

Patient Engagement: FDA Perspectives and Initiatives

Martha Donoghue, MD

REiNS Winter Meeting

December 5, 2016

I have nothing to disclose.

Outline

- Advantages of engaging patients in drug development
- Existing FDA programs and outcomes
- Future plans for increasing patient engagement through FDA
- Challenges to meaningful patient engagement

What is Patient Engagement?

- Refers broadly to obtaining patient perspectives at all stages of drug and device development
- Examples of strategies include:
 - Gathering patient advice and perspectives at meetings and public workshops
 - Obtaining written public comments
 - Acquisition of patient experience data generated through qualitative research or social media
 - Incorporation and analysis of clinical outcomes assessments data in clinical trials.



Goals of Patient Engagement in Drug Development

- More meaningful clinical trial endpoints
- Outcomes in alignment with patient needs
- More robust assessment of adverse events and post market surveillance
- Approvals of medical products that better reflect outcome and quality of life measures most important to patients
- FDA decisions that better reflect patient tolerance for risk



FDA Patient Representative Program

- Patients have an active role on FDA Advisory Committees and serve as consultants to offices across the agency
 - Over 200 patients and caregivers representing over 300 diseases and conditions
 - Special Government Employees (SGEs)
- Presence at the table

<http://www.fda.gov/ForPatients/About/ucm412709.htm>



Advisory Committees Meetings and Review Division Assignments

Year	Committee Meetings	Divisional Assignments
2012	27	4
2013	66	11
2014	55	12
2015	48	14
2016	34	3

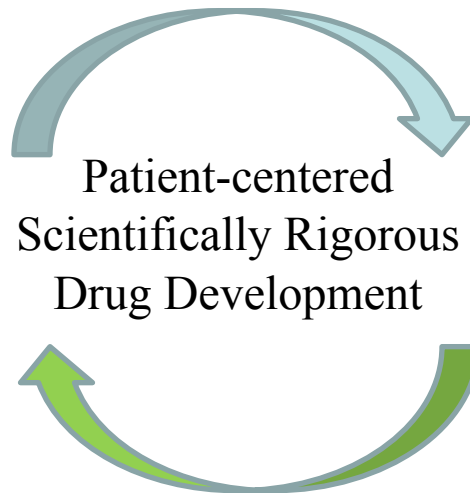
What is Patient Focused Drug Development?

- A systematic approach to incorporates the patient perspective throughout the drug development continuum
 - Translational
 - What symptoms or functions matter most to people with this disease?
 - How to best measure outcomes (frequency of assessments, mode of reporting, etc)?
 - Clinical studies
 - Do trial endpoints capture outcomes that matter to patients (including clinical outcomes assessments)?
 - Are the endpoints feasible?
 - Does the protocol facilitate enrollment and participation? How could it be improved?
 - Pre-market review
 - How can COA data be used in the risk:benefit assessment?
 - Post-market review
 - How best to convey information to facilitate informed clinician/patient/caregiver decision-making?

Successful Patient-Focused Drug Development Must be a Dialogue

Patients

- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly



Clinicians/ Trialists/ Health Policy Leaders

- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures

Patient-Focused Drug Development Program

- Part of FDA commitments under PDUFA V
 - FDA will conduct at least 20 meetings on specific disease areas FYs 2012 to 2017
 - Meetings help advance a systematic approach to gathering patients' input on their condition and treatment options
- The Program helps establish a therapeutic context
 - Patients are uniquely positioned to inform understanding of disease in broader context and scope
 - Patient representative input limited to specific marketing applications under review



<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm>

PDUFA = Prescription Drug User Fee Act

PFDD Meetings: Identifying Disease Areas

- Office of Strategic Programs worked with divisions in OND to identify candidate disease areas
 - Public input on these nominations was collected through a docket and at a public meeting held in October 2012
 - >4,500 comments were submitted, addressing 90+ disease areas
- Focused consideration on disease areas that:
 - Are chronic, symptomatic, affect functioning/daily activities
 - Currently have few or no therapies, or the available therapies do not directly affect how a patient feels or functions.
 - Have important aspects not formally captured in clinical trials
 - Have severe impact on identifiable subpopulations (e.g., children)
 - Represent a range in terms of size of the affected population

