Patient-Focused Drug Development

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Roadmap for Engaging with FDA CDER
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Creating Opportunities for Dialogue

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016	Fiscal Year 2017
• Chronic fatigue syndrome/ myalgic encephalomyelitis • HIV • Lung cancer • Narcolepsy	• Sickle cell disease • Fibromyalgia • Pulmonary arterial hypertension • Inborn errors of metabolism • Hemophilia A, B, and other heritable bleeding disorders • Idiopathic pulmonary fibrosis	• Female sexual dysfunction • Breast cancer • Chagas disease • Functional gastrointestinal disorders • Huntington's disease and Parkinson's disease • Alpha-1 antitrypsin deficiency	• Non-tuberculous mycobacterial lung infections • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Patients who have received an organ transplant	• Sarcopenia • Autism *To be announced* • Alopecia areata • Hereditary angioedema

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

Unite Narcolepsy

Working together toward a brighter future

- 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

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
  - Guidelines for the Letter of Intent (LOI): https://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM453857.pdf
- 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

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

- 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 FDA offices who work on external stakeholder engagement efforts


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