U.S. FOOD & DRUG ADMINISTRATION

Public Workshop on Patient Focused Drug Development

Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data

March 19, 2018

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#PFDD

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Welcome

Pujita Vaidya, MPH
Office of Strategic Programs
Center for Drug Evaluation and Research

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Opening Remarks

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
Center for Drug Evaluation and Research

Today's workshop will inform FDA implementation of statutory requirements for issuance of new guidance

21st Century Cures Act of 2016

21st CC Act Section 3001: Patient Experience Data

STATEMENT OF PATIENT EXPERIENCE IN GENERAL.—**Following the approval** of an application that was **submitted** under section 505(b) of this Act or section 351(a) of the Public Health Service Act **at least 180 days after the date of enactment** of the 21st Century Cures Act, the Secretary shall **make public a brief statement regarding the patient experience data and related information**, if any, submitted and reviewed as part of such application.

The data and information referred to in paragraph (1) are—(A) patient experience data; (B) information on patient-focused drug development tools; and (C) other relevant information, as determined by the Secretary.

PATIENT EXPERIENCE DATA

For purposes of this section, the term 'patient experience data' includes data that are collected by any persons (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and are intended to provide information about patients' experiences with a disease or condition, including—

(A) impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy or clinical investigation*; and

(B) patient preferences with respect to treatment of such disease or condition.

* As amended by FDARA Section 605

21st CC Act Section 3002: Patient-Focused Drug Development Guidance

PUBLICATION OF GUIDANCE DOCUMENTS. Secretary shall… issue draft and final versions of one or more guidance documents, over a period of 5 years, regarding the collection of patient experience data, and the use of such data and related information in drug development.

Guidance documents shall address—

1. Methodological approaches that a person seeking to collect patient experience data for submission to, and proposed use by, the Secretary in regulatory decision making may use, that are relevant and objective and ensure that such data are accurate and representative of the intended population, including methods to collect meaningful patient input throughout the drug development process and methodological considerations for data collection, reporting, management, and analysis;

2. Methodological approaches that may be used to develop and identify what is most important to patients with respect to burden of disease, burden of treatment, and the benefits and risks in the management of the patient's disease;

3. Approaches to identifying and developing methods to measure impacts to patients that will help facilitate collection of patient experience data in clinical trials;

21st CC Act Section 3002: Patient-Focused Drug Development Guidance

Guidance documents shall address—*(cont.)*

4. Methodologies, standards, and technologies to collect and analyze clinical outcome assessments for purposes of regulatory decision making;

5. How a person seeking to develop and submit proposed draft guidance relating to patient experience data for consideration by the Secretary may submit such proposed draft guidance to the Secretary;

6. The format and content required for submissions under this section to the Secretary, including with respect to the information described in paragraph (1);

7. How the Secretary intends to respond to submissions of information described in paragraph (1), if applicable, including any timeframe for response when such submission is not part of a regulatory application or other submission that has an associated timeframe for response; and

8. How the Secretary, if appropriate, anticipates using relevant patient experience data and related information, including with respect to the structured risk-benefit assessment framework described in section 505(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(d)), to inform regulatory decision making.

PDUFA VI (FDARA Title I) : Enhancing Incorporation of Patient's Voice in Drug Development and Decision-Making

Commitments:

- Conduct public workshops and develop series of guidance documents on:
  - Collecting comprehensive patient-community input on burden of disease and current therapy (FY18)
  - Development of holistic set of disease or treatment impacts most important to patients (FY19)
  - Development of measures for an identified set of impacts (FY20)
  - Clinical outcome assessments and better ways to incorporate COAs into endpoints (FY21)
- Revise MAPPs and standard operating procedures and policies (SOPPs) as needed to incorporate increased patient focus
- Repository of info on publicly available tools and ongoing efforts
- Conduct public workshop to gather experiences and recommendations of patients and caregivers on approaches to enhance engagement in clinical trials (FY19)
- Enhance staff capacity to facilitate development and use of patient-focused methods to inform drug development and regulatory decisions