PFDD Meetings

- A ***Voice of the Patient*** report is prepared for each meeting
 - Includes an analysis of the condition and current treatment options
- Input can support FDA and industry:
 - Assessing benefit-risk for products under review
 - Advising sponsors on their drug development programs
- Input can support other aspects of drug development:
 - Help identify areas of unmet need
 - Develop clinical outcome tools (e.g., patient reported outcomes) that better address patient needs
- OHOP has participated in 3 PFDD meetings to date
 - Breast cancer (2015); Sickle cell disease (2014); Lung cancer (2013)





U.S. Food and Drug Administration (FDA)

**PATIENT-FOCUSED
DRUG DEVELOPMENT**

SICKLE CELL DISEASE

Attention sickle cell disease patients!
(Caretakers and advocates too)

**FDA WANTS TO
HEAR FROM YOU ABOUT
YOUR DISEASE AND
YOUR TREATMENTS**

YOU CAN CONTRIBUTE IN MANY WAYS:

- ✓ Attend the public meeting in person
- ✓ Watch the live meeting webcast
- ✓ Share comments through our website

**FDA PUBLIC
MEETING**

DATE:
February 7, 2014

TIME:
10 a.m. to 4 p.m.

LOCATION:
FDA White Oak Campus
10903 New Hampshire Ave.
Building 31, Great Room
Silver Spring, MD 20993

