NATIONAL HEALTH COUNCIL

Implementation Manual

How to Operationalize
the National Health Council's Patient Information Tool

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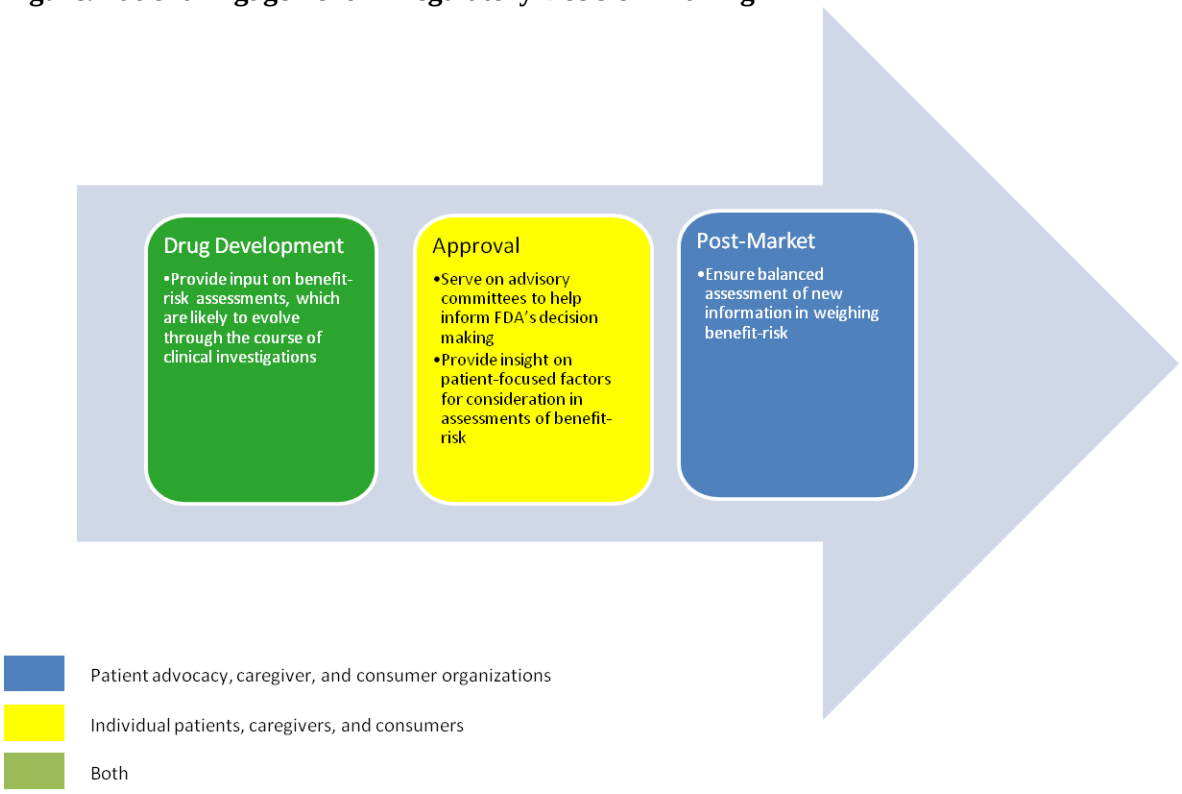
Background

Government agencies like the Food and Drug Administration (FDA), which is responsible for approving the drugs and medical devices that can be sold in the United States, are increasingly interested in collecting information on patient perspectives and preferences to better understand the needs of patients and caregivers. As part of its review process, the FDA must consider the benefits and risks of drugs and devices on patient populations. Often, the patient populations affected by certain diseases can be very diverse. Additionally, treatment options and patient needs can vary widely based on the severity of the disease or condition. As the figure below depicts, there are several points in the FDA process at which gathering this type of information from the patient community would be useful and warranted. To help gain this input, FDA has created the Patient-Focused Drug Development (PFDD) program, which aims to more systematically gather patients' perspectives on their condition and available therapies to treat their condition.[1]

Figure. Patient Engagement in Regulatory Decision-Making



As part of the PFDD program, FDA will hold 20 public meetings over the next five years, each focusing on a different disease or condition. The aim of these meetings is to hear from patients and their caregivers on the impact of the disease or condition on quality of life, to better understand individual experiences with treatment regimens, and to determine what aspects of treatment or symptom relief are most important to patients. FDA has already announced its intent to expand the program beyond these initial 20 disease areas and hold additional meetings in the future. In addition, they have expressed their willingness

[1] See Additional Resources section for more information on FDA's PFDD

to increase their availability to meet with patients and patient organizations outside of these formal meetings.

