listening Sessions

FDA-Requested Listening Sessions

May 13, 2019 - **Sanfilippo Syndrome**

February 20, 2019 - **Celiac Disease**

December 4, 2018 – **Fabry Disease**

October 23, 2018 – **Gene Therapy as a Treatment Modality for Hemophilia**

Patient-Requested Listening Sessions

September 17, 2019 - **Osteogenesis Imperfecta**

August 7th, 2019 - **Osteoarthritis (OA)**

June 13th, 2019 - **Neurofibromatosis (NF)**

May 29th, 2019 - **Fibrodysplasia Ossificans Progressiva (FOP)**

January 16, 2019 - **Amyotrophic Lateral Sclerosis (ALS)**

November 5, 2018 - **Biliary Atresia, Progressive Familial Intrahepatic Cholestasis, Wilson's Disease**

NORD®
National Organization for Rare Disorders

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 280 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

Listening Sessions: NORD's Role

Listening Sessions Coordination

1. Disease is identified by FDA division
2. NORD connects with disease-specific organization
    1. If no organization
    2. If multiple organizations
3. NORD recruits interested individuals
    1. Demographic diversity; age, location, knowledge, gender,
    2. FDA Questions,
        1. i.e. knowledge of gene therapies
        2. disease severity
        3. role (patient v caregiver)
4. FDA reviews and selects
5. NORD connects, one-on-one with selected participants

Listening Sessions: Feedback

Review Division Staff

The listening session format provides the opportunity for review staff to directly connect with patients, refocus on the ultimate mission of what we do, and integrate the patient voice and experience in our guidance and decision making. It is extremely valuable and appreciated. The materials that we were provided with prior to the session were excellent.

I think the prep sessions were very helpful and helped the reviewers to develop focused questions, maximize the time with the patients. I think the reviewers were very good at keeping things moving while making sure everyone participant had an opportunity to provide input.

Listening Sessions: Feedback

Patient/Caregiver Participants

It is very heartening for our community to see such interest and action taken to enhance the agency's understanding of what patients and families face with Sanfilippo!

I feel that all of us were able to explain our stories very clearly even in a short time. I understand that we have so much more to tell the FDA. But we were able to answer questions much longer than we intended, which I felt was amazing … I want to make a difference, and I want to inspire the world...thank you for giving us your time and your full attention. I'd love to come back and share more of my story in person.

Medical Product Center Patient Initiatives

Center for Drugs Evaluation and Research (CDER)

- Professional Affairs and Stakeholder Engagement (PASE)
- FDA-led Patient-Focused Drug Development Meetings (PFDD)
- Externally-led Patient-Focused Drug Development Meetings
- PFDD Methodological Guidance Series

Center for Medical Devices and Radiological Health (CDRH)