FOR MORE INFORMATION AND TO REGISTER

<https://patientfocussedicklecell.eventbrite.com>

or call (301) 796-5003

Registration closes on January 27th, 2014

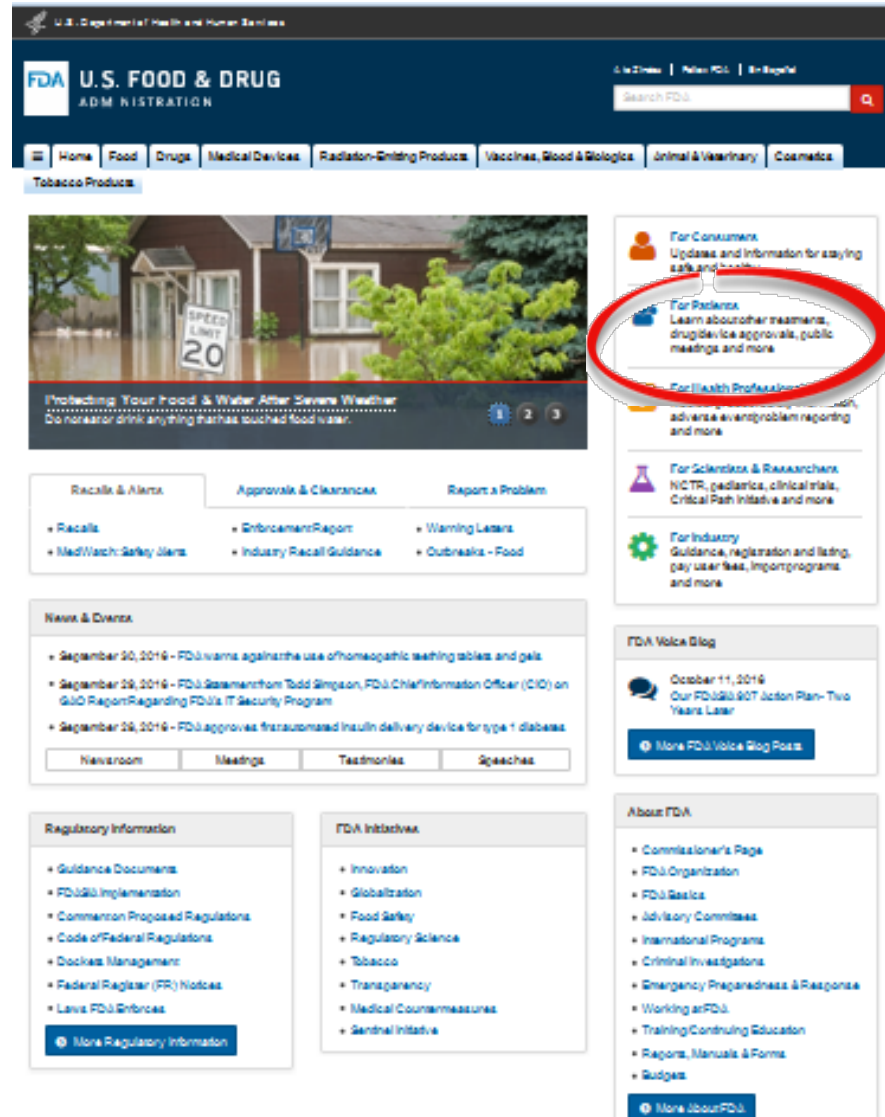


Patient Engagement Cluster



- Initiated June 2016
- Discuss strategies for encouraging sponsors to collect information from patients
- Compare patient/caregiver input and process for including patient/caregiver input in regulatory decisions between agencies
- Share input with sponsors to create meaningful changes
- Develop strategies to measure and report impact of patient engagement in both agencies

Individual patients, advocacy groups, and patient communities can take an active role



The screenshot shows the FDA website interface. At the top, there is a navigation bar with the FDA logo and the text 'U.S. FOOD & DRUG ADMINISTRATION'. Below this is a search bar and a menu with categories like Home, Food, Drugs, Medical Devices, etc. The main content area features a large banner for 'Protecting Your Food & Water After Severe Weather' and a grid of links for 'Recalls & Alerts', 'Approvals & Clearances', and 'Report a Problem'. On the right side, there is a vertical navigation menu with several categories: 'For Consumers', 'For Patients', 'For Health Professionals', 'For Scientists & Researchers', and 'For Industry'. A red circle highlights the 'For Patients' link, which includes the text 'Learn about other treatments, drug/device approvals, public meetings and more'. Below this menu are sections for 'FDA Voice Blog' and 'About FDA'.

Learn about and Participate in upcoming FDA Public Meetings

[Tobacco Products](#) | [Drugs](#) | [Medical Devices](#) | [Radiation-Emitting Products](#) | [Vaccines, Blood & Biologics](#) | [Animal & Veterinary](#) | [Cosmetics](#)

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Calendar of FDA Sponsored Public Meetings

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- Public Meeting: Progress Toward Implementing the Product Identification Requirements of the Drug Supply Chain Security Act**
 Date: October 17, 2016, 9:00 am to 4:00 pm
 Location: FDA White Oak Campus - 10903 New Hampshire Ave, Silver Spring, MD, 20993
 Agenda: The purpose of the meeting is to provide members of the pharmaceutical distribution supply chain and other interested stakeholders an opportunity to share information about current practices and industry efforts to implement the DSCSA's product identification requirements, including best practices. In each sector of the supply chain, the meeting involves presentations from the public and follow-up questions from an FDA panel. FDA will invite specific presenters; rather, with this notice, FDA is soliciting presentations from interested stakeholders. The main topic FDA is interested in discussing at the public meeting is the supply chain's progress toward implementing the DSCSA's product identification requirements, including best practices and product identification. This meeting is intended to enhance the public's understanding of the supply chain's progress and the FDA's role in the process, including allowing for verification, aggregation, and inference, as necessary. You may visit [www.fda.gov](#) to read Federal Register notices and to make comments electronically.

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Public Meeting

Advisory Committee meetings
 public workshops
 public conferences
 other FDA meetings

In this section you will find a comprehensive list of all the meetings that the FDA is involved with. The meetings may include advisory committee meetings, public workshops and public conferences that are seeking to hear from patients and caregivers.

Most FDA meetings are free to the public and do not require the public to register. Interested persons may present data, information, or views, orally at the meeting, or in writing, on issues pending before the committee. Other types of meetings listed may require prior registration and fees.

