Enhancing FDA's approach to Patient Engagement

Current State Analysis and Recommendations



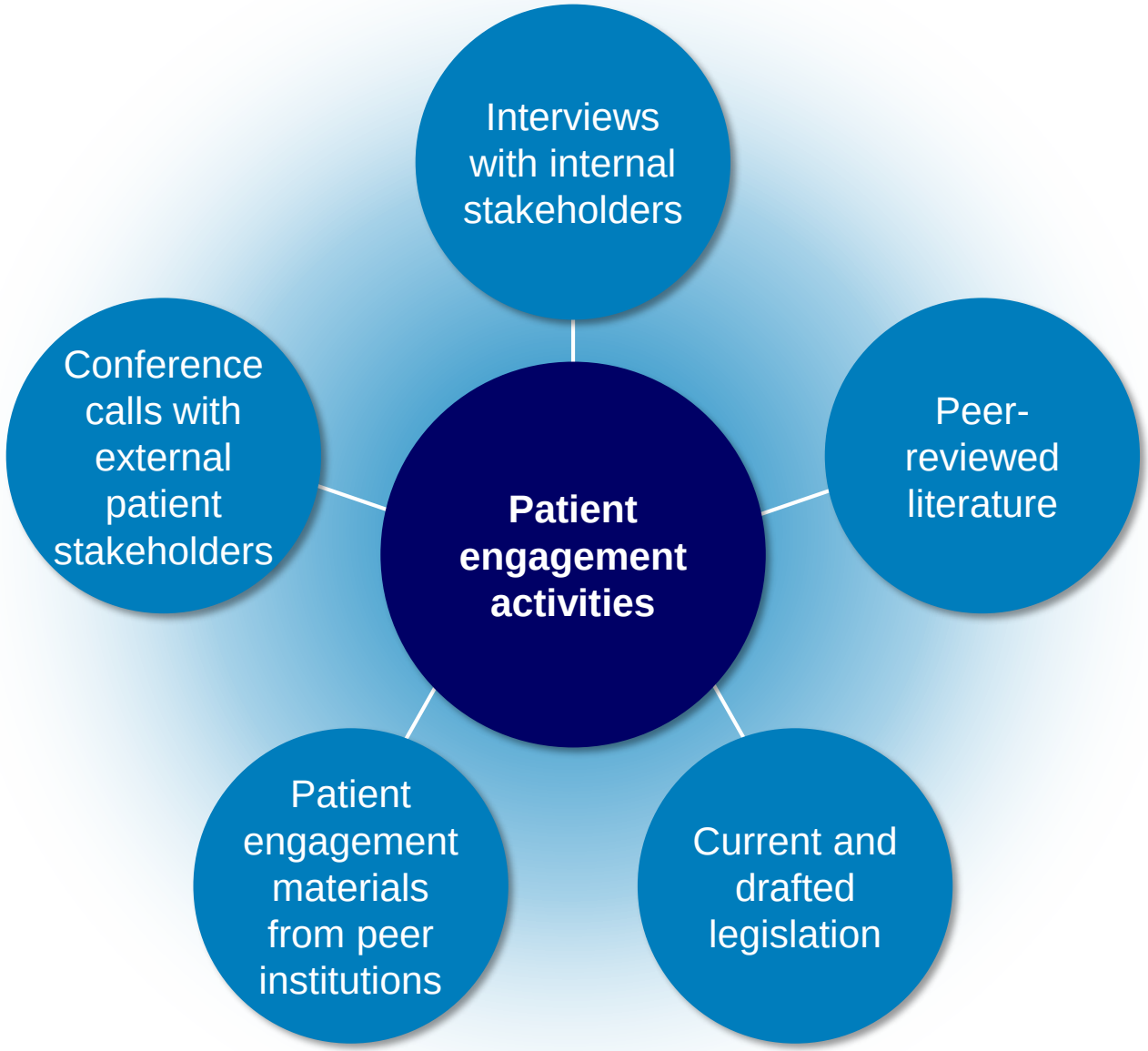



May 2017

Executive summary

- Patient input is playing an important and increasing role in the development and regulation of medical products.
- A large number of patient engagement activities are underway in FDA's medical product centers.
- With an eye to the future, FDA launched a ***current-state analysis*** of its patient engagement activities, which revealed that taking steps like clarifying responsibilities and fostering systems and tools that promote transparency, accessibility, and collaboration within the Agency and with the public would help strengthen patient engagement efforts.
- A resulting proposed ***future state*** for FDA patient engagement activities contains the following high-level objectives: **(1)** develop a nuanced understanding of the patient perspective of disease and **(2)** support patients and their advocates in understanding regulatory processes and in navigating FDA.