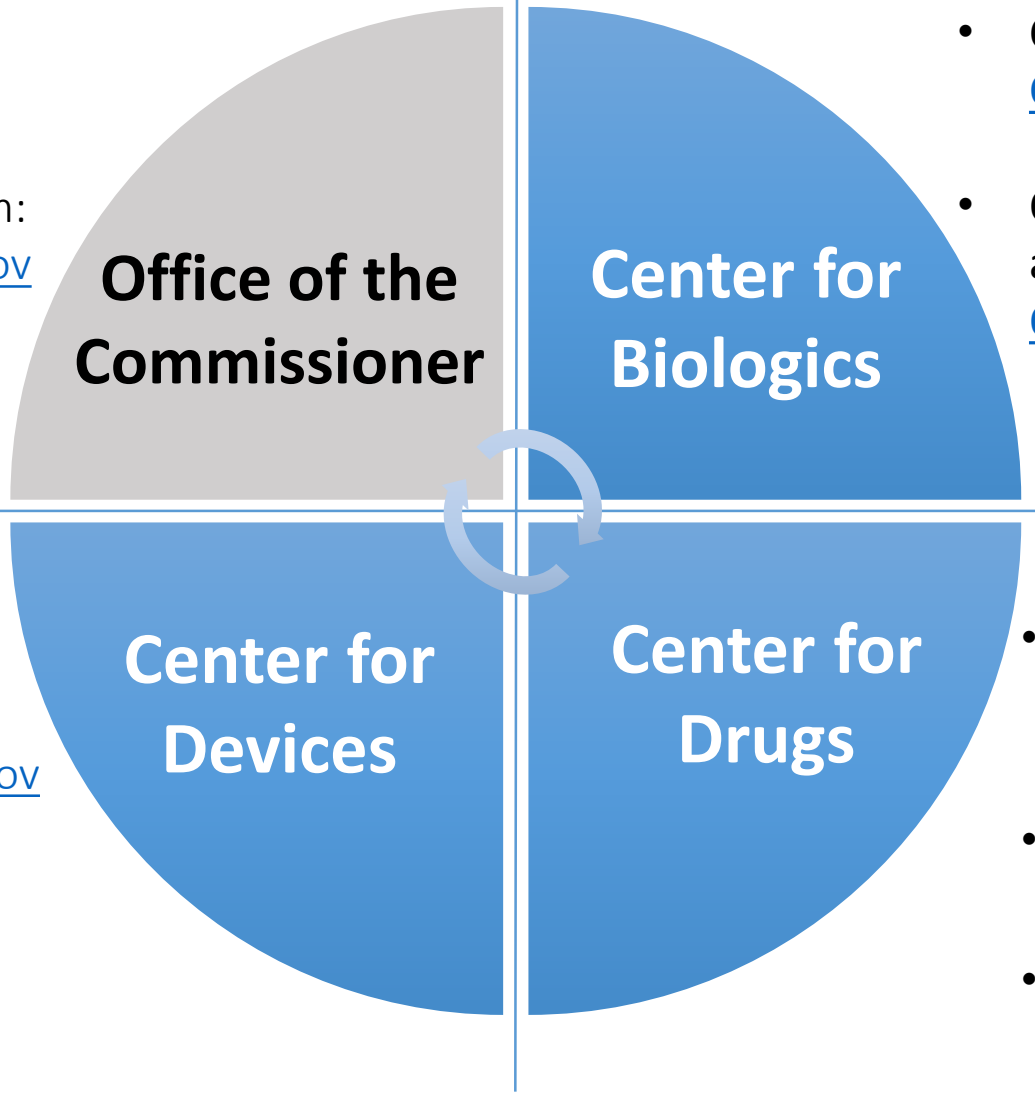
- Partner with Patients
- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program

Center for Biologics Evaluation and Research (CBER)

- Interactive Meetings with Patients
- CBER Workgroups:
- CBER Patient Engagement Workgroup
- CBER Rare Disease Coordinating Committee
- CBER Science of Patient Input (SPI) Team

Patient Engagement Across FDA

- FDA Patient Affairs Staff: PatientAffairs@fda.gov
- FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov

