FDA Public Workshop
CDER and You: Keys to Effective Engagement
Hosted by Professional Affairs and Stakeholder Engagement (PASE)

Tuesday, April 3, 2018, 9am - 3pm
FDA White Oak Campus | 10903 New Hampshire Ave, Silver Spring, MD, 20993 | Building 31 Great Room

Register Today:
https://Keys-to-Effective-Engagement-FDA-Public-Workshop.eventbrite.com
Introduction and Opening Remarks

John Whyte, M.D., M.P.H.
Director, Professional Affairs and Stakeholder Engagement (PASE), CDER
Welcome

Janet Woodcock, M.D.
Director, Center for Drug Evaluation and Research (CDER)
AUDIENCE RESPONSE QUESTIONS

Noah Goetzel

*Oak Ridge Institute for Science & Education (ORISE) Fellow, Professional Affairs & Stakeholder Engagement*
Join the Twitter Conversation
#CDERandUENgagementWorkshop

Center for Drug Evaluation and Research
Your source for the latest drug information. Know the moment it happens.

FDA

FDA Drug Information
@FDA_Drug_Info

Receive the latest drug information from the US FDA. Contact us at 1-855-543-3784 or druginfo@fda.hhs.gov. Privacy Policy - fda.gov/privacy.

Silver Spring
fda.gov/AboutDDI
Joined July 2009

Tweets
Following
Followers
Lists

3,559
28
239K
1

FDU Drug Information
@FDA_Drug_Info - 6h
TOMORROW: @FDA_Drug_Info will live tweet from the #CDERandUENgagementWorkshop from 9a-3p. go.usa.gov/xnA1R

FDU Public Workshop
CDER and You: Keys to Effective Engagement
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www.fda.gov
5 Things to Know About the New Drug Approval Process
Live Demo: FDA.gov/RequestAMeetingOnDrugs

Christopher Melton
Health Communications Specialist,
Professional Affairs and Stakeholder Engagement (PASE)
Collecting Patient Experience Data: How You Can Best Help FDA

Selena Daniels, Pharm.D., M.S.

Team Leader, Clinical Outcome Assessments (COA) Staff, Office of New Drugs (OND), CDER
COLLECTING PATIENT EXPERIENCE DATA: HOW YOU CAN BEST HELP FDA?
the FDA is working to give patients a greater voice in medical product development and evaluation.

Success in these efforts could lead to tremendous advances in the understanding of health, disease, diagnosis, treatment, and recovery, ultimately transforming patients’ experience of health care by enabling physicians to tailor care to an individual’s specific needs and preferences.

Including clinical outcomes that are meaningful to patients can profoundly influence drug development by ensuring the patient voice is captured.”
Our Ultimate Purpose: Understand Patients’ Perspectives on Benefits and Risks

• **Clinical benefit**: A *positive clinically meaningful effect* of an intervention, i.e., a positive effect on how an individual *feels, functions*, or *survives*
  – How long a patient lives
  – How a patient feels or functions in daily life (includes both improvement as well as prevention/slowing decline)

• **Clinical outcome**: An outcome that describes or reflects how an individual feels, functions or survives
  – Assessed using clinical outcome assessments (COAs)

• Careful assessment of patients’ views on benefits and risks are an important part of regulatory decision-making
Patient Experience
What Is Patient Experience Data?

• Data that are collected by any persons and are intended to provide information about patients’ experiences with a disease or condition

• Includes the experiences, perspectives, needs and priorities of patients related to (but not limited to)
  – Symptoms of their condition and its natural history
  – Impact of the conditions on their functioning and quality of life
  – Experience with treatments
  – Input on which outcomes are important to them
  – Patient preferences for outcomes and treatments
  – Relative importance of any issue as defined by patients

Source: Title III, Section 3002(c) of the 21st Century Cures Act
Where Does Patient Experience Data Come From?

- The patient’s journey should be defined from the patient perspective (where possible) informed by input from patient partners and clinicians
Patient Partners

- A **patient** is any individual with or at risk of a specific health condition, whether or not they currently receive any therapy to prevent or treat that condition. Patients are the individuals who directly experience the benefits and harms associated with medical products.

- A **caregiver** is a person who helps a patient with daily activities, health care, or any other activities that the patient is unable to perform himself/herself due to illness or disability. This person may or may not have decision-making authority for the patient and is not the patient’s healthcare provider.

- A **patient advocate** is an individual or group of individuals, who may or may not be part of the target patient population, who has a role in promoting an interest or cause to influence policy with respect to patients’ health or healthcare.
PATIENT EXPERIENCE

- Disease Symptoms
- Disease Impacts
- Treatment Burden
- Preferences
- Disease Burden
# How Do You Collect Patient Experience Data?

