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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

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016-2017
• 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 • Parkinson's disease and Huntington's disease • Alpha-1 antitrypsin deficiency	• Non-tuberculous mycobacterial lung infections • Psoriasis • Neuropathic pain associated with peripheral neuropathy (June 10) *To be announced* • Alopecia areata • Autism • Hereditary angioedema • Patients who have received an organ transplant • Sarcopenia

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

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

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."

"[We] 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

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