Patient-Focused Drug Development

Advancing the Science of Patient Input

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The views and opinions expressed in this presentation are those of the individual presenter and should not be attributed to or considered binding on the U.S. Food and Drug Administration (FDA).
Regulatory Context

• “To be approved for marketing, a drug must be safe and effective for its intended use.”

-PDUFA V Draft Implementation Plan (Feb 2013)*

• What does it mean to be effective?
  – Demonstrates “substantial evidence that the drug will have the effect it purports or is represented to have under proposed labeled conditions of use” (21CFR314.125, 21CFR314.126)

• The meaning of “safe” is not explicitly defined in the statutes or regulations that govern approval
  – The safety of a drug is assessed by determining whether its benefits outweigh its risks

### Benefit-Risk Summary and Assessment

<table>
<thead>
<tr>
<th>Dimension</th>
<th>Evidence and Uncertainties</th>
<th>Conclusions and Reasons</th>
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<tbody>
<tr>
<td>Analysis of Condition</td>
<td>Patient Focused Drug Development</td>
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<tr>
<td>Current Treatment Options</td>
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<td>Benefit</td>
<td>Clinical Outcome Assessments (e.g., PROs)</td>
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<td>Risk</td>
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<td>Risk Management</td>
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Patients are uniquely positioned to inform understanding of the therapeutic context for drug development and evaluation

- There is a need for more systematic ways of gathering patient perspective on their condition and treatment options
- Current mechanisms for FDA to obtain patient input often limited to discussions related to specific applications under review

Patient-Focused Drug Development (PFDD) is part of FDA commitments under PDUFA V*

- FDA is convening <20 meetings on specific disease areas in 2013-17
- Meetings can help advance a systematic approach to gathering input

*The fifth authorization of the Prescription Drug User Fee Act, enacted in 2012
### PFDD Meetings for 2013-2017

<table>
<thead>
<tr>
<th>Fiscal Year 2013</th>
<th>Fiscal Year 2014</th>
<th>Fiscal Year 2015</th>
<th>Fiscal Years 2016-2017</th>
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<tbody>
<tr>
<td>• Chronic fatigue syndrome/ myalgic encephalomyelitis</td>
<td>• Sickle cell disease</td>
<td>• Female sexual dysfunction</td>
<td>To be conducted</td>
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<td>• HIV</td>
<td>• Fibromyalgia</td>
<td>• Breast cancer</td>
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<td>• Lung cancer</td>
<td>• Pulmonary arterial hypertension</td>
<td>• Chagas disease</td>
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<td>• Narcolepsy</td>
<td>• Inborn errors of metabolism</td>
<td>• Functional gastrointestinal disorders</td>
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<td>• Hemophilia A, B, and other heritable bleeding disorders</td>
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<td>• Idiopathic pulmonary fibrosis</td>
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<td>• Parkinson’s disease and Huntington’s disease (Sept. 22)</td>
<td>To be conducted</td>
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<td>• Alpha-1 antitrypsin deficiency (Sept. 29)</td>
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<td>• Alpha-1 antitrypsin deficiency (Sept. 29)</td>
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<td></td>
<td>• Non-tuberculous mycobacterial lung infections (October 15)</td>
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<td>• Alopecia areata</td>
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<td>• Autism</td>
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<td></td>
<td>• Hereditary angioedema</td>
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<td></td>
<td>• Patients who have received an organ transplant</td>
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<td>• Psoriasis</td>
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<td>• Neuropathic pain associated with peripheral neuropathy</td>
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<td>• Sarcopenia</td>
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Meeting Output

• Each meeting results in a Voice of the Patient report that faithfully captures patient input from the multiple streams
  *http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm

• This input can support FDA staff, e.g.:
  – Conduct benefit-risk assessments for products under review
  – Advise drug sponsors on their drug development programs

• It might also support drug development more broadly:
  – Help identify areas of unmet need in the patient population
  – Help identify or develop tools that assess benefit of potential therapies
  – Help raise awareness within the patient community
Other Contributions of PFDD

• Complement scientific workshops
  – Ex. CFS/ME, Female Sexual Dysfunction, Chagas, Nontuberculous mycobacterial infections
  – Support development of disease-specific guidance (Ex. CFS/ME)

• Support efforts to develop PRO tools
  – Ex. Multi-partner working group on PRO development for CFS/ME

• Identify opportunities for further discussions
  – Ex. Brookings workshop in follow up to Sickle Cell Disease meeting

• Channel patient engagement
  – Patient representatives identified for CFS/ME and HIV
Externally-Led PFDD Meetings

• There is external interest in expanded efforts to gather patient input in support of drug development and evaluation

• Meetings conducted by external stakeholders provide an opportunity to expand the benefits of PFDD
  – Meetings should target disease areas where there is an identified need for patient input on topics related to drug development
  – FDA’s PFDD meetings can serve as a model

• For more information, please visit:
  http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm453856.htm
Patients with chronic serious disease are experts on what it’s like to live with their condition.

They are able to articulate specific disease impacts (symptoms, loss of function) in concrete terms.

They can identify and articulate what is important to them regarding treatment benefit.
- For progressive degenerative diseases, many patients/parents feel an ideal treatment would at minimum stop progression of their/their child’s loss of function.

Their “chief complaints” may not be factored explicitly into drug development plans.
- E.g., as endpoints and measures of drug benefit in clinical trials.
Some PFDD Learnings to Date (2)

- Patients want to be as active as possible in the work to develop and evaluate new treatments.
- They want their experience described using words that they consider to best describe how it feels.
- They and their caregivers are able and willing to engage via the Internet, social media, and all other means at their disposal.
- They aren’t expecting for FDA to address all the gaps in current treatment or current approaches to drug development but do want FDA to help identify the most effective pathway for them to play major contributing role.
FDA’s Role

- FDA’s mission is to protect and promote public health by evaluating the safety and effectiveness of new drugs.

- While we play a critical role in drug development, we are just one part of the process. We do not develop drugs or conduct clinical trials.

- FDA recognizes that it is not the agency’s role to lead much of the development work on specific tools for specific drug development programs.

- However, we do play a constructive role in guiding, helping, or evaluating at some stages of the pre-clinical translational and later clinical development work.
Some Key Questions to Address

- What impacts matter most to patients and how do we measure them?
- What aspects of clinical trials can be better tailored to meet the patients who (might) participate in the trial?
- How can FDA integrate collected patient experience data and information into its benefit-risk assessment?
- How to best communicate the information to patients and prescribers?

Translational Studies
Clinical Studies
Pre-market Review
Post-market
PFDD Potential Next Steps

Advance science of patient input

- Engage wider community to discuss methodologically sound approaches that:
  - Bridge from initial PFDD meetings to more systematic collection of patients’ input
  - Generate meaningful input on patients’ experiences and perspectives to inform drug development and B-R assessment
  - Are “fit for purpose” in drug development and regulatory context

Provide guidance

- To: patient communities, researchers, and drug developers
- On: pragmatic and methodologically sound strategies, pathways, and methods to gather patient and use input
Immediate Next Steps

• Continue fulfilling commitments for PDUFA V

• Further engagement and discussions with patients and other stakeholders in preparation for PDUFA VI
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