<table>
<thead>
<tr>
<th>Method</th>
<th>Qualitative Methods</th>
<th>Quantitative Methods</th>
<th>Mixed Methods</th>
</tr>
</thead>
<tbody>
<tr>
<td>Uses <strong>direct communication</strong> to explore or confirm the meaning of interpretation of a topic from the participant’s perspective</td>
<td>Uses a <strong>tool</strong> (survey or questionnaire) that provides <strong>numerical information</strong></td>
<td>Uses both qualitative and quantitative data and approaches in an integrated manner in the same study or a set of related studies</td>
<td></td>
</tr>
</tbody>
</table>

| Scientific Question* | What aspects of disease are important to patients for measurement and reporting of clinical trial results? | How do we design a questionnaire measuring aspects of disease? | Do we measure symptom severity or frequency? |

*Example questions

Source: Adapted from Teddlie & Tashakkori (2009)
Why Is It Important To Collect Patient Experience Data?

- Patients are experts in their own experience of their disease or condition and the ultimate consumers of medical products.
- Patient experience data can inform medical product development and enhance regulatory decision making to address patients’ needs.
When Do You Collect Patient Experience Data?

- Before and throughout the medical product development process
- Precompetitive collaboration is encouraged!
Who Can Collect & Submit Patient Experience Data?

- Anyone can collect and submit patient experience data, including
  - Patients
  - Family members and caregivers of patients
  - Patient advocacy organizations
  - Disease research foundations
  - Researchers
  - Drug manufacturers
How Can External Stakeholders Submit Patient Experience Data To FDA?

- Various pathways exist
- FDA guidance on how to submit patient experience data is under development
- Depending on the purpose and type of data, different content and formats may be appropriate
How Is Patient Experience Data Used For Regulatory Purposes?

- Patient experience data is used to inform
  - Clinical trial design
  - Trial endpoint development and selection
  - Regulatory reviews including benefit-risk assessments
Summary

- Patient engagement is critical throughout the medical product development process.

- You can best help FDA by using scientifically sound methods to collect robust, meaningful, sufficiently representative patient input to inform medical product development and regulatory decision making.
Questions and Answers
Supporting Rare Disease Drug Development: CDER’s Rare Diseases Program

Lucas Kempf, M.D.

Acting Associate Director,
Rare Diseases Program, Office of New Drugs (OND), CDER
CDER’s Office of New Drugs
Rare Diseases Program

Lucas Kempf, M.D.
Associate Director Rare Diseases Program (acting)
Office of New Drugs
Center for Drug Evaluation and Research/FDA
April 2018
Disclosures

• No Conflicts of Interest
• Nothing to Report
• Opinions expressed are personal and do not reflect those of the FDA
Rare Diseases Program

- The current team
  - Lucas Kempf
    - Associate Director (Acting)
  - Larry Bauer
    - Regulatory Scientist
  - Althea Cuff
    - Science Policy Analyst
  - Tracy Cutler
    - Science Policy Analyst
    - EMA cluster coordinator
Rare Diseases Program

- Rare disease have less than 200,000 people
- There are 7,000 known rare diseases
- 1 in 10 people are affected by a rare disease
Challenges for Rare Disease Drug Development

- Rare diseases **natural history** is often poorly understood/characterized
- Diseases tend to be progressive, **serious**, life-limiting and life-threatening and lack **approved therapy**
- **Small populations** often restrict study design and replication
- **Phenotypic** diversity within a disorder adds to complexity, as do **genetic subsets**
- Well defined and validated **endpoints, outcome measures/tools, and biomarkers** are often lacking
- Lack of **precedent** for drug development
- **Ethical** considerations for children in clinical trials
CDER Rare Diseases Program

Mission Statement:
• Facilitate
• Support
• Accelerate

…the development of drug and biologic products for the treatment of patients with rare disorders
Rare Diseases Program Responsibilities

Coordinate development of CDER Policies and Procedures

• Guidance development
• Continuing involvement with Senior FDA staff re: Rare Diseases Program and its role

Assist in development of good science

• Database adjudication committee for NMEs
• Specific projects/peer reviewed publications
• Workshop development
  • Rare disease trial designs
Rare Diseases Program Responsibilities

**Coordinate internal training in rare diseases**
- 101 course for new reviewers
- 102 advanced training day for review staff

**Assist in external training for the rare disease community**
- Presentations at national and international meetings
- Workshop development
  - Rare disease trial designs workshop
- Panel Participant/ Speaker at Patient Focused Drug Development Workshops
  - FDA
  - Externally Led
Rare Diseases Program Responsibilities

• Review Rare Pediatric PRV requests and Developed procedures for management
• FDA Rare Disease Council member
• NORD Registries Cooperative Agreement with FDA
Rare Diseases Program Projects

**Work collaboratively with stakeholders**

- NIH Collaborations
  - NIH/FDA Joint Task Force
  - Rare Disease Day Annual Meeting
  - CDER/TRND Drug Development Meetings
  - NCATS Natural History Studies Initiative
Rare Diseases Program Projects

Work collaboratively with stakeholders

• Patient/Patient Organizations Meetings
  – Face to Face meetings with patient advocacy groups often in collaboration with PAS, PASE, and/or OHCA
  – Presentations to stakeholder groups
  – Planning Committee members for NORD Annual Summit
CDER’s Annual Novel Drug Approvals: 2008 - 2017

In 2017, CDER approved 46 novel drugs. The ten-year graph below shows that from 2008 through 2016, CDER has averaged about 31 novel drug approvals per year.

