2015 Science Writers Symposium

The Science of Patient Input
FDA's Patient-Focused Drug Development Initiative

Sara Eggers, Ph.D.

Operations Research Analyst

Office of Strategic Programs

Center for Drug Evaluation and Research

September 18, 2015

Creating Opportunities for Dialogue

There is a need for more systematic ways of gathering patient perspectives on their condition and treatment options

- This input helps inform understanding of the *therapeutic context* for drug development and evaluation
- Current mechanisms for obtaining patient input often limited to discussions related to specific applications under review

Patient-Focused Drug Development is part of FDA commitments under PDUFA V*

- FDA is convening more than 20 meetings on specific disease areas between 2013 and 2017
- Meetings help advance a systematic approach to gathering input

*The fifth authorization of the Prescription Drug User Fee Act, enacted in 2012

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

PFDD Meetings for Fiscal Years 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 *To be conducted* • Parkinson's disease and Huntington's disease (Sept. 22) • Alpha-1 antitrypsin deficiency (Sept. 29)	*To be conducted* • Non-tuberculous mycobacterial lung infections (October 15) *To be announced* • Alopecia areata • Autism • Hereditary angioedema • Patients who have received an organ transplant • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Sarcopenia

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?

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 raise awareness within the patient community

Example

Chronic Fatigue Syndrome / Myalgic Encephalomyelitis (CFS/ME)

April 25, 2013: FDA conducted its first PFDD meeting, on CFS/ME

- Part of a 2-day workshop to explore important issues related to CFS/ME drug development

The meeting enabled patients and caretakers to share their experience and perspectives on their disease

The patient and advocate community was very engaged:

- ~70 patient and patient representatives attended the meeting
- Many others participated by web
- 228 comments were submitted to the public docket

*Voice of the Patient report and scientific meeting summary available on FDA website, http://www.fda.gov/Drugs/NewsEvents/ucm369563.htm

Example

Key Themes of the CFS/ME input

CFS/ME is much more than simply feeling fatigued.

- > 50 symptoms were described – physical and cognitive effects
- Cognitive effects ("brain fog") received the most attention
- "Fatigue" takes many forms: "wired but tired", "bone-crushing"
- A "crash" can occur without warning and is debilitating

Patients have tried everything.

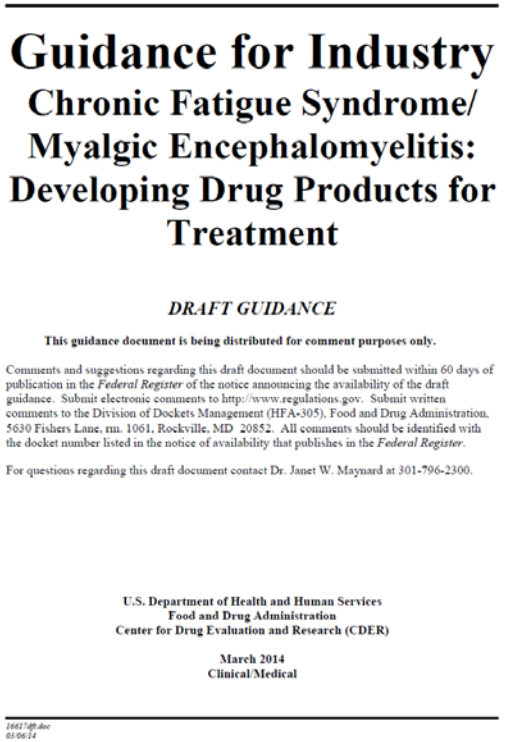
- Over 100 drug and non-drug therapies were mentioned, varying greatly in their perceived effectiveness and side effects
- They most want treatments that can address the underlying causes of illness

The disease can take a devastating toll on patients and families.

Example

Efforts Informed by the 2013 Meeting

- Voice of the Patient report (2013)
  http://www.fda.gov/downloads/Drugs/NewsEvents/UCM453718.pdf
- Draft Guidance on CFS/ME Drug Development (2014)
  http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM388568.pdf
- Input to 2014 Institute of Medicine report on CFS/ME
  http://iom.nationalacademies.org/Reports/2015/ME-CFS.aspx
- Establishment of multi-partner workgroup to advance Patient Reported Outcomes (PROs) for CFS/ME



Guidance for Industry

Chronic Fatigue Syndrome/ Myalgic Encephalomyelitis: Developing Drug Products for Treatment

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document contact Dr. Janet W. Maynard at 301-796-2300.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

March 2014
Clinical/Medical

166174fr.doc
03/06/14

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 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."

"[We] felt a validation and a peace that is often missing from our daily struggles."

" … a tremendously insightful meeting." (industry attendee)

"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."

"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.