Some Key Topics to be Addressed in the PDUFA VI Guidance

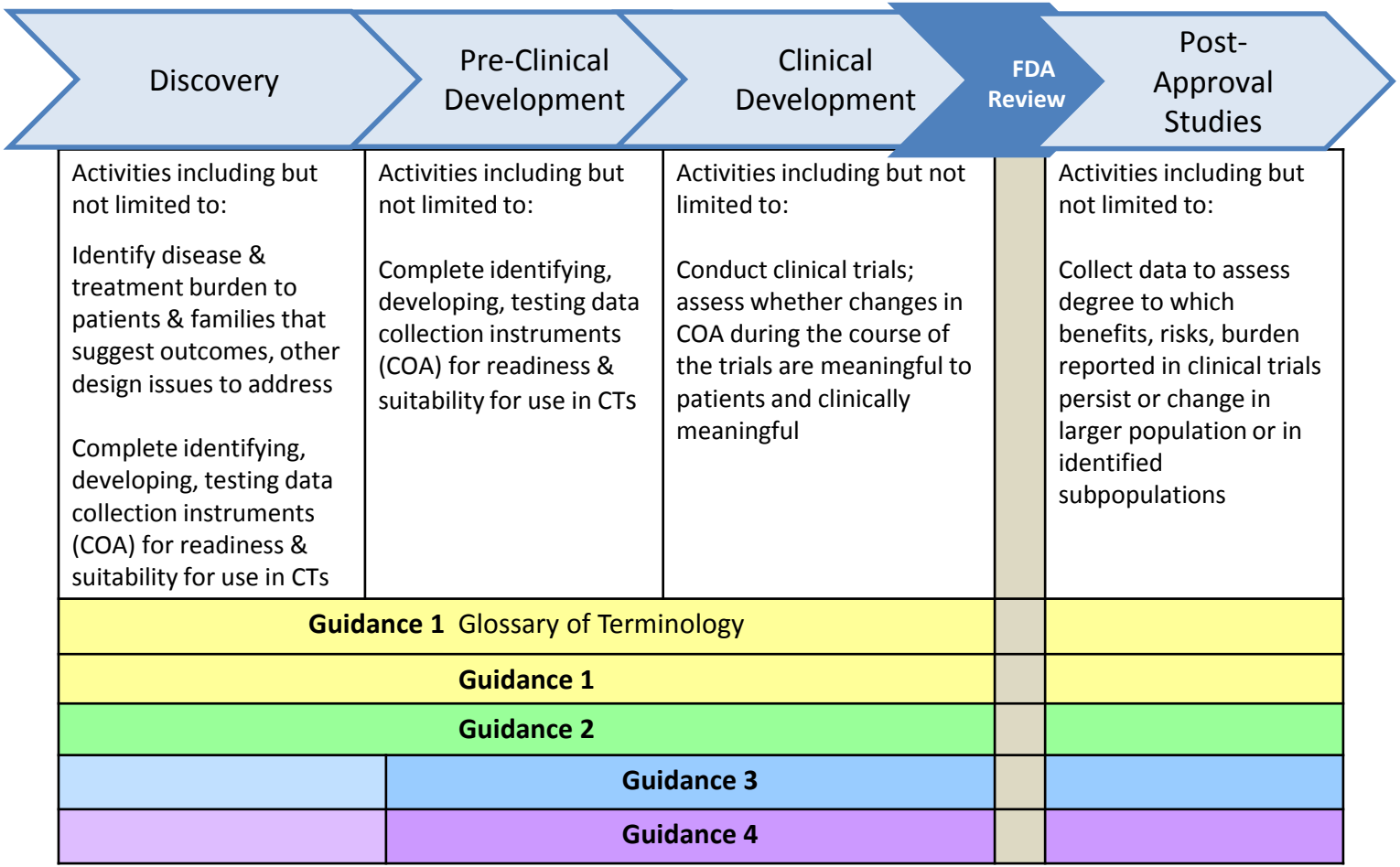
1. Collecting comprehensive patient community input on burden of disease and current therapy
   - How to engage with patients to collect meaningful patient input?
   - What methodological considerations to address ?

2. Development of holistic set of impacts (e.g., burden of disease and burden of treatment) most important to patients
   - How to develop a set of impacts of the disease and treatment?
   - How to identify impacts that are most important to patients?

3. Identifying and developing good measures for the identified set of impacts that can then be used in clinical trials.
   - How to best measure impacts (e.g., endpoints, frequency..) in a meaningful way?
   - How to identify measure(s) that matter most to patients?

4. Incorporating measures (COAs) into endpoints considered significantly robust for regulatory decision making
   - Topics including technologies to support collection through analysis of the data

When would the methods addressed in these guidances be applicable?