![Bar chart showing new drug approvals from 2015 to 2017, with categories for total NME and rare NME.](chart.png)
Expediting Rare Diseases
Drug Development

• Programs have been developed to target serious diseases with unmet medical needs when a new treatment could provide meaningful clinical benefit.

Guidance for Industry Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014
## 2017 Rare NME approvals

<table>
<thead>
<tr>
<th>Feature</th>
<th>Rare (#18)</th>
</tr>
</thead>
<tbody>
<tr>
<td>First in class</td>
<td>56%</td>
</tr>
<tr>
<td>Fast track</td>
<td>44%</td>
</tr>
<tr>
<td>Breakthrough</td>
<td>44%</td>
</tr>
<tr>
<td>Priority</td>
<td>78%</td>
</tr>
<tr>
<td>Accelerated</td>
<td>22%</td>
</tr>
<tr>
<td>First in the US</td>
<td>72%</td>
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</tbody>
</table>
Statistics: Orphan Drug Designations

Orphan Drug Designations Requested/Granted by Calendar Year

- No. Original Requests Received
- No. Designated

- 527
- 478
Rare Pediatric Disease (RPD) Priority Review Voucher Program: Background

• 2012 FDA Safety and Innovation Act (FDASIA) [Section 908]
  • Provides an incentive to encourage the development of drugs and biologics for rare pediatric diseases

• Upon approval, the sponsor may be issued a voucher redeemable for a priority review for a subsequent application that may not have otherwise qualified for a priority review

• The incentive offers a shorter review clock for marketing applications, 6 months compared with the 10 months standard review time

Rare Pediatric Disease Priority Review Vouchers, Guidance for Industry
RPD Requests and Determinations

Data as of September 15, 2016

www.fda.gov
Rare Pediatric Disease Priority Review Voucher Program

• The OOPD reviews requests for Rare Pediatric Disease designation
  • 41 Designated/6 Denied/7 Under Review

• Voucher requests are managed by the OND RDP
  • 11 Voucher requests were submitted with an NDA or BLA
    • 6 Vouchers awarded, 3 denied and 2 pending review
    • Two PRV’s have been redeemed

• Future (?)
  • Sunsets - 30 September 2016 although pending legislation may be extended to 31 December 2022 (for designation)/31 December 2027 (for redemption)
International Regulatory Communications: Development of an EMA/FDA Rare Disease Cluster

• Regularly scheduled teleconferences to exchange information and experiences
• Alternating Chairpersonship EMA/FDA
• Agendas circulated in advance of the teleconferences
• Core Members are joined by expert Reviewers
International Regulatory Communications

Development of an EMA/FDA Rare Disease Cluster

• To share scientific evaluation of various aspects of rare disease drug development
  • Identification of trial end points
  • Potential trial designs in small populations
  • Regulatory flexibility
  • Determination of the size of safety populations
EMA/FDA Rare Disease Cluster

• To share scientific evaluation of various aspects of rare disease drug development
  • Evaluation of pre-clinical data to support human trials
  • Design/conduct of post-marketing studies especially in the cases of breakthrough designation and accelerated approval (FDA) or PRIME designation and conditional/exceptional approval (EMA)
Overall, a total of fifty-three (53) agenda items were discussed between September 2016 – December 2017.
EMA/FDA Rare Disease Cluster

TOTAL DISCUSSIONS SINCE INCEPTION

- Percent of product discussions: 33%
- Percent of protocol assistance discussions: 67%

Total number of discussions: 30
Overall Impact of the Rare Diseases Cluster

- Percent of discussions led to alignment of understanding: 90%
- Percent of discussions led to alignment of actions: 63%
- Percent of discussions led to change in regulatory action: 20%
Thank you very much for your attention

Send us a Question at:
CDERONDNRareDiseaseProgram@fda.hhs.gov

Lucas.Kempf@fda.hhs.gov
Rare Diseases Program/OND/CDER/FDA
Questions and Answers
AUDIENCE RESPONSE QUESTIONS

Jamie Bishop
Project Manager,
Professional Affairs and Stakeholder Engagement (PASE)
BREAK

10:45 – 11:00 a.m.

Attendees interested in purchasing lunch should order now at the kiosk in the lobby.
Understanding the Needs of CDER Review Divisions

Elizabeth Hart, M.D.

