FDA's Patient-Focused Drug Development Initiative

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Patient-Focused Drug Development

- There is a need for more systematic ways of gathering patient perspective on their condition and treatment options
  - Patients are uniquely positioned to inform understanding of the therapeutic context for drug development and evaluation
  - Current mechanisms for FDA to directly 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*
  - CDER and CBER are convening 24 meetings on specific disease areas in FY 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

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Years 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) • Patients who have received an organ transplant (Sept 27) *To be announced* • Alopecia areata • Autism • Hereditary angioedema • Sarcopenia

What does a meeting entail?

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

- Symptoms that have the most significant impact on their daily lives
- Most bothersome effects and aspects of treatment
- Factors they take into account when making treatment decisions

Input is generated in multiple ways:

- Patient panel comments and facilitated discussion
- Interactive webcast and phone line for remote participants
- A federal docket allowing for more detailed comments

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
- 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 foster engagement within the patient community

Lessons learned that may be applicable to post-market assessment

- Patients (and caregivers) living with chronic, symptomatic conditions are experts on what it's like to live with their disease
  - They are able to articulate specific disease impacts (symptoms, loss of function) in concrete terms
  - At times, it can be difficult to distinguish between effects associated with disease vs. effects associated with treatment (e.g., fatigue)

- Many patients and caregivers want to be as active as possible in the work to develop and evaluate new treatments
  - They greatly appreciate the opportunity to directly engage with FDA
  - They are able and willing to engage via the Internet, social media, and all other means at their disposal

Other Considerations

- Collecting patient input in this novel way has raised a number of questions, e.g.,:
  - Understanding who participates and to what degree they may be representative of the broader patient population
  - Receiving a wide range of input (e.g., multiple surveys conducted in varying ways) and understanding how best to interpret this input

- Current efforts are aimed at understanding how best to advance methodologically sound approaches:
  - Bridge from PFDD meetings to more systematic collection of patients' input
  - Are "fit for purpose" in drug development and regulatory context
  - Provide pragmatic methods for patient communities, researchers and drug developers