21st Century Cures Act	CY 2017	CY 2018	CY 2018	CY 2019	CY 2019	CY 2020	CY 2020	CY 2021	CY 2021
Provisions of Section 3002	**Q4**	**Q2**	**Q4**	**Q2**	**Q4**	**Q2**	**Q4**	**Q2**	**Q4**
(1) methodological approaches that a person seeking to collect patient experience data for submission to, and proposed use by, the Secretary in regulatory decision making may use, that are relevant and objective and ensure that such data are accurate and representative of the intended population, including methods to collect meaningful patient input throughout the drug development process and methodological considerations for data collection, reporting, management, and analysis; (6) the format and content required for submissions	Q4 2017: Public Workshop	Q2 2018: Draft Guidance			Q4 2019: Final guidance				
(2) methodological approaches that may be used to develop and identify what is most important to patients with respect to burden of disease, burden of treatment, and the benefits and risks in the management of the patient's disease; (6) the format and content required for submissions			Q4 2018: Public Workshop	Q2 2019: Draft Guidance			Q4 2020: Final guidance		
(3) approaches to identifying and developing methods to measure impacts to patients that will help facilitate collection of patient experience data in clinical trials; (6) the format and content required for submissions					Q4 2019: Public Workshop	Q2 2020: Draft Guidance			Q4 2021: Final guidance
(4) methodologies, standards, and technologies to collect and analyze clinical outcome assessments for purposes of regulatory decision making; (6) the format and content required for submissions				Q2 2019: Public Workshop		Q2 2020: Draft Guidance			Q4 2021: Final guidance
(5) how a person seeking to develop and submit proposed draft guidance relating to patient experience data for consideration by the Secretary may submit such proposed draft guidance to the Secretary;		Q1 2018: Draft Guidance			Q3 2019: Final guidance				
(6) the format and content required for submissions under this section to the Secretary, including with respect to the information described in paragraph (1); SEE guidance for (1) through (4) above									
(7) how the Secretary intends to respond to submissions of information described in paragraph (1), if applicable, including any timeframe for response when such submission is not part of a regulatory application or other submission that has an associated timeframe for response; and					Q4 2019 : Draft Guidance		Q3 2020: Final Guidance		
(8) how the Secretary, if appropriate, anticipates using relevant patient experience data and related information, including with respect to the structured risk-benefit assessment framework described in section 505(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(d)), to inform regulatory decision making				Q2 2019: Public Workshop		Q2 2020: Draft Guidance			Q4 2021: Final guidance

https://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm563618.pdf

Progress Update

- Public Workshop on Guidance 1 "Collecting Comprehensive and Representative Input"
  - Workshop held December 18, 2017
  - Discussion Document https://www.fda.gov/downloads/Drugs/NewsEvents/UCM586195.pdf
- Draft Guidance 1 "Collecting Comprehensive and Representative Input"
  - Target publication in June 2018
- Launch website for Externally-Submitted Information Resources re: Patient Experience Data - January 2018
  - Our new homepage https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm579400.htm
  - Our new external resources page https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm579132.htm
- Public workshop on 21st CC Section 3002 (c)(5) "Developing and submitting proposed guidance relating to patient experience data for FDA consideration"
  - March 19th 2018 https://www.fda.gov/Drugs/NewsEvents/ucm582081.htm

Thank you

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CDER'S External Resources and Information Related to Patients' Experience Webpage

Pujita Vaidya, MPH
Office of Strategic Programs
Center for Drug Evaluation and Research

TOPICS TO COVER

1. What is Patient Experience Data?
2. Overview of CDER External Resource Webpage
3. Categories of External Resources
4. Frequently Asked Questions
5. Cover Page Guidelines

Patient Experience Data* (PED)

....data that are:

- **collected by any persons** (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers)
- intended to provide information about **patients' experiences with a disease** or condition, including—

  (A) impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy or clinical investigation; and

  (B) patient preferences with respect to treatment of such disease or condition.

*This definition is from the 21st Century Cures Act: https://www.congress.gov/114/plaws/publ255/PLAW-114publ255.pdf

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External Resources or Information Related to Patients' Experience

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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

Disclaimer: Our website has links to other organizations. You should be aware that:

- This graphic notice, means that you are leaving the U.S. Food and Drug Administration (FDA) site and entering a non-federal website.
- This external link provides additional information that is consistent with the intended purpose of the FDA site.
- The FDA cannot attest to the accuracy of information provided by this link.
- Linking to a non-federal site does not constitute an endorsement by FDA or any of its employees of the sponsors or the information and products presented on the site.
- You will be subject to the destination site's privacy policy when you leave the FDA site.