Medical Officer,
Division of Gastroenterology & Inborn Errors Products (DGIEP), OND, CDER
Understanding the Needs of CDER Drug Review Divisions

Elizabeth Hart, MD
Medical Officer
Division of Gastroenterology and Inborn Error Products
Office of New Drugs
Center for Drug Evaluation and Research
4/3/18

www.fda.gov
CDER Review Divisions

- Evaluate efficacy and safety of new drug applications for specific indications by Sponsors
- Agency involvement & advice often begins early during drug development
- Agency involvement continues post-marketing to further assess safety
New Drug Development

IND Phases

- Clinical Phase 1: Safety/Tolerability and Pharmacological Studies
- Clinical Phase 2 (proof-of-concept): Early Efficacy Testing & Dose Determination
- Clinical Phase 3: Safety and Efficacy Studies

Non-Clinical: Research Lab & Animals
- Discovery & chemical synthesis

Clinical Outcomes Assessments & Natural History Studies
Level of Evidence of Efficacy: Legal Requirements

• 1962 Drug Amendments to the Food Drug & Cosmetic Act:
  – Required establishment of effectiveness of the drug as a prerequisite for marketing approval
  – Effectiveness established by “Substantial Evidence”
  – Substantial evidence consists of “Adequate & Well Controlled Investigations”
What are Adequate and Well-Controlled Studies?

- Studies that have been designed well enough so as to be able “to distinguish the effect of a drug from other influences, such as spontaneous change, placebo effect or biased observation” (21 CFR 314.126)

- Adequate and well controlled trials have:
  - Appropriate control for valid comparisons
  - Appropriate selection of subjects
  - Well-defined and reliable methods of assessing response
  - Adequate measure to minimize bias
  - Prospectively planned analyses designed with rigor
Defining Clinical Benefit

• Treatment benefit occurs when a drug positively effects
  – How a patient **feels** (e.g. symptoms)
  – How a patient **functions** (e.g. walks)
  – How a patient **survives** (e.g. improved mortality)

• Clinical effect must be clinically meaningful in the context of a given disease
Challenges in Drug Development for Rare Diseases

- Small population
  - Limited opportunity for study & replication

- Often Heterogeneous
  - Study population size limits statistical analysis

- Incomplete understanding of disease manifestations

- No precedent for drug development
  - Lack established endpoints, outcome measures & tools/instruments for the population
Drug Development:
(Especially for Rare Diseases)

Start with the end in mind: Obtain clinically meaningful evidence of benefit in how patients feel, function or survives from adequate and well controlled trial(s)
What Can Patient Organizations do to Facilitate Drug Development?

• Preform Natural History Study

• Provide Patient Experience Data

• Develop and Validate Qualitative and/or Quantitative Assessment Methods

• Encourage enrollment in randomized, controlled trials
Natural History Studies

• Comprehensive study characterizing a disease or subset of disease over time

• Identify variables that correlate with disease progression and outcomes in the absence of experimental treatment
  • Demographic, genetic, environmental

• Cohort
  • Prospective or Retrospective
  • Include all stages of disease from pre-symptomatic to death/cure/non-progressive chronic disability
Why are Natural History Studies Important?

• Scientific framework for rigorous investigation
  – Understanding disease outcomes and variability
    • Endpoints
    • Population
    • Sample Size

• External control population for pivotal trial*
  – Reserved for special circumstances in which there is a dramatic treatment effect & disease course is highly predictable & endpoints are objective
    • Population & efficacy assessments comparable to interventional study populations

Natural History Studies within the Regulatory Framework of Rare Disease Development
Patient Experience Data

- Inform Clinical Endpoints
  - Ensure bothersome signs and symptoms assessed
  - Ensure impact of condition on functioning and quality of life assessed

- Inform Benefit-Risk Assessment
  - Patient preference and tolerance for side effects
Assessment Tools

• Design and validate novel patient reported outcome measures

• Design and validate novel observer reported outcome measures

• Validate accuracy and reliability of tools originally developed for other disease populations
Clinical Trial Participation

• Patient participation is necessary for clinical trials & new drug development
  • Individual patients need to decide whether they are willing to undertake the burdens and potential risks associated with clinical trial participation

• Randomized, placebo/standard of care, controlled clinical trials are the most informative as they control bias
Conclusions

• Best access for patients to an effective therapy is an approved drug.

• Patient engagement early & throughout development process is important to informing drug development and regulatory decision making.

• You can help the FDA by early engagement and use of scientifically sound methods to collect representative patient data for natural history studies and endpoint selection and measurement.
Questions and Answers
AUDIENCE RESPONSE QUESTIONS

Portia Seals, J.D.
Health Communications Specialist,
Professional Affairs and
Stakeholder Engagement (PASE)
What CDER Can & Can’t Do

LCDR Sadhna Khatri, Pharm.D., M.P.H.

Regulatory Officer,
Professional Affairs and Stakeholder Engagement (PASE),
Office of the Center Director, CDER
Our discussion for today

- CDER Mission
- Opportunities for Engagement with CDER
  - Types of Engagement
- Value of Patient Engagement
- What CDER can’t do or say
CDER’s Public Health Mission

CDER’s mission is to:

- Promote and protect public health by assuring that safe and effective drugs are available to Americans

Ultimately, patients are the focus of all CDER activities and we need to engage with them
Opportunities for Engagement at CDER

• External Stakeholder Meeting Requests (ESMR) System
• Patient-Focused Drug Development meetings (PFDD)
  -Focused on better understanding the disease and patient experience.
• Advisory Committee Meetings
  - Open Public Hearing Portion
• Patient Representative Program
• Ad hoc FDA meetings
  -Typically scheduled with the Review Division
Opportunities for Engagement at CDER (continued)