- Drug Advisory Committee Meeting: Bone, Reproductive and Urologic**
 Date: October 19, 2016, 8:15 am to 2:00 pm
 Location: FDA White Oak Campus - 10903 New Hampshire Ave, Silver Spring, MD, 20993
 Agenda: The committee will discuss the efficacy and safety of new drug application (NDA) 201456 (desmogresin) 0.75 mg/0.1 mL and 1.5 mg/0.1 mL nasal spray, submitted by Genentech Pharmaceuticals, LLC, for the proposed treatment of adult-onset nasozila.
- Public Meeting: Biotinilic Ular Fee Act**
 Date: October 20, 2016, 9:00 am to 2:00 pm
 Location: FDA White Oak Campus - 10903 New Hampshire Ave, Silver Spring, MD, 20993
 Agenda: The purpose of the meeting is to hear the public's views on the proposed recommendations for the authorization of the following information is provided to help potential meeting participants better understand the history and evolution of the GDUFA program and the proposed GDUFA II recommendations.
- Meeting: Generic Drug Ular Fee Act**
 Date: October 21, 2016, 9:00 am to 2:00 pm
 Location: FDA White Oak Campus - 10903 New Hampshire Ave, Silver Spring, MD, 20993
 Agenda: The purpose of this meeting is to discuss proposed recommendations for the reauthorization of the Generic Drug User Fee Amendments of 2012 (GDUFA), which authorizes FDA to collect fees and use them for the development of new generic human drug applications and associated Type II active pharmaceutical ingredients (API) (DMEFs), and for conducting associated inspections for fiscal years (FYs) 2016 through 2022. FDA's authority for GDUFA expires at the end of September 2017. In addition, new legislation will be introduced to continue to collect generic drug user fees for future fiscal years.
- Meeting: Health Canada and United States Food and Drug Administration Joint Public Meeting: International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH) Q10: Pharmaceutical Quality Management**
 Date: October 21, 2016, 1:00 pm to 2:00 pm
 Location: Sir Frederick G. Banting Research Centre, 251 Sir Frederick Banting Drive, Ottawa, Ontario, Canada

Submit comments through the Federal Register



Comment on Current FDA Draft Guidances



- + foods
- + drugs
- + biologics
- + cosmetics
- + radiation-emitting electronic products
- + medical devices
- + tobacco products

As a Regulatory agency, FDA publishes rules that establish or modify the way things are done. These rules are not created by chance but are based on the nation's health, industries and economy. These rules are not created by chance but are based on the nation's health, industries and economy. These rules are not created by chance but are based on the nation's health, industries and economy.

By law, anyone can participate in the rule-making process by commenting in writing. FDA routinely allows plenty of time for public input (typically 60 days) and carefully considers these comments when it drafts up a final rule. The public can submit comments about the proposed regulation directly to the agency (through the mail or online at www.regulations.gov).

On regulations.gov you can find, read, and comment on current FDA draft guidances and other FDA related documents. Your comments do make a difference and can impact the outcomes of FDA regulatory policy. Share your knowledge and experience and make your voice count.

Although you can comment on any guidance any time (see 21 CFR 10.112(g)(5)), to ensure that the agency considers your comment on a draft guidance before it begins work on the final version of the guidance, submit your electronic or written comments on the draft guidance before the close of date.

All written comments should be identified with the document number found in brackets in the heading of this document.

Opportunities to Comment Closing October 2016

The following topics were published in the Federal Register by the Food and Drug Administration (FDA). The intent of this page is to provide patients, caregivers and the general public an opportunity to provide their voice and expertise as a patient to the FDA during the review period. We have only provided you with a few paragraphs on each topic. If you want to learn more and submit comments, please click on "make comment" electronically.

On this page, you will find the following topics:

- + Draft Guidance: Voluntary Sodium Reduction Goals: Target Mean and Upper Bound Concentrations for Sodium in Commercially Processed, Packaged, and Prepared Foods due by October 17, 2016
- + Request for comment: Disinfectant Use Fee Act due by October 17, 2016

Request for comment by October 17, 2016: Draft Guidance - Voluntary Sodium Reduction Goals: Target Mean and Upper Bound Concentrations for Sodium in Commercially Processed, Packaged, and Prepared Foods

FDA has extended the comment periods for the Draft Guidance entitled, "Voluntary Sodium Reduction Goals: Target Mean and Upper Bound Concentrations for Sodium in Commercially Processed, Packaged, and Prepared Foods" that appeared in the Federal Register of June 2, 2016. In the notice, we requested comments on developing the sodium targets and for implementation of the guidance document. We are asking this action in response to requests to extend the two comment periods to allow interested persons additional time to submit comments.