- Intended to **facilitate public discussion on** patient-focused drug development and evaluation
- Provides **links to certain publicly available** external reports and resources
- Serves as **resource** for patient community, patient advocates, researchers, drug developers, and federal agencies
- FDA reviews submissions to ensure materials are within scope -- **does not assess scientific merit or compliance with regulatory requirements**
- FDA decision to post links to these materials **does not reflect an endorsement**

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Externally-led PFDD Meeting Reports or Other Stakeholder Meeting Reports

To help expand the benefits of FDA's Patient-Focused Drug Development (PFDD) initiative, FDA welcomes patient organizations to identify and organize patient-focused collaborations to generate public input on other disease areas. Submitted links to summary meeting reports from these externally-led PFDD meetings may be found here. FDA also welcomes submission of links to meeting reports from other stakeholder meetings collecting patient perspectives on disease burden and treatment burden.

- Amyloidosis
  In November 2015, the Amyloidosis Research Consortium hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with systemic amyloidosis and their loved ones on the impact of amyloidosis on their daily lives, and their perspectives on approaches to treating amyloidosis.
- Friedreich's Ataxia
  In June 2017, the Friedreich's Ataxia Research Alliance hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with Friedreich's Ataxia and their loved ones on the impact of Freidreich's Ataxia on their daily lives, and their perspectives on approaches to treating Friedreich's Ataxia.
- Tuberous Sclerosis Complex
  In June 2017, the Tuberous Sclerosis Alliance hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with tuberous sclerosis complex and their loved ones on the impact of tuberous sclerosis complex on their daily lives, and their perspectives on approaches to treating tuberous sclerosis complex.

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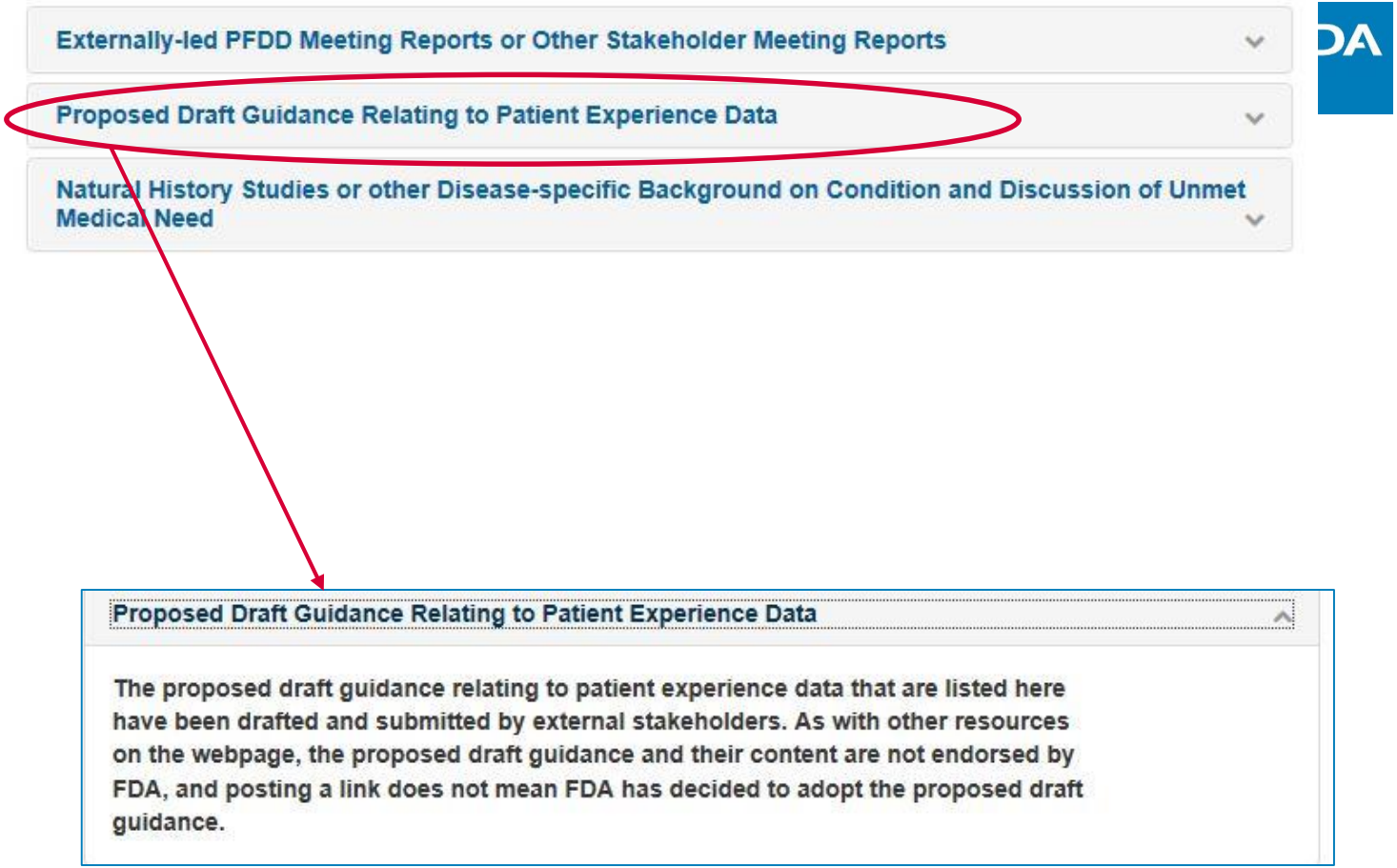