- Citizen Petitions
- Comments to the docket for Federal Register Notices
- Guidance development
- Emails, letters and phone calls
Why Patient Recommendations are Valued

• Identify what matters/what is important to patients

• Benefit in development of clinical trials that are meaningful and realistic

• Raise FDA’s Awareness
The Value Patient Engagement Adds

- Patient input can direct drug development in many ways:
  - helps with the understanding of diseases and their impact
  - helps identification of specific symptoms that are significant to patients

- Helps design better clinical trials

We want to hear from you...
Transparency, The Law, and Confidentiality
(What we can’t do or say)

THE FDA CODE OF FEDERAL REGULATIONS

- FDA Code of Federal Regulations (CFR) is a huge sea of regulations that the FDA has created for regulating all products that come under its purview of regulation. The FDA codes of federal regulations are numbered and cover all products, processes and the activities that go into their creation.
Bias, Fairness, and Consistency

- Avoid bias to one company over another
- Focus on the specific scientific facts presented
- Meetings are granted free of bias
- Fairness, and consistency
- Open dialogue with patients and industry
- Points of view connected with sponsor support (financial for example) may have less credibility
Patient Recommendations are Valued, But….We Can’t Always Follow Them

• Statute
• Differences of opinion on interpretation of underlying facts
• Differences in views on practicality
• Conflict with laws or regulations creating legal risk
• Inconsistency with policy position or previous decisions
• Evolution of underlying data
Thank you

Contact Info:  CDERPASE@fda.hhs.gov or Sadhna.Khatri@fda.hhs.gov
Questions and Answers
Morning Recap

John Whyte, M.D., M.P.H.

Director, Professional Affairs and Stakeholder Engagement
Attendees interested in playing CDER Jeopardy should meet Noah Goetzel on stage to sign up.
CDER Jeopardy

John Whyte, M.D., M.P.H.
Director, Professional Affairs and Stakeholder Engagement
Rocking the Docket

John Wright, J.D.

Dockets Management Specialist,
Division of Docket Management, Office of the Commissioner
Rocking the Docket: What on Earth is Dockets Management and What do they do?
The Division of Dockets Management is under the FDA’s office of the Commissioner, Office of the Executive Secretary. We service private industry, individual stakeholders, all of the FDA’s regulatory centers, and 15000+ personnel.

FROM DRUGS TO LASER BEAMS, WE PROCESS IT ALL!
**FDA – OC – OES - DDM**

- The Division of Dockets Management is made up of three teams.
- The Administrative Proceedings Management Team (APMT), The Dockets and Document Management Team (D&D), and the Project and Public Reading Room (P_PRR).
DDM/APMT

• The Administrative Proceedings Management Team handles the bulk of external contact.
• Petitions to the government (Citizen Petitions, Laser Variance Applications etc.)
• Petition and other document comment management
• Federal Register related activities.
• Administrative Records Requests and Freedom of Information Act Requests
How Can We Help You?

• If it involves an administrative decision or will result in a regulatory change or clarification it will at some point involve Dockets Management. We house all of the Agency’s records of Administrative Decisions.

• Most commonly, our services to industry involve Records Requests (FOIA), Citizen Petition Processing, and Public Comment Management.
RECORDS

Docket Management Houses All FDA Administrative records and other records going back to the 1950s.

Rule of thumb: If you see it in the Federal Register and it has a FDA Docket Number we likely have the record.

Most records requests are handled very quickly.....
Citizen Petitions

- Citizen Petitions are the mechanism by which a person or organization may request agency administrative action. - Reference listed drug identities are a great example.

- Dockets Management will happily guide all submitters to ensure that they can adhere to format and content requirements for petitions.
Citizen Petitions

Regulations
• 21 CFR 10.20
• 21 CFR 10.30
• And others...

Content
• Action Requested
• Statement of Grounds
• Environmental Impact
• Economic Impact
• Certification
Comment Management

Whenever a Citizen Petition has been submitted, the public may provide their opinion.

Whenever the Agency releases a Guidance, Notice, or Final Rule, the public may have their say.

The Division of Dockets Management assists the public in their effort to be heard, we then use our resources to aid the Centers in compiling the various opinions that have been received.

Last Year, Dockets Management Processed over 100,000 documents, each generating uncountable public interest, from one comment to thousands depending upon the issue.
Where do I go for answers about submissions to dockets?
Get to know us!

Dynna Bigby
Supervisory Administrative
Proceedings Officer, Administrative
Proceedings Management Team
(APMT)
Dynna.bigby@fda.hhs.gov
(301) 796-0407

John Wright, JD
Dockets Management and FOIA
Specialist, Administrative Proceedings
Management Team (APMT)
John.wright@fda.hhs.gov
(240) 402-7507

• Believe it or not.....You can have our PHONE NUMBER!
  The Division of Dockets Management
  (240) 402-7500
Any
Questions......