- To achieve these objectives, we propose a comprehensive portfolio of patient engagement activities, supported by the following structure:
  - A new central staff, the ***Patient Affairs Staff (PAS)*** (located in OMPT), would be responsible for **(1)** managing inquiry triage and navigation, **(2)** supporting development of tools and services, and **(3)** coordinating outbound communications to patient stakeholders. The supporting services currently provided by OHCA must be expanded and enhanced, and new talents and skillsets are needed as are clear mechanisms for accountability to FDA centers.

Sources of insights for this effort



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Context for this effort

Patient engagement at FDA traces its roots to FDA's response to the HIV/AIDS advocacy movement of the 1980s and 1990s.

Over the past three decades, patient engagement has expanded and evolved to include a range of institutional mechanisms for patients to engage with FDA (see next slide).

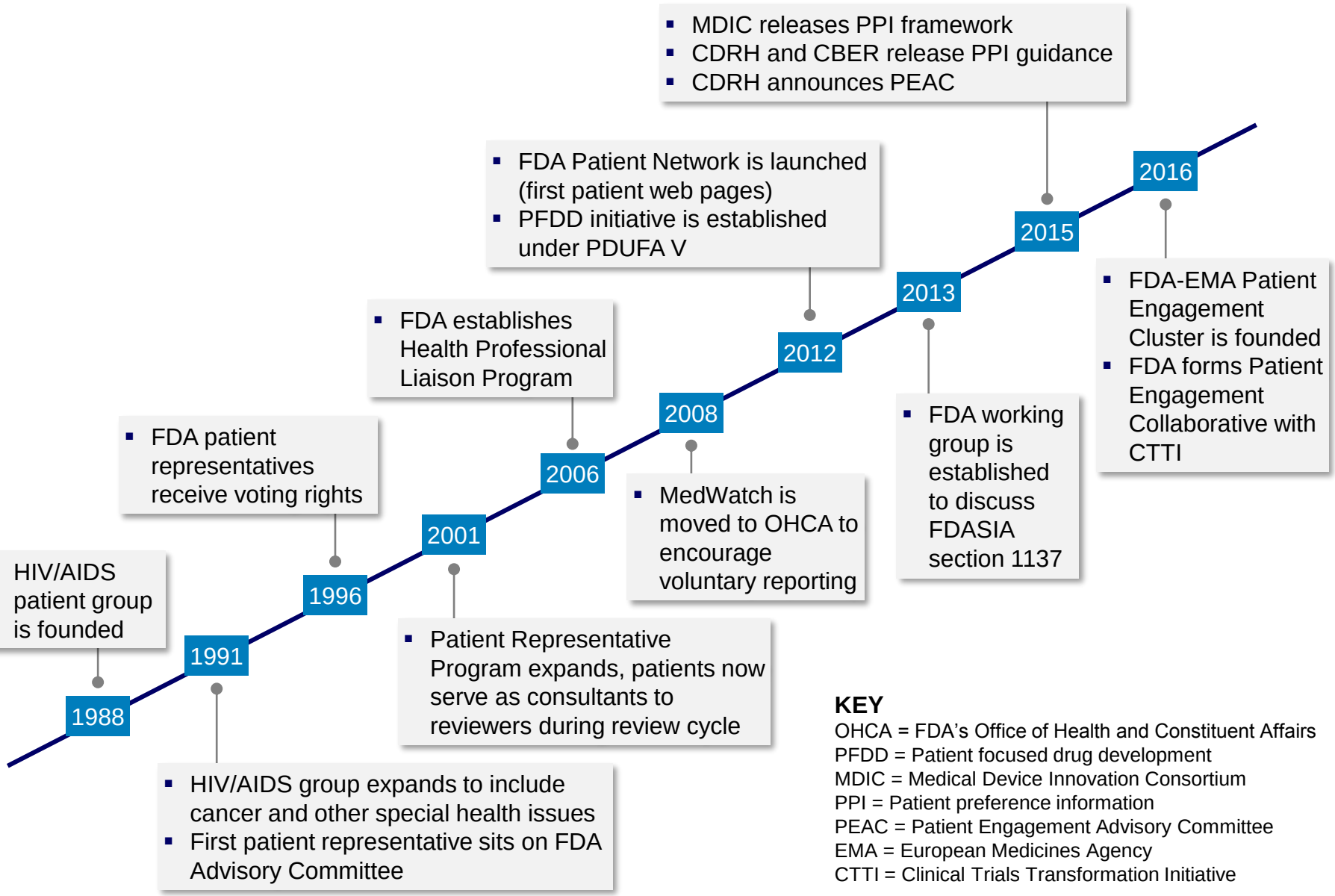
Today, FDA is advancing the science of incorporating patient perspectives into regulatory decision-making and policy development.