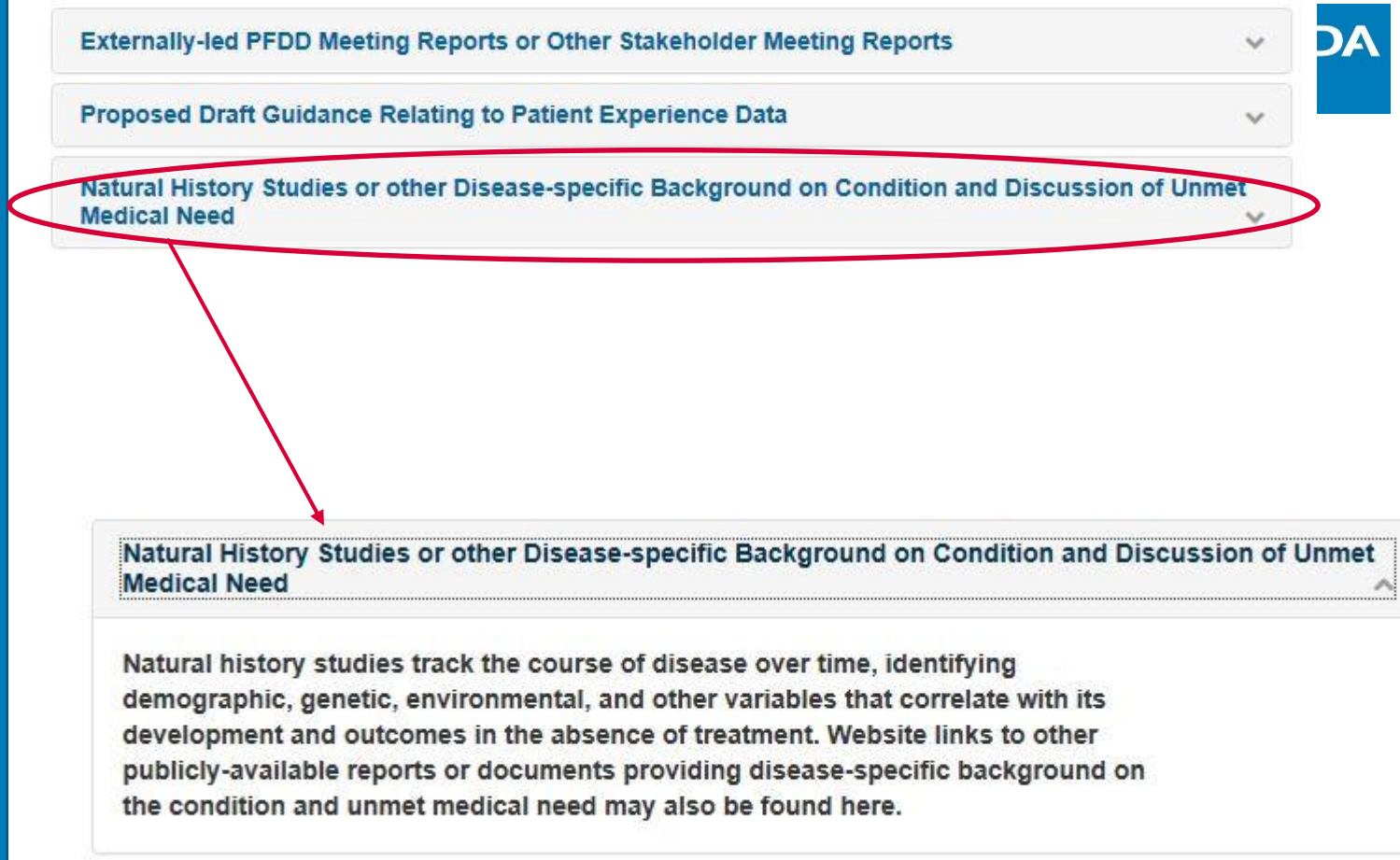
Externally-led PFDD Meeting Reports or Other Stakeholder Meeting Reports
Proposed Draft Guidance Relating to Patient Experience Data
Natural History Studies or other Disease-specific Background on Condition and Discussion of Unmet Medical Need
Natural History Studies or other Disease-specific Background on Condition and Discussion of Unmet Medical Need
Natural history studies track the course of disease over time, identifying demographic, genetic, environmental, and other variables that correlate with its development and outcomes in the absence of treatment. Website links to other publicly-available reports or documents providing disease-specific background on the condition and unmet medical need may also be found here.