John.wright@fda.hhs.gov

?
Questions and Answers
How to Get Your Voice Heard

Moderator:
Rea Blakey
Communications Policy Strategist & Engagement Team Lead, Professional Affairs and Stakeholder Engagement (PASE)

Panelists:
• Pujita Vaidya, M.P.H., Acting Director, Decision Support and Analysis Team, Office of Strategic Programs (OSP)
• Andrea Furia-Helms, M.P.H., Acting Director, Patient Affairs Staff (PAS), Office of Medical Products and Tobacco (OMPT)
• Salina Miller, M.S., M.B.A., Health Programs Coordinator, Office of Health and Constituent Affairs (OHCA)
Externally-Led Patient-Focused Drug Development Meetings

Pujita Vaidya, M.P.H.
Acting Director,
Decision Support and Analysis Team (DSAT),
Office of Strategic Programs (OSP), CDER
Externally-led Patient-Focused Drug Development Meetings

PUJITA VAIDYA, MPH
Acting Director, Decision Support and Analysis
Office of Program & Strategic Analysis
Office of Strategic Programs
FDA Center for Drug Evaluation and Research

CDER and You: Keys to Effective Engagement
April 2, 2018
Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients’ experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.
Externally-led PFDD: The Opportunity

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

- PFDD meetings provide an important opportunity to hear directly from patients, patient advocates, and caregivers about the symptoms that matter most to them, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments.

- While FDA will be open to participating in a well-designed and well-conducted meeting, an externally-led PFDD meeting and any resulting products (e.g., surveys or reports) will not be considered FDA-sponsored or FDA-endorsed.
Planning a PFDD meeting

**KEY PARTICIPANTS:**

Patients, patient representatives, patient advocates

**TARGET AUDIENCE (LISTENING MODE):**

Regulatory/other federal agencies, medical product developers, researchers, healthcare professionals

**DO NOT HAVE TO BE STANDALONE MEETINGS:**

Consider incorporating PFDD-style sessions in annual conferences, scientific workshops, etc.

**FDA-led meetings can serve as a model:**

- Target disease areas where there is an identified need for patient input on topics related to drug development
- Main discussion topics: (1) Symptoms and daily impacts that matter most to patients and (2) current approaches to treatment
- Facilitator-led large group discussion, interactive webcast, discussion aids (e.g., polling tools)
- Meeting deliverables: Web recording, transcript, summary report
Key Considerations

Submit a letter of intent to CDER’s Office of Strategic Programs. Our team is here to **serve as a helpful resource** to you.

While we truly understand the effort it takes to plan a PFDD meeting, it can be done **without being resource intensive**!

The key to an insightful, robust, and informative PFDD meeting is **active community outreach** to ensure a representative group of patient perspectives in the room.

We must be **respectful of the time of patients** and their caregivers.
Patient input from meetings can support FDA staff:
• In conducting benefit-risk assessments for products under review, by informing the therapeutic context
• Advising drug sponsors on their development programs

It might also support drug development more broadly:
• Identify areas of unmet need in the patient population
• Identify or develop tools that assess benefit of potential therapies
• Raise awareness and channel engagement within the patient community

Meeting summary reports capturing patient experience data may be shared on FDA’s website:
• FDA’s External Resources or Information Related to Patients’ Experience webpage provides links to certain publicly available external reports and resources.
This webpage is intended to facilitate public discussion of patient-focused drug development and evaluation. This webpage provides links to certain publicly available external reports and resources relating to patient experience data. The patient community, patient advocates, researchers, drug developers, and federal agencies may find these materials useful.

Please note that although FDA reviews the materials at these links before posting them to ensure that the materials are within the scope of the webpage, FDA does not assess their scientific merit or compliance with regulatory requirements. Our decision to post links to these materials does not reflect an endorsement of their authors, sponsors, or content.

For more information regarding what types of resources may be included on this webpage, how to submit a publicly available website link to FDA, and other general questions, please review our Frequently Asked Questions. We request that links include a cover page or similar opening statement as part of their report or resource to provide information about the authors, funding, and related information. For specific questions related to a report or resource, FDA recommends reaching out to the point of contact listed on this cover page.

Externally-led PFDD Meeting Reports or Other Stakeholder Meeting Reports

Notes:
- Proposing new patient-focused drug development initiatives
- Directing current patient-focused drug development initiatives
- Reviewing new patient-focused drug development initiatives
- Alternative to FDA guidance
- But not replacing FDA guidance
- All guidance is considered to be part of the “patient-focused drug development process”
CDER’s Patient-Focused Drug Development Homepage

Email: patientfocused@fda.hhs.gov
Patient Affairs Staff
Enhancing FDA Patient Engagement

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

• Established December 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
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 Engagement Collaborative (PEC)

Why PEC?

