FDA’s Patient-Focused Drug Development

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Navigating the Center for Drug Evaluation and Research
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Creating opportunities for dialogue

• There is a need for more systematic ways of gathering patient perspective on their condition and treatment options
  – This input helps inform understanding of the therapeutic context for drug development and evaluation
  – 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 24 meetings on specific disease areas in FY 2013-17
  – Meetings help advance a systematic approach to gathering input

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

<table>
<thead>
<tr>
<th>Fiscal Year 2013</th>
<th>Fiscal Year 2014</th>
<th>Fiscal Year 2015</th>
<th>Fiscal Year 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>• Non-tuberculous mycobacterial lung infections</td>
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<td>• HIV</td>
<td>• Fibromyalgia</td>
<td>• Breast cancer</td>
<td>• Psoriasis</td>
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<td>• Lung cancer</td>
<td>• Pulmonary arterial hypertension</td>
<td>• Chagas disease</td>
<td>• Neuropathic pain associated with peripheral neuropathy (June 10)</td>
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<td>• Narcolepsy</td>
<td>• Inborn errors of metabolism</td>
<td>• Functional gastrointestinal disorders</td>
<td>To be announced</td>
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<td>• Hemophilia A, B, and other heritable bleeding disorders</td>
<td>• Parkinson’s disease and Huntington’s disease</td>
<td>• Alopecia areata</td>
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<td></td>
<td>• Idiopathic pulmonary fibrosis</td>
<td>• Alpha-1 antitrypsin deficiency</td>
<td>• Autism</td>
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<td>• Hereditary angioedema</td>
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<td>• Patients who have received an organ transplant</td>
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<td>• Sarcopenia</td>
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Tailoring Each Meeting

• Meetings follow similar, but tailored, design
  – Takes into account current state of drug development, specific interests of FDA review division, needs of the patient population

• Discussion elicits patients' perspectives on their disease and on treatment approaches

• Input is generated in multiple ways:
  – Patient panel comments and facilitated discussion with in-person participants
  – Interactive webcast and phone line for remote participants
  – A federal docket allowing 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?
Patient stakeholders have taken initiative

- Spread word through websites, social media or flyers
- Facilitated registration or docket submission
- Organized transportation, pre or post-meeting get-togethers
- Conducted webinars to prepare participants to “use their voice most effectively”
- Conducted surveys
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.:
  – Conducting B-R assessments for products under review
  – Advising drug sponsors on their drug development programs
  – Identify opportunities for further dialogue (e.g., future workshops)

• 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
More work to do, but a step in the right direction

“We need to have this kind of open exchange consistently... even if it's uncomfortable, because that's where all of this insight and perspective comes from.”

“[W]e felt a validation and a peace that is often missing from our daily struggles.”

“By ... 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.”

“..information we received from stakeholders...was priceless, and ......will impact drug development well into the future” (FDA participant)

“[W]e have opened up to you in ways that many of us do not open up to our hematologists, and...in ways that many of our families have never seen us before.”

“... a tremendously insightful meeting.” (industry attendee)

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

*Select quotes from participants during or after a PFDD meeting.*
An Opportunity for Externally-Led PFDD Meetings

- There are many more disease areas than can be addressed in the FDA meetings planned under PDUFA V
- FDA welcomes patient organizations to identify and organize patient-focused collaborations to generate public input on other disease areas
- FDA will be open to participating in a well-designed and well-conducted meeting
- We recommend using the process established through Patient-Focused Drug Development as a model
- An externally-led PFDD meeting and any resulting products (e.g., surveys or reports) will not be considered FDA-sponsored or FDA-endorsed
- Meetings conducted by external stakeholders provide an opportunity to expand the benefits of PFDD
Considerations on Externally-led Meetings

• Meetings should target diseases where there is an identified need for patient input on topics related to drug development, e.g.,
  – Diseases that chronic, symptomatic, or affects functioning and activities of daily living;
  – Disease area for which aspects of the disease are not formally captured in clinical trials;
  – Disease area for which there are currently no therapies or very few therapies;
• The target patient population should be carefully considered and clearly defined
  – Is it valuable to focus the meeting on any particular subpopulation(s), such as children, people with metastatic forms of the disease, etc.?
• Meeting success will require a joint and aligned effort by all interested stakeholders
• Valuable deliverables: Summary report, meeting recordings, transcripts
Other Considerations on Externally-Led PFDD Meetings

• The Letter of Intent (LOI) should communicate the importance of the meeting in the context of the disease area and the meeting plan:
  – Proposed timing, location
  – Proposed format, agenda, discussion questions
  – Patient representation and outreach strategy
  – Collaborators, sponsorship

• LOI should be submitted approximately **1 year before** the anticipated meeting date

• LOI submissions will be reviewed on a quarterly schedule

• When determining our level of participation, FDA will consider:
  – Specific need for more input from patient perspective
  – Recent interactions with patients/stakeholders
  – Meeting time/location
  – Division staff capacity
For more info

• FDA website on Externally-Led PFDD Meetings
  – http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm453856.htm

• FDA website on FDA’s PFDD Meetings
  – http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm
  – The previously conducted meetings include all of the meeting materials, such as agendas, discussion questions.

• Email patientfocused@fda.hhs.gov
  – FDA CDER’s Office of Strategic Programs is leading FDA’s PFDD effort
  – We can also connect you to other offices in FDA who work on external stakeholder engagement efforts
Acknowledgement

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