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Cover Page Guidelines

FAQs are available for more information on scope and process

- What is Patient Experience Data?
- Who can provide a publicly available website link to a report or other resource?
- What types of resources will be included on this webpage?
- What types of resources will not be included on this webpage?
- How can you submit a publicly available website link to FDA?

Please include a cover page to promote **transparency**

- Title of resource
- Author(s) or Collaborator(s)
- Funding received or granted (if any)
- Version date
- Statement that the resource has not been revised or modified
- Statement that the resource can be linked from the FDA website

Submit Resources to:
PFDDresources@fda.hhs.gov

For more information visit:
CDER's Patient-Focused Drug Development Homepage

If you have questions, please email:
patientfocused@fda.hhs.gov

U.S. FOOD & DRUG ADMINISTRATION

Session I: Opportunities for Patient Stakeholders

FDA PERSPECTIVE

Moderator: Sara Eggers, PhD
Office of Strategic Programs
Center for Drug Evaluation and Research

Panelists

- **Larissa Lapteva**, Office of Tissues and Advances Therapies, CBER
- **Naomi Lowy**, Office of Drug Evaluation I (ODE I), Office of New Drugs (OND), CDER
- **Susan McCune**, Office of Pediatric Therapeutics, Office of the Commissioner
- **Laurie Muldowney**, Office of Translational Sciences, CDER
- **Theresa Mullin**, Office of Center Director, CDER
- **Elektra Papadopoulos**, Clinical Outcome Assessments Staff, OND, CDER
- **Ellis Unger**, ODE I, OND, CDER

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Session I

PRESENTATION ON OPPORTUNITIES

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
Center for Drug Evaluation and Research

Opportunities for Complementary Efforts

- In parallel with FDA's expanded efforts (e.g., PDUFA VI commitments and 21st CC requirements) related to "patient focused drug development" many patient and disease advocacy groups have substantially increased their own efforts related to patient focused drug development.
- A number external stakeholders (including ones supported or led by regulated industry) have indicated a strong interest in helping FDA.
- Where are there good opportunities?

Potential Capabilities That External Groups May Offer

- Patient/disease advocacy groups may have good access to the following sources of expertise:
  - Patients who are living with the disease, and their caregivers
  - Clinical disease experts (who may also have experience conducting trials)
  - Academic experts in the disease or relevant research methodologies
  - Drug developers with particular interest and experience in this disease area
  - Communications and outreach expertise with this disease community
  - Awareness of other issues affecting patient access –to Clinical Trials, to approved therapies, etc.

Some Potential Areas for Contributions

- Support research
  - E.g., advocating for increased funding for research
- Help people with the disease
- Natural history development
  - Informs future research and can provide basis for recruitment for clinical trials (being clinical trial ready)
- Formation of Centers of Excellence in study and treatment of disease
- Venture philanthropy– to push best treatment in development "over the finish line"

Some Potential Areas for Contributions (cont)

- Policy participation and response
  - In FDA-convened meetings and workshops and FDA request for comment
- Coordination
  - Of work among different patient advocacy groups –work to align efforts in a given disease area
- Communication, Education and Outreach
  - Gather input from and help inform patient community
- Convene meetings and workshops
  - Help further advance understanding of patient perspectives, and discussion and development of identified issues
- Contribute to guidance
  - Submit proposed new or enhancement to existing guidance

External Stakeholder Participation and Response

Participation in FDA-convened meetings and workshops and Response to FDA request for comment

- FDA will be planning many meetings related to PFDD issues.
- These meetings would be greatly enriched by inclusion of patients with the identified condition, and also researchers and clinicians with expertise in studying or treating the condition.
- Patient/disease advocacy groups often play a critical role in helping identify potential meeting participants.
- They may also be able to help identify issues to be covered in the meeting, and can help conduct outreach to potential participants, especially for meetings open to the general public—increasing the value and impact of the meeting.