• FDA listened
  ✓ Public comments from patients and other stakeholders recommended that FDA create an outside group to provide input on patient engagement across the FDA

• The laws
  ✓ Facilitated by recent legislation in both the 21st Century Cures Act and FDARA for fostering patient participation and incorporating patient experiences in the regulatory process

• A model
  ✓ The European Medicines Agency’s Patients’ and Consumers’ Working Group (ECWaC)
Patient Engagement Collaborative (PEC)

Membership Criteria

• Patients who have personal disease experience

• Caregivers who support patients, such as a parent, child, partner, other family member, or friend, and who have personal disease experience through this caregiver role

• Representatives from patient groups who, through their role in the patient group, have direct or indirect disease experience.
Patient Engagement Collaborative (PEC)

- Next Steps
  - 200 nominations received (closed January 29, 2018)
  - Review and select members
  - Schedule first meeting (TBD)

For more information:
FDA Voice Blog December 20, 2017
You Spoke, FDA Listened: New Patient Engagement Collaborative, Call for Nominations
Patient Experience Listening Sessions
Patient Experience Listening Sessions

- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- Pilot listening sessions in rare diseases to enhance the incorporation of patient experience into regulatory discussions
- Assess value added to possibly expand
Patient Experience Listening Sessions

Next steps:
• Identify pilot therapeutic area
• Develop process with NORD
• Conduct pilot listening sessions
• Evaluate internal and external feedback
• Develop recommendations
Thank you

patientaffairs@fda.hhs.gov
FDA Patient Representation Program

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FDA Patient Representative Program℠

Salina Miller, M.S., M.B.A.
Director, FDA Patient Representative Program℠
Advisory Committee Oversight and Management Staff

April 3, 2018
FDA Patient Representative Program

- Began in 1990s and evolved.
- Patients having an active role on FDA Advisory Committees and in consultations with review divisions.
- Patient voice represented in important discussions about regulatory decision-making.
- Furthers an understanding and appreciation for FDA’s role in medical product development, review and patient protection.
- Presence at the table.
Experiences Represented

200 FDA Patient Representatives
300-500 diseases/conditions/device experiences

- AIDS/HIV
- Alzheimer’s Disease
- Asthma
- Cancer (various)
- Cardiovascular disease
- Cerebral Palsy
- Crohn's disease
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Diabetes
- Diabetes (insulin pumps)
- Fabry Disease
- Hepatitis B
- Hepatitis C
- Infantile Spasms
- Lung Transplantation
- Lupus
- Lysosomal Acid Lipase Deficiency
- Major Depressive Disorder
- Muscular Dystrophy
- Obesity/Weight Control
- Opioid Use
- Parkinson's Disease
- Pompe Disease
- Sickle Cell Disease
- Short Bowel Syndrome
- Temporomandibular joint disorder
- Transplantation
Becoming an FDA Patient Representative

- Personal experience with the disease or condition as either a patient or primary care giver.
- **Patient community awareness**: active in patient advocacy organizations, knowledgeable about treatment options and research, other advocacy activities.
- Someone who is **analytical and objective**, doesn’t need to be a scientist but should grasp scientific principles and understand issues, experienced with decision making based upon complex information.
- **Minimal or no conflict of interest**.
- **Good communications skills**.
- **Commitment** to serve.
Where Patient Reps Intersect with Drug/Biologic Development?

Patient Reps serve as Consultations (divisional assignments)

Drugs Developers

Basic Research/Discovery

Pre-IND

Clinical

NDA/BLA Review

Post-marketing

Advisory Committee

Patient Reps serve on FDA Advisory Committees
FDA Patient Representatives on Advisory Committee Meeting Panels

- Temporary voting members.
- Screened for each assignment.
- Other disciplines at the table.
- Across all medical product Centers.
- 40-60 assignments/year.
FDA Patient Representatives also serve...

...as consultants with review divisions:

- Brings the voice earlier in the regulatory process.
- Divisional “homework” assignments.
- Consult directly with scientific review staff and sponsor
- Closed meeting (telecon).

...at workshops

...on symposiums
We Train and Prepare for Service!!

- Describe significance of program
- Describe FDA regulatory framework and decision-making process (FDA 101)
- Share experiences: internal and peer
- Describe scenarios for the meeting
- Offer regular webinars (training modules)
- Provide online resources for patients
- Host Annual Patient Representative Workshop
FDA Patient Representative Program
2017 Training Workshop Cohort
CBER Patient Engagement

Diane Maloney, J.D.
Associate Director for Policy
Center for Biologics Evaluation and Research (CBER)
Disclaimer

My comments are an informal communication and represent my own best judgment. These comments do not bind or obligate FDA.
CBER AND PATIENT ENGAGEMENT
INTRODUCTION TO THE CENTER FOR BIOLOGICS EVALUATION AND RESEARCH
We are Listening

- Patients provide an important and unique perspective that is critical for consideration as part of the regulatory process
- We highly value patient engagement and its contribution to the development of biological products
Patient-Focused Product Development

• Evolving:
  – FDASIA, FDAMA, 21st Century Cures Act

• Sections 3001-3004 of the Cures Act
  • Patient Experience Data as part of a marketing application
  • Issuance of guidance documents addressing methodological approaches to collecting, analyzing, and submitting patient experience data
  • FDA to publish a report on patient experience data

Products Regulated by CBER

- Vaccines (preventative and therapeutic)
- Allergenics
- Live Biotherapeutic Products
- Blood Products
- Devices Related to Biologics
- Human Tissues and Cellular Products
- Xenotransplantation Products
- Gene Therapies
Types of CBER Meetings with Patient Involvement – Product Specific