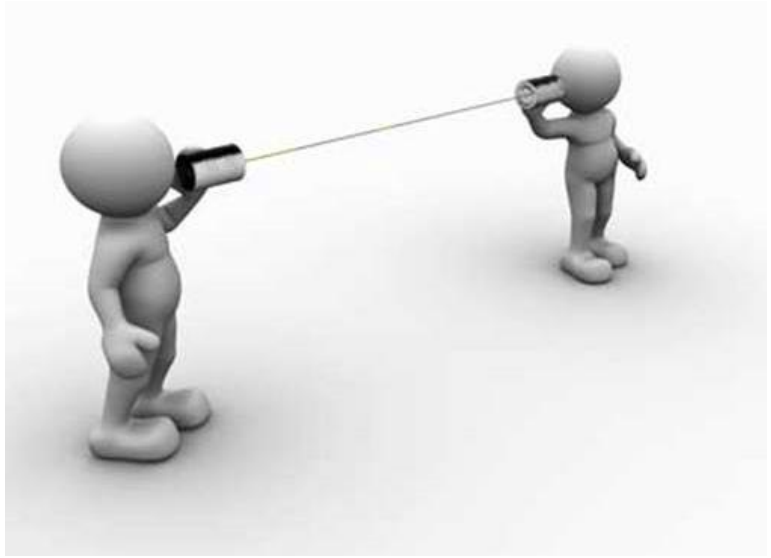


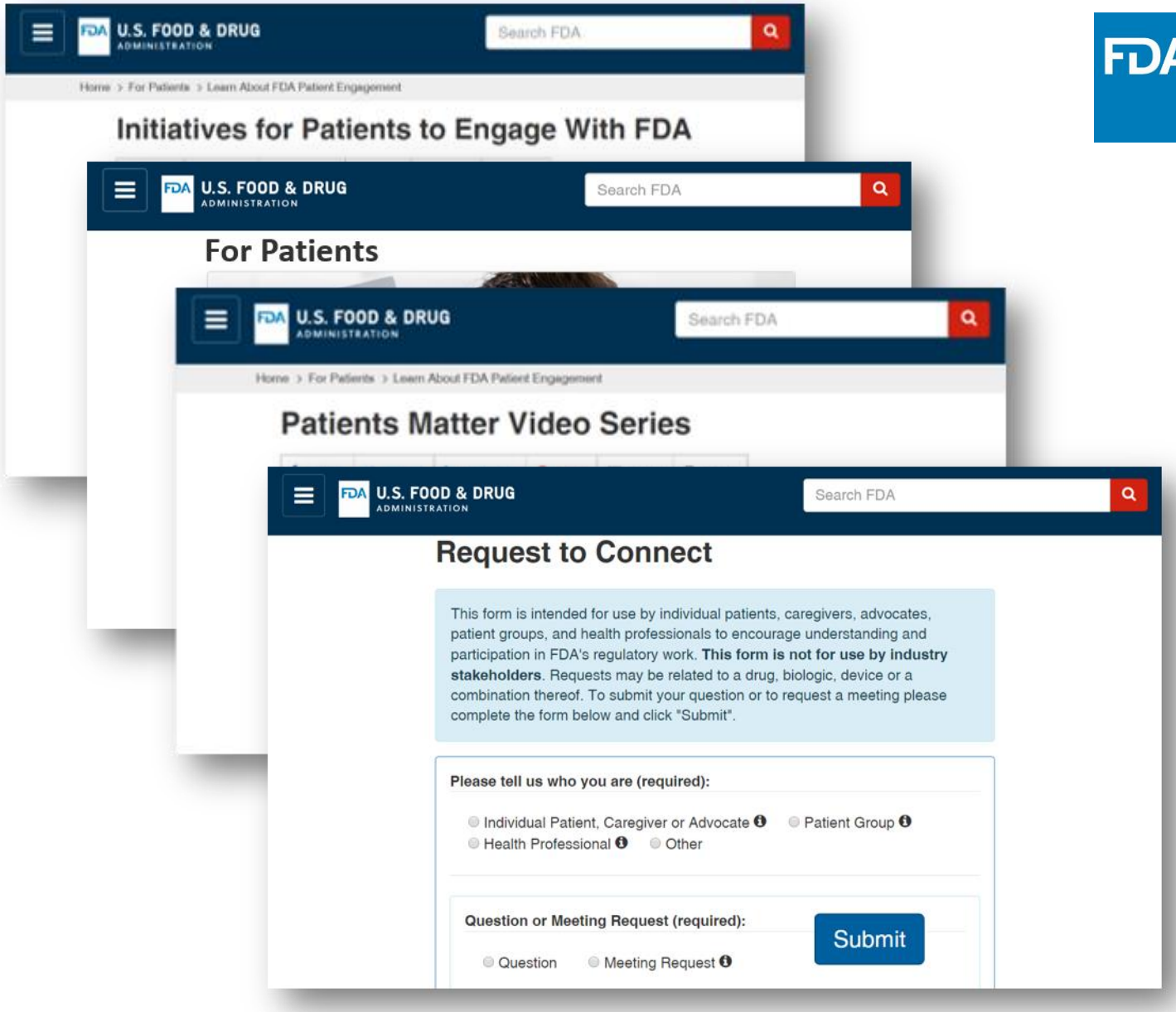
- CBER's Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov
- Office of Communication, Outreach and Development: OCOD@fda.hhs.gov

- Patient Engagement Meeting Requests: CDRH_PatientMeetings@fda.hhs.gov
- CDRH's Division of Industry and Consumer Education: DICE@fda.hhs.gov

- Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov
- CDER Division of Drug Information: DrugInfo@fda.hhs.gov
- Patient Focused Drug Development: patientfocused@fda.hhs.gov

External Communication Tools





www.fda.gov/**RequestToConnect**

NORD/FDA Promotional Opportunities

- 300 Member Organizations
- Communication channels:
  - Facebook Group
  - E-newsletter
  - Annual training and local networking
- Webinar Resources
  - FDA EL-PFDD
  - Online Portal
  - Listening Sessions

NORD®
National Organization for Rare Disorders

www.rarediseases.org

When in doubt…contact Patient Affairs!

FDA

PatientAffairs@fda.gov

301-796-8460

@FDAPatientInfo

www.fda.gov/RequestToConnect