It is critical to build a forward-looking, collaborative, and patient-centric engagement program that will carry us into the next generation.

 Source: FDA website, client materials

Evolution of patient engagement at FDA

AS OF DEC 1, 2016



KEY

OHCA = FDA's Office of Health and Constituent Affairs
PFDD = Patient focused drug development
MDIC = Medical Device Innovation Consortium
PPI = Patient preference information
PEAC = Patient Engagement Advisory Committee
EMA = European Medicines Agency
CTTI = Clinical Trials Transformation Initiative

 Source: FDA website, client materials

Current congressional requirements for patient engagement at FDA

AS OF DEC 1, 2016

PDUFA V and FDASIA introduced new requirements for FDA's patient engagement efforts

- **PDUFA V Section X.C**: Holds CDER and CBER responsible for hosting 20 patient-focused drug development meetings and outlining approach for incorporating patient input into regulatory decision-making
- **FDASIA Section of 1137**: Requires earlier and greater patient participation in medical product discussions

"Develop a systematic process to include patients earlier in drug development than Advisory Committee Stage through consultation with scientific review divisions"

PDUFA VI shifts the focus of patient engagement efforts while MDUFA IV includes its first patient engagement provisions

- **Draft of PDUFA VI Section I.J.1**: Holds CDER and CBER responsible for advancing science of patient input by:
  - Strengthening staff with expertise in advancing science of patient input
  - Drafting guidances on collecting and interpreting patient input, including patient information collected through externally held PFDD meetings
  - Maintaining a public catalogue of tools for external stakeholders
  - Holding a public workshop on this topic
- **Draft of MDUFA IV Section IV.F**: Holds CDRH responsible for advancing science of patient input by:
  - Strengthening staff with expertise required for responding to applications with PRO or PPI materials
  - Holding public meetings on this topic

PRO = Patient reported outcomes

Source: Published legislation, draft legislation

Patient engagement expectations held by patients and industry

Direct quotes from patients / their advocates

"The Centers are all doing good, exciting work relating to patient engagement… now we d like to see a movement towards coordination

"Soon the Agency will have to provide guidance to industry. Companies worry that premarket interactions will be a COI issue, but including patients early in development is key"

COI = Conflict of interest

PDUFA VI changes the game. We re eager to know how FDA plans on gathering actionable patient voice information now that PFDD meetings aren t internal"

"The new Advisory Committee could signal a new, increased role for the patient. That would be exciting

Press clippings regarding industry players

"[It will be important] to get agreement with the FDA on how to validate these patient informed endpoints and how to move forward with labeling, and I'm not convinced the FDA actually knows the answer"

Vas Narasimhan, Global Head of Development for Novartis Pharmaceuticals

"Sanofi Appoints Dr. Anne C. Beal to the Newly Created Position of Chief Patient Officer"

Globe Newswire, March 2014

"[Pharmaceutical] companies say the agency needs to more clearly define the standards they require for patient focused outcomes to become a true measure of a drug s success"

IB Times, September 2015

"We need a sweet spot [on patient input], and it hasn't been found. And the FDA has not done a good job of finding that sweet spot"

Diana Zuckerman, President of the National Center for Health Research

Source: Conference calls with patient stakeholders, published reports

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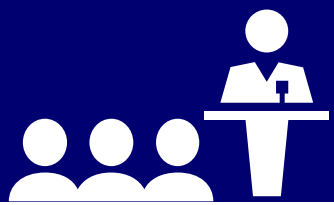
Patient engagement at FDA today "by the numbers"