– With Product Office/Review Team
  • Investigational Stage
  • May include IND Sponsor

– Advisory Committee Meetings
  • For specific issues during development
  • During BLA review
Types of CBER Meetings with Patient Involvement – Issue or Disease Specific

Advisory Committee Meetings

Public Meetings/Workshops
• Topics facilitate product development & regulation
• Usually collaborate with organizations & other agencies

Meetings with Patient Organizations

Patient Focused Drug Development Meetings
• Internally led
• Externally led
Contact Information

- **CBER website:**
  
  www.fda.gov/BiologicsBloodVaccines/default.htm

- **Phone:** 1-800-835-4709 or 240-402-8010

- **Consumer Affairs Branch:** ocod@fda.hhs.gov

- **Manufacturers Assistance and Technical Training Branch:** industry.biologics@fda.hhs.gov

- **Follow us on Twitter:** https://www.twitter.com/fdacber
Questions and Answers
AUDIENCE RESPONSE QUESTIONS

Christopher Melton
Health Communications
Specialist, Professional Affairs & Stakeholder Engagement
Learn from the Pros

Alexandra Kruse
Research Coordinator,
Platelet Disorder Support Association (PDSA)

Phyllis Foxworth
Vice President of Advocacy,
Depression and Bipolar Support Alliance (DBSA)
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Platelet Disorder Support Association

Alexandra Kruse

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Platelet Disorder Support Association (PDSA)
Platelet Disorder Support Association

Empowering ITP Patients

Engaging with the fda

1998
PDSA Founded

2008
ITP
Patients, PDSA Medical Advisors testify to FDA (ODAC) for approval of two new ITP therapies

2016
PDSA receives grant from NORD via the FDA for ITP Natural History Study Patient Registry
Attendance at 1st FDA Public Workshop

2017
ITP Registry Launches
FDA Workshop #2
FDA Workshop #3
Meeting with OHOP/PASE

2018+
FDA Workshop #4
FDA Workshop #...

Externally-led
Submission of Registry data?
Further testimonies for new ITP therapies?
Guidances?
Planning your meeting

1. INVOLVE KEY OPINION LEADERS: patients and caregivers, patient advocacy group, physicians, researchers

2. CLEARLY ESTABLISH GOALS:
   1. Educate the FDA on the most significant symptoms, current treatment side effects, burden of disease, and impact of condition on daily life.
   2. Ensure that the patient voice is included in providing guidance and advancing science.
   3. Serve as a comprehensive resource on the patient experience to provide input and guidance in new drug development research moving forward.

3. DEVELOP YOUR ASK: PRIORITIZE THE UNMET NEEDS OF PATIENT POPULATION

4. PROVIDE PATIENT EXPERIENCE DATA
TAKE-AWAYS: BENEFITS OF COLLABORATING WITH THE FDA

“Meetings are greatly enriched by the inclusion of patients with the condition... they provide the most valuable insights” - Theresa Mullin, Associate Director for Strategic Initiatives, CDER (3/19/18)

- **Involvement of all stakeholders**
- The FDA wants to include the patient perspective: help them to help you
- Have the right people in the room and ask the right questions: identify issues up-front that FDA should be addressing to maximize impact of meeting
- Encouraging to patient population that advocacy groups are collaborating with the agency
- Patients are able to express what matters most to them and take charge of their health
- Advocacy work is never done, follow up!

**BENEFITS OF ENGAGING EARLY AND OFTEN: ACCESS!**

Future opportunities to express to FDA what matters most to patients
Learn from the Pros
Phyllis Foxworth, Advocacy Vice President
Depression and Bipolar Support Alliance
DBSA Campaign Overview

**Identify Unmet Need**
- Current clinical trial endpoints focus on symptom reduction
- Patients report what is most important to them is improvement in domains that support functionality

**Utilize Resources**
- FDA: PACE, CDER
- White Paper: Describe unmet need and offer a path forward
- Mentors: Learn from others’ past experience

**Meaningful Output**
- Scientific Workshop: Convened all the stakeholders to explore patient defined wellness
- Externally-led PFDD Meeting: format for patients to share what outcomes are important to them
Collaborative Strategy

Key to successful campaign

Identify the Intersection Between the Needs of the Agency and the Community

FDA
U.S. FOOD & DRUG ADMINISTRATION

Patient Community
Develop an Attainable Strategy

FDA language:
- homogeneous dimensions and domains
- validated scales

More similarities than we might think

DBSA Role
Bridging the Gap

Patients language:
- what’s working, what’s not working in my life
- heterogeneous life circumstances
Questions and Answers
AUDIENCE RESPONSE QUESTIONS

John Whyte, M.D., M.P.H.
Director, Professional Affairs and Stakeholder Engagement
Final Words of Wisdom

John Whyte, M.D., M.P.H.

Director, Professional Affairs and Stakeholder Engagement (PASE), CDER
Thank You for Attending!

Safe Travels Home