



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA 2012-N-0001]

Food and Drug Administration Patient Network Annual Meeting; Input Into Food and Drug Administration Benefit-Risk Decisionmaking: Opportunities and Challenges; Hosted by the Food and Drug Administration Office of Special Health Issues; Notice of Meeting

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

The Food and Drug Administration (FDA) is announcing a meeting for patients, caregivers, independent patient advocates and patient advocate groups, and health professional groups to explore ways to more effectively include patient input in regulatory decisionmaking on drug, device, and biological products. The meeting will serve as a forum for FDA's patient stakeholders and the general public, including health professionals, academia, and industry to learn about the regulatory process related to the medical product life cycle, analyze where in the process patient input may be most practical and most valuable, and explore practicable approaches to collecting and incorporating meaningful input that well represents broad patient perspectives into regulatory decisions.

Date and Time: The meeting will be held on May 18, 2012, from 9 a.m. to 4:30 p.m. Register at <http://fda.contractmeetings.com/home> on or before May 4, 2012. Please include the name and title of the person attending, the name of the organization, the role within the organization, email address, and telephone number. There is no registration fee for this conference. Early registration is suggested because space is limited. We request that

organizations limit the number of representatives to two. For further registration information or problems with the Web site, call Cindy de Sales, 1-240-316-3200, ext. 207.

If you need special accommodations due to a disability, please contact Steve Morin at least 7 days in advance.

Location: The meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (rm. 1503) Silver Spring, MD 20993.

Contact Person: Steve Morin, Office of Special Health Issues, 10903 New Hampshire Ave., Silver Spring, MD 20993, 301-796-0161, FAX: 301-847-8623, Steve.Morin@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. FDA Patient Network

This is the inaugural FDA Patient Network Annual Meeting hosted by the FDA Office of Special Health Issues, the Agency's liaison to the patient and health professional communities. This annual meeting is being hosted in conjunction with the launch of the overarching FDA Patient Network program. The FDA Patient Network is a new resource for patients, caregivers, independent patient advocates, and patient advocate groups that seek to:

- Educate and inform patient stakeholders about FDA, its regulatory authorities and processes, its initiatives and programs, etc.; and,
- Provide a venue for advocacy for patient stakeholders within FDA and be transparent to patients about Agency actions.

In addition to an annual meeting, the FDA Patient Network consists of other activities, including the:

- FDA Patient Network Web site--A new, patient-centered Web site that contains educational modules, centralized Agency information, and multidirectional communication tools;
- Biweekly FDA Patient Network News email newsletter; and hosting of periodic meetings, briefings, and listening session between patient advocates and FDA staff.

II. Patient Perspectives in Regulatory Decisionmaking

Establishing a means for obtaining input from patients and patient advocate groups will allow FDA to further enhance its benefit-risk assessment in regulatory decisionmaking. Patients who live with a disease have a direct stake in the outcomes of the review process and are in a unique position to contribute to the weighing of benefit-risk considerations that can occur throughout the medical product development process. Though several programs exist that facilitate patient representation on Advisory Committees or participation in selected review meetings, there are currently few venues in which the patient perspective is discussed outside of a specific product's marketing application review. The medical product review process could benefit from a more scientific, systematic, and expansive approach to obtaining input from patients who are experiencing a particular disease condition.

As part of the proposed agreements for Prescription Drug User Fee Act (PDUFA) V, FDA plans to conduct meetings with patients and patient advocacy groups to gather broader patient input. This meeting kicks off these efforts and provides an opportunity to gain feedback on how FDA can best structure these upcoming meetings.

FDA seeks public discussion based on the following questions. These questions are intended to frame patient input at the May 18, 2012, meeting and there will be time at the meeting to discuss the following issues.

(1) How can FDA ensure gathering a broad range of representative patient input that is relevant to a specific disease area during its meetings with patients? For example, who should serve as representatives of patients?

(2) What methodological and practical issues should FDA consider as it develops its strategy for eliciting the patient perspective? For instance, FDA is interested in addressing topics including, but not limited to, the following:

(a) Are there particular advantages or disadvantages to utilizing face-to-face meetings versus web-based or other methods in obtaining the patient perspective on a particular disease condition and its treatment?

(b) How can FDA ensure that certain subpopulations, such as patients with the most severe form of the disease, are represented?

III. Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research Efforts

Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research plan to conduct a series of patient-focused drug development meetings to gather patient input on the clinical context of a disease and its impact on a patient's daily life. These considerations, which would include an analysis of the severity of the disease condition and the current state of the available treatment options, can be critical in regulatory decisionmaking. FDA is interested in obtaining patient input on the context of specific disease areas through the patient-focused drug development meetings. The following questions are examples of topics for which FDA believes the patient perspective could add valuable insight. They are presented in this document for general discussion at the Patient Network Conference.

A. Understanding the Disease Condition

(1) What are the clinical manifestations of the disease that have the greatest impact on patients?

(2) Are there other aspects of the disease that have a significant impact on a patient's daily life? (e.g., impaired mobility, sleep problems, etc.)

(3) How do the clinical manifestations change with disease progression?

(4) How do the other aspects of the disease change with disease progression?

B. Assessment of Treatment Options

(1) How effective are approved therapies at treating the clinical manifestations of the disease?

(2) How well do approved therapies mitigate the other aspects of the disease?

(3) How does the effectiveness of approved therapies change with progression of the disease?

(4) Does therapy effectiveness vary by patient subpopulation?

FDA is continuing to make plans for its efforts and will be able to provide more detail on the patient-focused drug development meetings at the Patient Network Conference.

IV. Center for Devices and Radiologic Health Efforts

Center for Devices and Radiologic Health is interested in a public discussion on issues related to risk associated with medical products, and on avenues for patients to provide input into regulatory decisionmaking related to the amount of risk patients may be willing to accept in exchange for a potential treatment benefit. The following questions are presented in this document for general discussion at the Patient Network Conference.

(1) How do patients perceive and weigh risks associated with medical treatment in light of the risk associated with the underlying condition being treated and the potential benefit from the treatment?

(2) Under what circumstances and in which populations would various levels of risk be appropriate/acceptable?

(3) How can medical device companies, government, academia, community physicians and patients collaborate to account for the level of risk acceptable to patients affected by serious or life threatening illnesses?

(4) What mechanisms would be appropriate for patients to provide input into regulatory decisionmaking for new therapeutic and diagnostic products--e.g., web-based survey instruments? Patient representation at advisory committee meetings? Patient input to medical device companies during clinical trial design? Who (FDA, patient advocate groups, medical device companies, etc) could sponsor such surveys?

(5) Are patients willing to accept responsibility for the level of risk to which they may be exposed if patient input increases risk tolerance?

Dated: April 13, 2012.

Leslie Kux,

Assistant Commissioner for Policy.