## Creating Opportunities for Dialogue

<table>
<thead>
<tr>
<th>Fiscal Year 2013</th>
<th>Fiscal Year 2014</th>
<th>Fiscal Year 2015</th>
<th>Fiscal Year 2016</th>
<th>Fiscal Year 2017</th>
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</thead>
<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>
<td>• Sarcopenia</td>
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<td>• HIV</td>
<td>• Fibromyalgia</td>
<td>• Breast cancer</td>
<td>• Psoriasis</td>
<td>• Autism</td>
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<tr>
<td>• Lung cancer</td>
<td>• Pulmonary arterial hypertension</td>
<td>• Chagas disease</td>
<td>• Neuropathic pain associated with peripheral neuropathy</td>
<td><strong>To be announced</strong></td>
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<td>• Narcolepsy</td>
<td>• Inborn errors of metabolism</td>
<td>• Functional gastrointestinal disorders</td>
<td>• Patients who have received an organ transplant</td>
<td><strong>To be announced</strong></td>
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<td></td>
<td>• Hemophilia A, B, and other heritable bleeding disorders</td>
<td>• Huntington’s disease and Parkinson’s disease</td>
<td></td>
<td><strong>To be announced</strong></td>
</tr>
<tr>
<td></td>
<td>• Idiopathic pulmonary fibrosis</td>
<td>• Alpha-1 antitrypsin deficiency</td>
<td></td>
<td><strong>To be announced</strong></td>
</tr>
</tbody>
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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?
Key to Success: Active Outreach

• 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

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Meetings strengthen understanding of disease and treatment burden

- Each meeting results in a Voice of the Patient report that faithfully captures patient input from the various information streams.
- This input can support FDA staff, e.g.:
  - In conducting benefit-risk assessments for products under review, by informing the therapeutic context.
  - Advising drug sponsors on their development program.
- 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 and channel engagement within the patient community.
Externally-led PFDD: The Opportunity

• There is interest to expand the 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

• An externally-led PFDD meeting and any resulting products (e.g., surveys or reports) will not be considered FDA-sponsored or FDA-endorsed

• The success of an externally-led patient-focused meeting require a joint, aligned effort by multiple patient advocacy groups associated with the disease area, and other interested stakeholders
Externally-led PFDD: Planning a Meeting

- **Key participants:** Patients, patient representatives, patient advocates

- **Target audience (listening mode):** Regulatory / other federal agencies, medical product developers, researchers, healthcare professionals

- **FDA planned meetings can serve as a model:**
  - Target disease areas where there is an identified need for patient input on topics related to drug development
  - Main discussion topics: (1) Symptoms and impacts that matter most to patients and (2) current approaches to treatment
  - Facilitator-led large group discussion, interactive webcast, discussion aids (e.g., polling tools)
  - Meeting deliverables: Web recording, transcript, summary report

- **Do not have to be standalone meetings:** Please consider incorporating PFDD-style sessions in annual conferences, scientific workshops, etc.
Externally-led PFDD: Key Considerations

• Please submit a letter of intent (LOI) to OSP: Our team is here to serve as a helpful resource to you
  

• While we truly understand the effort it takes to plan a PFDD meeting, it can be done without being resource intensive!

• The key to an insightful, robust, and informative PFDD meeting is active community outreach to ensure a representative group of patient perspectives in the room.

• We must be respectful of the time of patients and their caregivers
For more information

• FDA website on FDA’s PFDD Meetings
  – The previously conducted meetings include all of the meeting materials, such as agendas and discussion questions, as well as the summary Voice of the Patient reports
  – [http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm](http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm)

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

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