NOT EXHAUSTIVE

24
Patient Focused Drug Development meetings sponsored Agency-wide



350,000
Recipients on MedWatch listserv

Relationships with more than **500** patient advocacy groups

96%
Adv Committees with patient reps

63%
CDRH staff engaged directly with patients in 2016

201
patient reps for **300** conditions and diseases

Source: Client interviews, Center-provided data

Current patient engagement activities: OHCA

Engagement coordination

Strategy:

- Provide point of contact for patients and their advocates and tools and services for patient engagement across centers
- Serve as central coordinating office for patient and HCP engagement across FDA (e.g., Patient Council)

Inventory of activity

Host & attend meetings	• Liaise with patient and HCP groups to keep communities informed, jointly solve problems and shape policy • Facilitate 400-500 *ad hoc* meetings per year with individual patients, HCPs and advocacy groups • Coordinate Commissioner listening sessions
Respond to requests	• Coordinate and support expanded access programs • Serve as one of the initial points of contact for individual patients, patient advocacy groups and HCPs • Triage and respond to ad hoc patient inquiries on wide variety of issues (e.g., answer questions about regulatory process, navigate clinicaltrials.gov and serve as empathetic sounding board)
Outbound communication	• Manage MedWatch security alerts • Manage FDA Patient Network (i.e., website, bi-weekly newsletters, webinars, and Twitter feed)
Solicit targeted input	• Identify issues and concerns for patient and provider groups to drive Agency communication strategy • Solicit comments on FDA documents (e.g., guidance documents, regulations, requests for input)
Inform regulatory decisions	• Manage Patient Representative Program (recruited and trained ~200 patient reps since 2010) • Lead Agency participation in EMA-FDA Patient Engagement Cluster

OHCA also responsible for delivering patient facing FDA messaging as part of broader OEA mandate

Source: Client interviews, Center-provided data

OHCA = Office of Health and Constituent Affairs

Current patient engagement activities: CDER

Engagement coordination

Strategy:

- Promote awareness of regulatory processes and resources within CDER amongst patients, their advocates and HCPs through outreach and educational programming
- Increase accessibility of CDER through creation of a dedicated office with low threshold for facilitating a meeting

(PASE = Professional Affairs and Stakeholder Engagement)

(OSP = Office of Strategic Programs)

Inventory of activity

Host & attend meetings	• Coordinated 24 patient focused drug development meetings aimed at systematically gathering patient perspectives on their condition (OSP) • Participate in 30-40 ad hoc meetings per year with individual patient groups to discuss disease and treatment related issues (PASE)
Respond to requests	• Respond directly or set up meetings with relevant CDER stakeholders in response to specific patient requests (PASE) • Process expanded access requests
Outbound communication	• Maintain Drug Trial Snapshots website, which presents demographic data on Office of New Drug clinical trials (PASE) • Host Roadmap for Engagement workshop to orient patient advocacy groups on how regulatory decisions are made and how they can best engage CDER (PASE) • Lead Safe Use Initiative to combat medication misuse and errors (PASE)
Solicit targeted input	• Convene patient advocacy group / HCP meetings on specific topics on behalf of offices / divisions (PASE)
Inform regulatory decisions	• Publish Voice of the Patient reports as resource for *reviewers (OSP)* • Include patient reps as voting members in most Adv Comms • Participate in EMA-FDA Patient Engagement Cluster

 Source: Client interviews, Center-provided data

CDER = Center for Drug Evaluation and Research

Current patient engagement activities: CBER

Engagement coordination

Strategy:

- Participate in cross-Center programs led by the OC and other centers
- Allow individual offices and divisions to drive engagement on specific topics of interest (collaborating across offices through virtual working group)

Inventory of activity

Host & attend meetings	• Sponsor patient focused drug development (PFDD) meetings (primary sponsor of 3 meetings; participation in others) • Host patients and patient groups on Rare Disease Day (in collaboration with CDER) • Participate in Commissioner listening sessions • Facilitate ~10-20 ad hoc meetings per year with individual patient groups about disease and treatment related issues • Rare disease coordinating committee attends NORD to interact with patients and advocacy groups each year
Respond to requests	• Respond to individual meeting requests at level of offices and divisions • Process expanded access requests
Outbound communication	• Distribute targeted communications on specific topics of interest or concern (e.g., to parent groups about vaccines) • Publish demographic information for clinical trials on CBER website
Solicit targeted input	• Participate in Agency-wide calls for input • Engage individual special govt. employees for feedback on specific patient communications
Inform regulatory decisions	• Publish Voice of the Patient reports on PFDD meetings • Issue guidance (e.g., on *Factors to Consider When Making Benefit-Risk Determinations*) for industry and FDA staff (August 2016, in collaboration with CDRH) • Include patient reps as voting members in most Adv Comms • Participate in EMA-FDA Patient Engagement Cluster

