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FDA's Patient-Focused Drug Development Initiative

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Public Workshop: Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks

Patient involvement in regulatory decision-making

- FDA has a number of mechanisms to involve patients in regulatory decision making on medical products:
  - FDA Patient Representative Program
  - Patient Consultant Program
  - Patient Liaison and Patient Network programs
  - CDER's Professional Affairs and Stakeholder Engagement

- PDUFA V* and FDASIA** introduce additional mechanisms to facilitate patient involvement
  - ~~"Section 1137"~~
  - Patient-Focused Drug Development

*Fifth authorization of the Prescription Drug User Fee Act
** Food and Drug Administration Safety and Innovation Act

Basic Observations

- For a drug to be approved for marketing, FDA must determine that the drug is effective and that its benefits outweigh its risks
  - The benefit-risk assessment takes into account the severity of the condition and the degree to which current therapies are meeting patients' needs
- Patients are uniquely positioned to inform FDA's understanding of this clinical context
- FDA could benefit from a more systematic method of obtaining patients' point of view on their condition, its impact on daily life, and available treatment options
  - Current mechanisms for obtaining patient input are often limited to discussions related to specific applications under review, such as Advisory Committee meetings

Patient-Focused Drug Development under PDUFA V*

- FDA committed to convening at least 20 public meetings in Fiscal Years 2013 – 17
  - Each meeting addresses a specific disease area
  - Meetings can help advance a systematic approach to gathering input

- 16 diseases areas were selected for the first three years
  - In 2012, FDA nominated candidates and sought public input
  - We considered several criteria (e.g., diseases for which important aspects are not formally captured in clinical trials)
  - Another public process in 2015 will determine the set for FY 2016-17

*Fifth authorization of the Prescription Drug User Fee Act

PFDD meetings for FY13-15

FY 2013 (**Conducted**)

- **Chronic fatigue syndrome/myalgic encephalomyelitis**
- **HIV**
- **Lung cancer**
- **Narcolepsy**

FY 2014 (**Conducted** or Announced)

- **Sickle cell disease**
- **Fibromyalgia**
- Pulmonary arterial hypertension
- Inborn errors of metabolism

FY 2014 – 2015 (to be announced)

- Alpha-1 antitrypsin deficiency
- Breast cancer
- Chronic Chagas disease
- Female sexual dysfunction
- Hemophilia A, B, and other heritable bleeding disorders
- Idiopathic pulmonary fibrosis
- Functional gastrointestinal disorders
- Parkinson's disease and Huntington's disease

Tailoring each meeting

- Meetings follow a similar, but tailored, design
  - They consider the current state of drug development, specific interests of FDA review divisions, and the needs of patients
  - Each meeting focuses on a set of questions that aim to elicit patients' perspectives on their disease and on treatment approaches

- Input is generated in multiple ways:
  - At the meeting: panel comments, facilitated discussion, polling questions, interactive webcast and phone participation
  - A federal docket enables input from a wider audience and allows for more detailed comments

A sample of what we ask

- Which symptoms have the most significant impact on your daily life?... On your ability to do specific activities?
- How well does your current treatment regimen treat the most significant symptoms of your disease?
- What specific things would you look for in an ideal treatment for your condition?
- What factors do you take into account when making decisions about using treatments? …. Deciding whether to participate in a clinical trial?

A sample of what we hear

Panelist A: [My type of attack] starts in the morning, I will wake up like I have been hit by a bus… the pain is so bad, if somebody walks across the floor, I can feel that.

Panelist B: [My biggest problem is] touch. Having my two-year-old granddaughter hug me hurts so much, I would like to push her away, but I don't want to.

Facilitator: We heard various ways that pain manifests. *Asks for show of hands for a few types*. Any pain not yet described that you want to talk about*?*

Participant A: I get a burning pain… like the burning you get from exercise, I get that from just standing up or walking in the kitchen…

Participant B: Headaches… if you can imagine making a mold of your head that's a quarter inch too small all around and put it on. It's crushing from every direction.

Product of PFDD meetings

- Each meeting results in a summary report that faithfully reflects the input heard from patients
  - The reports are shared within FDA and posted on our website
- The patient input provides insights for FDA reviewers conducting future B-R assessments for drugs to treat this disease
  - E.g., it helps our understanding of what clinical outcomes matter most and what risks may be considered acceptable to patients
- This patient input may also support drug development more broadly
  - It may help identify specific areas of patients' unmet need (e.g., fatigue)
  - It may support identification and development of patient-reported outcome measures

Addressing issues of uncertainty

- Although not the main focus, issues of uncertainty have been addressed in several meetings

- HIV – Emerging "cure research" is essential for advancing drug development, but the risks of experimental therapies such as gene therapy are highly uncertain
  - What factors do patients take into account when considering whether to participate in a research study?
  - How should the uncertainties about the study's benefits and risks be communicated through the informed consent process?

- Lung cancer – The benefits of cancer treatments to an individual patient can be highly uncertain
  - What are patients' priorities re: prolonging life vs. preserving quality of life?
  - How might patients' priorities change as their situations change?

Reflecting on our experience to date

- Patient input has been powerful and insightful
- Patient stakeholder involvement has been a key to success
- Meetings can be effectively tailored to fit the needs and interests of FDA and the patient community
- We continually learn how to best engage patients through this public meeting process:
  - How to reach a broad population that reflects the range of patient experiences and perspectives
  - How to enable patients to feel that their perspectives have been shared
  - How to represent the input in an accessible summary report

Positive feedback from participants

The patients truly appreciate … that you contributed to our cause and plea for help. **We feel heard** and we have hope for the future… (CFS/ME)

I was very inspired by the event and **left wanting to do more** for lung cancer, survivors and of course FDA...

… a tremendously insightful meeting. (sickle cell disease, industry participant)

I was part of the webcast … **I could relate** to almost all the symptoms, many much more severe than I suffer. (fibromyalgia)

…. By listening to ME/CFS patients first, and listening fully as demonstrated in the Voices report, **FDA sent our community a powerful message**: we hear you, we know you are seriously ill, and we want to help.

[We] felt **a validation and a peace** that is often missing from our daily struggles. (fibromyalgia)