Introduction from the original June 2, 2016 (FR) Posting:
 Many expert advisory panels have concluded that scientific evidence supports the value of reducing sodium intake in the general population (Ref. 1). Recent analyses, including the findings of the 2013 Institute of Medicine (IOM) report, "Sodium Intake in Populations: Assessments of Evidence" (IOM report), continue to support this conclusion (Ref. 2). The 2013 IOM report confirmed a positive relationship between higher levels of sodium intake and the risk of heart disease, and found substantial evidence of population benefit and no evidence of negative health effects associated with reductions in sodium intake down to 3,500 milligrams of sodium per day (mg/day) (Ref. 2). Members of the Institute of Medicine (IOM) report also clarified in a subsequent publication that different groups using a variety of methods and data have obtained results consistent with the committee's analysis that sodium intake in children, that should be reduced, and that reduction is expected to have a significant public health benefit (Ref. 3). The 2015 Dietary Guidelines Advisory Committee Sodium Working Group examined the relationship between sodium and blood pressure and other cardiovascular outcomes in adults, as well as sodium and blood pressure in children. The Committee's recommendations concurred with previous reports that sodium intake among children remains high and that higher levels of sodium intake are associated with increased blood pressure and risk of cardiovascular disease. To read the entire introduction and the background statement from the Federal Register Notice and to view the entire August 20, 2016 Federal Register Notice and to make comments, click on the link below.



Patient Network News

from the FDA Office of Health and Constituent Affairs

Volume 6 | Number 21 | October 12, 2016



- Home
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- Drugs
- Medical Devices
- Radiation-Emitting Products
- Vaccines, Blood & Biologics
- Animal & Veterinary
- Cosmetics
- Tobacco Products

For Patients

Home > For Patients



Patient Network Newsletter



A bi-weekly newsletter for patients and caregivers that helps you stay up-to-date on FDA related information.

Sign Up for the Patient Network Newsletter

[View the September 28, 2016 Patient Network Newsletter](#)

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Spotlight

- [View the September 28, 2016 Patient Network Newsletter!](#)
- [Our 20th Patient-Focused Drug Development meeting: Enhancing the patient's voice in FDA's approach to drug review and development](#)
- [October 22, 2016 - National Prescription Drug Take-Back Day](#)
- [Listen to webinars with FDA experts](#)

FDA continues to increase efforts to engage with patients

FDA Patient Network: Twitter

@FDA_Patient_Net



U.S. FDA ✓
@US_FDA



Join for a Twitter chat on lymphoma next week.

FDA Patient Network @FDA_Patient_Net
Join @FDA_Patient_Net and @lymphoma twitter chat #FDALRFChat on September 27 - 1:00 pm ET [twitter.com/lymphoma/statu...](https://twitter.com/lymphoma/status...)

Meetings with Patient Advocates



Meetings between patients and advocacy organizations and various agency components to discuss issues of importance to patients



Public Meeting: Childhood Cancer Advocacy Forum 2016

The FDA Offices of Hematology and Oncology Products, and Health and Constituent Affairs invite you to participate in a FDA Outreach to the Pediatric Cancer Advocacy

DATE:

Held on Friday April 22, 2016

TOPICS FOR DISCUSSION:

Update of cancer drugs approved for pediatric use, BPCAWR study results which have informed product labeling, PREA and iPSPs for oncology drugs- impact on issuance of WRs, Expanding patient-focused drug development to children with cancer and Pediatric PROs, Expanded Access to investigational drugs, Expanding Eligibility Criteria for clinical trials to accommodate early evaluation of certain products in children, and promising new Vaccine and Engineered Cell Products for cancer.

PDUFA Reauthorization Performance Goals And Procedures FYs 2018 through 2022

"Enhancing Benefit-Risk Assessment in Regulatory Decision-Making

*FDA will further the agency's implementation of structured benefit-risk assessment, **including the incorporation of the patient's voice in drug development and decision-making**, in the human drug review program through the following commitments to be accomplished during PDUFA VI..."*