Source: Client interviews, Center-provided data

CBER = Center for Biologics Evaluation and Research

Current patient engagement activities: CDRH

Engagement coordination

Strategy:

- Partnering with patients is one of three 2016-2017 strategic priorities with two defined goals:
  - Promote culture of meaningful patient engagement
  - Increase use and transparency of patient input in decision-making

Inventory of activity

Host & attend meetings	• Hold Town Hall Sessions with invited patient speakers • Host public workshops about patient-focused topics
Respond to requests	• Process compassionate use requests
Outbound communication	• Publish patient-facing materials on website, Twitter feed, and mailing lists
Solicit targeted input	• Establish mechanisms for CDRH staff to engage directly with patients to obtain patient input on pre- and postmarket issues (partnered with 30+ advocacy organizations; >60% staff participated in 2016) • Chartered Patient Engagement Advisory Committee (PEAC) to obtain patient input on variety of patient-related issues (first meeting anticipated in first half 2017)
Inform regulatory decisions	• Developed Patient-Centered Benefit-Risk Framework and catalog of assessment methods in partnership with MDIC • Issue guidance (e.g., on *Factors to Consider When Making Benefit-Risk Determinations*) for industry and FDA staff (August 2016, in collaboration with CBER) • Included patient perspective data in 50% of PMA, *de novo* and HDE decisions in 2016 (target of 100% in 2017) • Include patient reps on most Adv Comms • Participate in EMA-FDA Patient Engagement Cluster

Source: Client interviews, Center-provided data

CDRH = Center for Devices and Radiological Health

Assessment of current state: Strengths

Internal stakeholder | External stakeholder

Area	Description	What we heard
Conviction and commitment	• Centers perform meaningful patient engagement work, and FDA staff are committed to working with patients	*"People at the FDA are very committed, they are willing to bend over backwards when it comes to patient communities"*
Problem solving with patients	• FDA has a variety of mechanisms for interacting with patients about their specific needs • FDA staff work flexibly to solve patient issues	*"FDA is very helpful in guiding us to the people who can help solve our problems"*
Leadership alignment	• FDA leaders agree that patient engagement is an important priority both for soliciting patient input into regulatory policy decisions and as part of the Agency's public service mandate	*"The question is when and how to engage patients, not whether or not to do it"* *"We have a duty as public servants to respond to inquiries from our constituents"*
Connectivity within centers	• Patient engagement staff within centers have relationships and visibility across divisions to support productive exchanges with patients	*"When a patient group approaches us in the center, we know who best to include in the discussion"*
Understanding patient perspectives	• Patient engagement efforts have given FDA real insight into clinically meaningful patient outcomes and how the disease process affects patients and their caregivers	*"These forums have given us key, and often surprising, insight into what is clinically meaningful for patients"*
Innovative approaches	• Innovative, forward-looking patient engagement experiments are occurring throughout the Agency (e.g., Roadmap for Engagement workshop, benefit-risk frameworks and assessment methods)	*"Advancing both the art and the science of patient engagement is critical to our mission of better incorporating patient perspectives into our decisions"*

 Source: Interviews with FDA staff, conference calls with patient stakeholders

Assessment of current state: Opportunities (1/2)

Internal stakeholder | External stakeholder

Area	Description	What we heard
Strategic intent of patient engagement	• FDA lacks widely understood and clearly defined objectives to guide patient engagement activity *across* centers; and the degree of strategic clarity varies from center to center	*"FDA is lacking a strategy or operational plan for engaging with patients, HCPs, and the general public"*
Cross Agency coordination	• Limited cross-Agency coordination to share best practices, jointly address shared policy questions, and present a single FDA voice to patient communities portrays a disjointed image of the Agency to the public	*"The centers are doing good work, but not coordinating at all, to our knowledge"* *"Centers do not always work collaboratively on cross-cutting issues"*
Institutionalization	• FDA currently depends on personal relationships / lacks a central repository for patient engagement information, impeding information-sharing across the Agency and challenging continued progress	*"A lot depends on who you know, so when there's turnover, you have to ask, 'is that information gone now?'"*
Improved access to FDA	• Lack of a single point of contact for patients or triage system for routing inquiries impedes both patients and Agency staff from rapidly accessing the right resources	*"A central entry point to the FDA would be tremendously helpful, so long as pre-existing relationships could continue"*
Feedback loop to patients / advocates	• Processes for communicating the outcomes of FDA touchpoints are inconsistent and often lacking • Lack of transparency to patients and their advocates about how patient voice has been incorporated into Agency decisions and policies causes frustration	*"When clear expectations are not communicated to patients, there is the danger of setting false expectations"* *"The lack of transparency is frustrating"*