Patient Disease Advocacy Group Coordination

Coordination of work among different patient advocacy groups e.g., to align efforts in a given disease area

- The large number and limited resources of many patient groups can create challenges with avoiding duplicative or conflicting efforts to advance work in the same disease area.
- Patient/Disease Advocacy Groups can help align efforts within a given disease area.
- Groups with a national perspective, more resources, and greater capacity may be able to help coordinate work, support information sharing, and help smaller groups maximize their contribution to shared interests and outcomes in a disease area

Communication, Education and Outreach

Patient/disease advocacy groups may have capabilities to conduct surveys, conduct outreach, and communicate to their patient communities and other key audiences in their networks.

Groups may be able to survey the patient community to collect more comprehensive and representative input about patients' experiences living with their disease, using available treatments, and accessing and participating in clinical trials.

These external groups are well positioned to help educate their communities about drug development

- The types of research and testing needed to develop and manufacture safe and effective treatments, including research risks and uncertainties
- Timeframes typically required for development, phases of clinical development, when to get involved, tools available, and how to help

Groups' outreach and messaging may help advance culture change that may be needed to further integrate the patient's voice in drug development work of academic researchers, global regulators, regulated industry, and into health care delivery

Convening Meetings and Workshops

External Stakeholders can also convene meetings and workshops to further advance understanding of patient perspectives, and discussion and development of identified issues. This can help to inform new policy and practice.

- Externally-led PFDD meetings and related meeting reports can help advance and augment FDA, industry and community understanding of the burden of disease and burden of currently available treatments
  - FDA-committed guidance under PDUFA VI will provide stakeholders with the tools needed to build even further on the findings of such PFDD meetings
- External groups with more scientific and technical capabilities might convene workshops focused on issues that pose scientific and technical uncertainty which pose barriers to success in clinical development, clear regulatory standards and guidance.
  - Such technical workshops may convene experts from government, industry and academia together to address specific scientific/technical issues that may need further structuring or identification and articulation of the issues that need further research and data collection
  - Workshops can also provide an effective venue for surfacing issues or limitations of current guidance that current users are well-positioned to identify

Contributing to Guidance

- Patient/disease advocacy groups may be able to contribute to regulatory guidance
- FDA disease (clinical) guidance may provide more general treatment of issues related to drug development for a given disease and with broad coverage of subpopulation issues.
- Similarly methodological guidance may provide general treatment of methodological issues and cover a range of research study settings, patient populations, cultural contexts, sociodemographic considerations.
- Patient/Disease Advocacy Groups may be able to contribute:
  - Developing specific "use cases" or scenarios tailoring the general clinical guidance to address considerations related to a specified subpopulation or special issues defined by factors such as patient age, disease severity, co-morbidities, and other concerns.
  - Developing examples of how specific methods recommended in guidance would be applied --or would not be applicable and alternatives would be better-- in specific study settings, for specified patient subpopulations, including variations in economic and cultural context, language ability, literacy, numeracy, mobility, age group, etc.

Examples of Questions Patient/Disease Advocacy Groups May be Well-Positioned to Help Address

- What disease impacts matter most to patients?
  - How does that vary by socio-demographic factors? By subgroup group of patients (e.g., a pediatric subpopulation, geriatric subpopulation, subpopulation with major co-morbidities), by culture? Severity of disease? Other life circumstances?
- How do attitudes toward or tolerance of potential drug risks or therapy side effects ("preference" considerations) vary by patient subgroup?
  - By subgroup group of patients (e.g., a pediatric subpopulation, geriatric subpopulation, subpopulation with major co-morbidities), by culture? Severity of disease? Other life circumstances?
- How well do the most commonly studied endpoints in clinical trials for a given disease area align with outcomes or aspects of disease that matter most to patients?
  - How does that vary by subgroup?
- Are currently conducted clinical trials in a given disease area excluding patients who want to be enrolled? If so, why and how might it be addressed?

Examples of Questions Patient/Disease Advocacy Groups May be Well-Positioned to Help Address (cont)

- Are currently or commonly used clinical trial protocols intolerable or otherwise unworkable for some patients who are otherwise eligible to participate?
  - Why? What might be done to address that?
- What measures can be taken to increase the likelihood of patient enrollment in a study and increase the likelihood of participant retention in a study in a given disease area?
  - Are there further suggested considerations by patient subgroup?
- What if any challenges do patients face in trying to adhere to their prescribed drug regimen?
  - How does this vary by patient subgroup? What might be considered to address this?
- How well is currently approved labeling communicating the information that patients need to know in order to use drugs safely and most effectively?
  - How does this vary by demographic subgroup, by health literacy, by other relevant patient characteristics? What might be done to address any identified gaps or challenges?