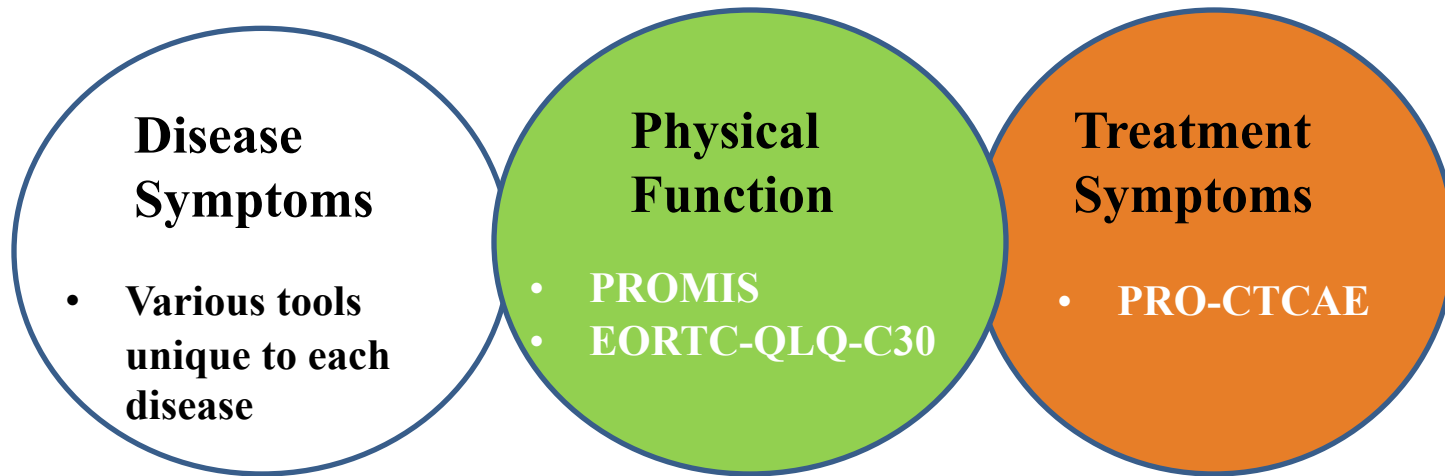
Patient Engagement, OHOP Initiatives

- Peds cancer advocacy community public meetings 2014, 2016
- Lung cancer, breast cancer, sickle cell disease public meetings
- Pediatric subcommittee of the ODAC meetings followed by workshops on relevant topics
 - Developing anticoagulants for pediatric patients
 - PROs in oncology trials
 - Brainstem biopsies in patients with diffuse intrinsic pontine glioma
- Patient representative SGE consults requested on many applications
- Patient representatives are invited to relevant symposia conducted through OHOP

PRO Assessment in Oncology

- Identify Core PRO Measures
 - Relevant to context, responsive to drug
- Identify/Validate Instruments
- Optimize Trial Design and Conduct
 - Increased engagement with FDA COA and Statistical reviewers
 - Providing consistent advice to sponsors
- Commitment to review PRO data as part of risk:benefit assessment in marketing applications
- Standardize Data Analysis and Presentation
- Identify Venues to Get the Information to Patients and Prescribers

Core measures, tools and trial design & conduct



- Optimize the frequency and timing of assessments
- Include procedures for minimizing missing data
- Provide a pre-specified plan for the analysis of PRO data including the threshold for and interpretation of a meaningful change in score(s).
- Carefully record the use of concomitant medications that may affect the interpretation of the concept(s) being measured

Challenges to Meaningful Patient Engagement

- Understanding of trial design
(meaningful endpoints and data, measuring outcome, control arms)
- Understanding the regulatory framework, standards, and requirements (level of evidence)
- Legal and practical limitations facing sponsors
(promotion v. education and engagement)
- Division within patient communities
- Different objectives or agendas among organizations
- Disagreement on meaningful measurement



Conclusions

- FDA is committed to increasing patient engagement in the drug development process
- There are ongoing FDA efforts and new initiatives to engage patients in a meaningful way
 - Patient representative/SGE program
 - PFDD disease-specific meetings
 - Public meetings/workshops/joint projects
 - Careful use and interpretation of COA assessments
- Ultimate goal: facilitate development of medical products that meet patient needs and improve methods to provide information to patients and healthcare providers to optimize medical decision-making
- PFDD is an evolving, iterative process and much work needs to be done

Acknowledgements

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- Patricia Keegan, Paul Kluetz, and Denise Casey, Office of Hematology and Oncology Products

Thank you!