Source: Interviews with FDA staff, conference calls with patient stakeholders

Assessment of current state: Opportunities (2/2)

Internal stakeholder | External stakeholder

Area	Description	What we heard
Approach to incorporating patient input	• Mechanisms for incorporating patient input into decision-making are variable (sometimes appropriately) and often lacking	*"Once patient information is gathered, what is the mechanism for incorporating it into regulatory work across the Agency?"*
Proactive posture	• Approach to engagement is largely reactive, driven largely by inbound requests or external mandates, rather than deliberate choice	*"Our approach to engagement is highly reactive, and underserved groups have less voice than perhaps they should"*
Performance	• Existing tools and processes within patient engagement functions are often inefficient or outdated • Lack of connectivity between centers and OHCA results in services that do not meet center needs	*"There is opportunity to professionalize some of our tools for patient engagement to make the experience better for both patients and FDA"*
Clarification of mission	• Lack of delineation between media communication activities and patient engagement efforts limits the credibility of the Agency as a patient partner	*"There's a danger of patients feeling like the FDA is just paying lip service"*
Additional forums for patient voice	• Absence of patient representatives prior to Adv Comms (e.g., in EOP1, EOP2) limits patient impact on drug development • Lack of clarity on acceptable interactions between sponsors and patients discourages engagement	*"Industry worries premarket interactions will be a conflict of interest issue, but including patients early in development is key"*
Appropriate metrics	• Absence of clearly defined metrics and mechanisms to review performance prevents Agency-wide evaluation of patient engagement effectiveness and identification of improvement opportunities	*"I'm not sure we measure any of our patient engagement activities today"*

 Source: Interviews with FDA staff, conference calls with patient stakeholders

Feedback from patient advocacy group conference calls

Area		Description	What we heard
Strengths	Conviction and commitment	• Centers perform meaningful patient engagement work and FDA staff is enthusiastically committed to working with patients	*"People at the FDA are really committed, they are willing to bend over backwards when it comes to patient communities"*
Strengths	Problem solving with patients	• Centers have a variety of mechanisms for interacting with patients and their specific needs • FDA staff works flexibly to solve patient issues	*"FDA is very helpful in guiding us to the people who can help solve our problems"*
Opportunities	Cross Center coordination	• Cross-Agency coordination would build on valuable ongoing work by encouraging centers to identify best practices, jointly address pressing policy issues and present a single FDA voice to patient communities	*"The centers are doing good work, but not coordinating at all, to our knowledge. It would be better to work in tandem"*
Opportunities	Communi cation mechanisms	• A central FDA entry point / triage system for inquiries would be helpful (particularly for individual patients), so long as pre-existing relationships can continue	*"There are far too many places to go, but, at the same time, sometimes even I could benefit from an operator"*
Opportunities	Institution alization	• Institutionalization of patient engagement efforts would ensure continued progress in a way not guaranteed by FDA's current dependence on personal relationships	*"A lot depends on who you know, so when there's turnover, you have to ask, 'Is that information gone now?'"*
Opportunities	Transparent follow up	• Transparency in communicating the outcomes of patient interactions so that impact is known would reduce frustration and guide future patient efforts	*"The lack of transparency is frustrating. Ten years later, if a guidance emerges, does that mean they were listening?"*
Opportunities	Forums for patient voice	• Inclusion of patient reps prior to Ad Board (e.g., EOP1, EOP2) would allow earlier input into development • Clarity on acceptable interaction between sponsor and patients would encourage use of patient perspective	*"Industry worries premarket interactions will be a conflict of interest issue, but including patients early in development is key"*

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- **Future state recommendations**

Core components of a robust patient engagement program

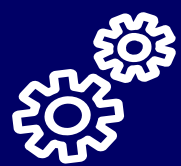
Direction

Center-led strategic guidance and evaluation frameworks that direct patient engagement efforts