About the National Health Council's Patient Information Tool

The National Health Council created The Patient Information tool to help patient organizations systematically stratify their patient populations, collect information, and organize issues, to ensure communications with FDA and other stakeholders regarding benefit-risk are as comprehensive as possible.

The tool outlines three major categories of topics based on FDA's stated areas of interest. The questions have been crafted to reflect issues that are important to patients while also being responsive to specific information that FDA has indicated would be useful for their review. The three categories focus on: (I) Identification of Subpopulations, (II) Description of Disease Impact, and (III) Description of Treatment and Management Options.

This Implementation Manual is organized into three stages:

(1) Gathering Information;
(2) Translating Information; and
(3) Sharing Information.

Included under each of these stages are specific steps that should be taken and options to be considered when operationalizing the tool.

In this manual, many examples are borrowed from Unite Narcolepsy, an education and empowerment initiative created by the narcolepsy community to help inform people affected by narcolepsy about the FDA's PFDD program and to prepare them to respond.[2] Narcolepsy was among the first topics addressed by FDA in its PFDD meetings and thus the experience of the Unite Narcolepsy initiative can offer valuable learnings for patient organizations that will engage with FDA in the future.

[2] http://www.unitenarcolepsy.org/about-us/

Instructions

Stage 1. Gathering Information

In this initial stage, there are five main steps you can take to ensure that the information you are gathering from your patient populations is relevant and comprehensive. These steps are outlined below:

Step 1. Define Patient Subpopulations
Step 2. Identify Potential Partners
Step 3. Develop Educational Resources
Step 4. Prioritize Information that You Want to Gather
Step 5. Develop an Outreach Strategy

Step 1. Define Patient Subpopulations

One of the primary goals of the Patient Information Tool is to capture information on diverse groups of patients. To ensure the understanding of a disease's impact includes the views of all the subgroups that may be affected by a disease, an important initial step will be to delineate each of these subpopulations.

In determining how to stratify patient populations into these smaller groups, consider factors such as:

- Age (child, adult, elderly, or elderly adult)
- Severity of disease (mild, moderate, severe, end-of-life)
- Other factors or predispositions (gender, race, occupation)
  Example: Veteran population

Subpopulations are not always clear or well-defined. As you embark on this information collection activity, it is possible that subpopulations that had previously not been considered will emerge. Identifying these new subgroups could prove to be particularly helpful in gaining a comprehensive picture of the patient population.

Step 2. Identify Potential Partners

To ensure the information you collect is as comprehensive as possible, consider potential partners in this effort. These partners can range from:

- Individuals (influential people, such as healthcare professionals and prominent Web bloggers)
- Organized groups (patient advocacy organizations)
- Networks (community groups, online support groups)

Bringing together other organizations or entities will help to determine who and where your patient constituents are, how they are organized, and what may be the best ways to reach them. Different subpopulations identified in Step 1 may require customized outreach approaches. Many patients may already be in touch with and accessible through potential partners, for example members of online communities or members of patient organizations.

Example: Unite Narcolepsy – A PFDD Initiative

To prepare for the FDA PFDD meeting on narcolepsy, Wake Up Narcolepsy, a non-profit patient organization dedicated to funding narcolepsy research and raising awareness of narcolepsy, led the development of an education and engagement initiative called Unite Narcolepsy. The initiative was launched by a partnership of narcolepsy patient and advocacy organizations in the summer of 2013 to mobilize the narcolepsy community.

Step 3. Develop Educational Resources

Many people may not be familiar with FDA and its current effort to engage patients. To generate a robust response, develop user-friendly, educational background materials that could communicate to patients the purpose of FDA, how FDA engages with patients, and why engaging with FDA presents an opportunity to help bring new treatments to market.

Step 4. Prioritize Information that You Want to Gather

The Patient Information Tool provides the full range of information that would be helpful to FDA and other potential users seeking to better understand patient perspectives on a disease or condition. Based on this range, you may find that some areas may not be very relevant to your disease. In this case, prioritize the information that you will want to gather.