U.S. FOOD & DRUG ADMINISTRATION

Session I

PRESENTATION ON GUIDANCE DEVELOPMENT

Keith Flanagan, J.D.
Transition Lead for Policy, Office of New Drugs
Center for Drug Evaluation and Research

Background on Guidance Development

- What is guidance?
- Substantively, what type of information is most useful and relevant for guidance development?
- Procedurally, what are the main windows for patient/disease advocacy groups to provide input concerning guidance?
- Practical considerations.
- FYI -- OND "Bulleted" Guidance Pilot.

What is guidance?

- A guidance document represents the Agency's current thinking on a regulatory issue.
- Guidance is prepared for FDA staff, industry, external stakeholders and the public.
- FDA issues a draft, considers public comments, then finalizes a guidance.
- Guidance may be updated as the science in a field progresses.
- Guidance is not legally binding, but shows one way to achieve the regulatory goal.
- Industry may take an alternative approach that complies with relevant statutes and regulations.
- FDA may depart from guidance documents with appropriate justification and supervisory concurrence.

Substantively, what type of information is most useful and relevant for guidance development?

In general, information that can bring a patient's perspective to specific drug development and regulatory challenges.

- Dr. Mullin, the previous FDA speaker, provided additional background and context.
- Next, FDA panelists will discuss types of patient experience data that are most useful and relevant for guidance development.

Procedurally, what are the main windows for patient/disease advocacy groups to provide input concerning guidance?

- Suggest that FDA revise or withdraw existing guidance documents.
  - This is not the focus of today's discussion, but it's important.
  - Sometimes, instead of charting a new path forward, we need to refine or update our prior work.
- Comment on draft guidance that FDA has issued.
- Submit drafts of proposed guidance documents for FDA to consider.
- Suggest specific issues on which FDA should undertake guidance development, explain why a guidance document is needed, and – ideally -provide information that would be useful and relevant in its development.

Practical Considerations

- FDA's role and responsibilities.
- Novelty, complexity and difficulty.
- Legal and regulatory issues, issues related to specific submissions.
- Occasional delays or omissions.
- You may want to know what is going on in FDA's internal deliberations, but:
  - We lack capacity to provide status reports on demand.
  - We can't communicate policy to one party before another.
  - We need to follow certain procedures before communicating official policies to the public.

OND "Bulleted" Guidance Pilot

The Office of New Drugs has undertaken a pilot project to develop and issue "bulleted" guidance rapidly.

- Bullet points on critical elements of drug development.
- OND is strongly committed to expanding our issuance of disease- and indication-specific guidance – e.g., Division of Neurology Products recently developed five.
- Focus on critical elements streamlines guidance development.
- Patient experience data can be very helpful.

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Session I

MODERATED PANEL DISCUSSION

Moderator: Sara Eggers, PhD
Office of Strategic Programs
Center for Drug Evaluation and Research

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Session II: Opportunities for Patient Stakeholders

STAKEHOLDER PERSPECTIVE

Moderator: Meghana Chalasani, MHA
Office of Strategic Programs
Center for Drug Evaluation and Research

Panelists

- **Jeff Allen**, Friends of Cancer Research
- **Marc Boutin**, National Health Council
- **Annie Kennedy**, Parent Project Muscular Dystrophy
- **Kimberly McCleary**, FasterCures
- **Paul Melmeyer**, National Organization for Rare Diseases
- **Anne Pariser**, National Institutes of Health
- **Bray Patrick-Lake**, Duke Clinical Research Institute
- **Mary Jo Strobel**, American Partnership for Eosinophilic Disorders

What questions would be helpful for FDA to address in its forthcoming guidance?

- What types of information on patient experience might be most suitable to submit in the format of proposed draft guidance?
  - If external stakeholders do not plan to develop a proposed draft guidance, what are other ways to submit PED-related information, including those types of information identified above?
- What is the process for planning and developing a proposed draft guidance relating to patient experience data to FDA?
  - Is there a recommended format or list of topics for guidance documents that an external stakeholder might develop and submit?
- What is the process for submitting a proposed draft guidance to FDA?
- What will happen after external stakeholders submit a proposed draft guidance relating to patient experience data to FDA?
- How may an external stakeholder submit proposed revisions to an existing FDA guidance?

U.S. FOOD & DRUG ADMINISTRATION

Session II

FACILITATED AUDIENCE DISCUSSION

Moderator: Meghana Chalasani, MHA
Office of Strategic Programs
Center for Drug Evaluation and Research

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Open Public Comment

Moderator: Pujita Vaidya, MPH
Office of Strategic Programs
Center for Drug Evaluation and Research

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Closing Remarks

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
Center for Drug Evaluation and Research

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THANK YOU!