Support & coordination

Systems and processes that support the coordinated delivery of patient engagement efforts across FDA

Design



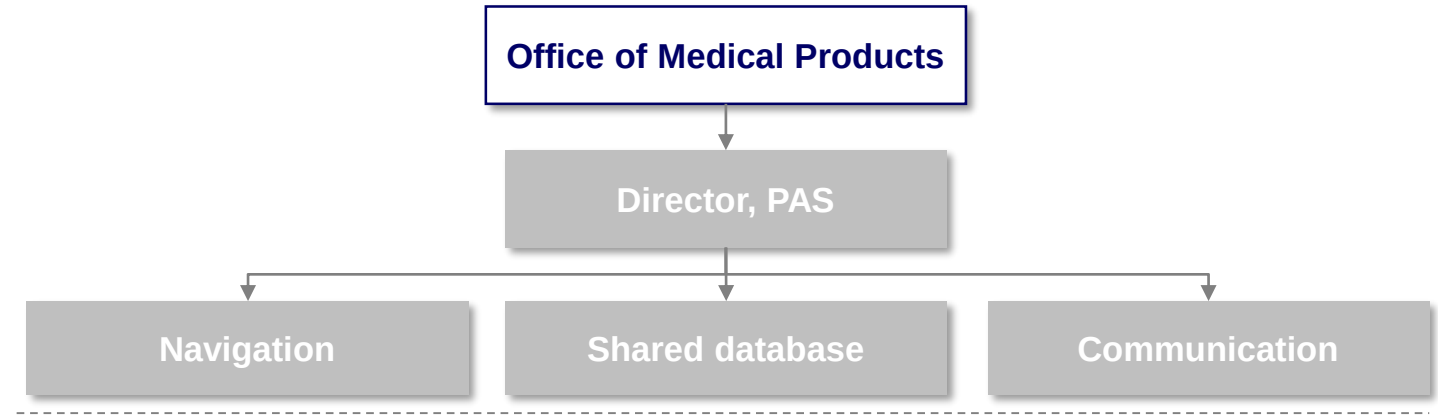
Design and delivery of center patient engagement activities and incorporation of patient perspectives into FDA work

Guiding principles for the Patient Affairs Staff (PAS)

Culture	PAS is guided by a strong sense of responsibility for supporting centers' patient engagement work and serving patients / their advocates
Talent & skills	PAS is staffed by talent whose background and skillset are in line with the office's work activities / mission and can drive the office forward
Workforce size	PAS is supported by a lean staff model that drives value to the centers without placing undue burden on resources
Informal networks	PAS is an active member of FDA's medical product community such that informal networks can develop alongside formal ones

Proposed PAS organizational profile

High level structure



Core responsibilities

- **Navigation**
  - Triage and navigation for inbound patient inquiries
  - Resolution of non-center specific inquiries
- **Shared database**
  - Maintenance of a shared database and calendar of patient engagement information / events
- **Communication**
  - Management of patient communication platform

Required skillset

- **Navigation**
  - **Knowledge of FDA** policy, processes and resources
  - **Connectivity** with centers
  - **Health and/or advocacy** background
- **Shared database**
  - **Technical capabilities** to deploy and maintain CRM tools
  - Understanding of **"customer" needs** (both those of PAS and centers)
- **Communication**
  - Experience with **web platforms, digital broadcast** and **social media**
  - **Communications** and/or editorial skills

Director of PAS must have familiarity with the Agency and possess the leadership qualities required to effectively work across centers and offices

Potential impact of PAS

- Development and communication of a **shared mission and vision for patient engagement** to clarify expectations for patients and provide high-level framework for center and office programs
- **Structured mechanisms for internal communication and collaboration** to reduce duplication of effort, improve coordination, and facilitate cross-pollination of ideas and innovation
- A **single point of entry** for patients / their advocates and resources for the **effective triage and navigation** of inquiries to level the playing field for less experienced groups and improve access to FDA overall
- An improved set of **scalable and sharable tools** to institutionalize and efficiently coordinate patient engagement efforts at FDA
- **Advancement of the science for integrating patient voice** into the regulatory process to better enable patient perspectives to shape product development and approval
- **Identification and monitoring of key indicators of success** for FDA's patient engagement program to enable continuous improvement and greater impact
- Tools and services for patient engagement that can **support HCP needs where there are clear operational synergies**