Consider the Questions You Need to Ask

From the Patient Information Tool, identify questions most relevant and applicable to your patient population. If your disease/condition is one of those on which FDA is having a PFDD meeting, cross-reference these with questions that FDA may have released in advance of the meeting, such as below:

Example: Federal Register Notice for Narcolepsy Public Meeting on Patient-Focused Drug Development[3]

Three months before the FDA meeting on narcolepsy, the agency released a notice in the Federal Register that included a brief list of questions it was seeking to address. Sample questions from this notice include:

Topic 1: Disease symptoms and daily impacts that matter most to patients:

1. *Of all the symptoms that you experience because of your condition, which one to three symptoms have the most significant impact on your life? (Examples may include excessive daytime sleepiness, cataplexy, etc.)*
2. *Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition? (Examples of activities may include sleeping through the night, work and school performance, etc.)*
3. *How have your symptoms changed over time?*
   - 3.1. *Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?*

[3] See Additional Resources section for more information on FDA's Federal Register Notice for the Narcolepsy PFDD Meeting

Develop a Strategy for Gathering Information

Prior to gathering any information, develop a strategy for how to most effectively and efficiently collect the information. Consider what methods or combination of methods you should use, and also in what order you should to deploy these methods. Formulating a strategy for collecting information will help ensure that resources are efficiently utilized.

Select Method(s) for Gathering Information

Determining the best way to gather the information will depend on the information that you want to collect and on factors, such as patients' accessibility, geographical location, health literacy, and access to technology. For hard-to-reach patient communities, special consideration should be given as to how to overcome these barriers.

Surveys

A survey presents a series of questions to gather information about what people do or think. This is the best way to reach the broadest base of patients and is appropriate for collecting information on a broad range of topics.

- In formulating the survey, consider which types of questions would be most suitable for the patient population you are targeting. Certain types of questions may require a combination of formats.

    - Multiple Choice: Questions with discrete answer choices that require participants to choose among a few possible answers. The benefit to multiple-choice format is that it is easier to compile and analyze all the responses.

        Example: How often do you experience side effects related to your disease/condition?

        A. Never
        B. Occasionally
        C. Sometimes
        D. Frequently
        E. Constantly

    - Ranking: Questions that ask patients to rank order their response (may be best to seek information regarding preferences)

        Example: Rank order the side effects based on how significantly they affect your life

        ___ *Chronic fatigue*
        ___ *Depression*
        ___ *Reduced mobility*
        ___ *Vision problems*
        ___ *Abdominal discomfort*

- Open Text: Questions that allow patients to describe their experiences and preferences in free text

  Example: How do the treatment and management options for your disease/condition affect your daily life on the best days and the worst days?

- Assemble a group of "beta testers" before distributing the survey to assess:
  - Readability and clarity of the survey questions
  - Format of the survey questions
  - Length of the survey
  - Amount of time it takes to answer questions

Focus Groups

A focus group is a form of group interview that allows for an open discussion and is usually led by a moderator. Focus groups may be helpful when the primary objective is to elicit a broad range of reactions or interpretations.[4]

Focus groups usually consist of a small number of participants (e.g. 6-10 people) and the discussions are structured around sets of prepared questions that prompt participants to share their perspectives and encourage further group discussion.

Deliberative [5]Methods

Deliberative methods are a more intensive form of engagement. It entails identifying the values underlying an individual's views and asks participants to focus on the reasons for their perspectives. Deliberative methods address an ethical- or values-based dilemma in which the process of deliberation is intended to allow the negotiation of competing viewpoints.

Targeted Interviews

Targeted interviews involve selecting individual patients or caregivers to ask specific questions and have more in-depth conversation. These interviews may potentially arise as follow-up to previous focus group discussions or survey responses.

Step 5. Develop an Outreach Strategy

Once you have decided on how to collect the information, consider different ways to reach patients that are representative of the disease subpopulations. Outreach strategies can range in scale based on the number of patients that you are trying to reach or the size of your organization and its potential partners.

Smaller scale outreach strategies can include:	Examples include:
Distribute mail	Mailing of educational resources, surveys, or notices through traditional mail or electronically

[4] http://grants.nih.gov/grants/guide/rfa-files/RFA-HS-14-007.html
[5] http://grants.nih.gov/grants/guide/rfa-files/RFA-HS-14-007.html

	via email
Disseminate web surveys	Creation of a survey webpage that can be distributed through email or posted on targeted websites
Administer in-person surveys	Soliciting input or survey responses real-time and in-person (e.g. at health fairs; cold-calling) through administration by a staff member or volunteer

Larger-scale outreach strategy can include:	**Examples include:**
Launch a broader campaign that, in conjunction with survey dissemination, includes related information and other resources. This effort could be co-sponsored by a group of patient organizations and related entities to raise awareness.	The Unite Narcolepsy launched multi-pronged campaign to both educate patients and stakeholders in the narcolepsy community and engage them. The initiative sought to inform patients and stakeholders on the FDA meetings and directed them to take a survey posted to the Unite Narcolepsy website. [6]

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[6] See Additional Resources section for more information on Unite Narcolepsy

Stage 2. Translating Information

In this next stage, there are two main steps involved with ensuring the data you have collected from patients is turned into useful and accessible information. These steps are outlined below:

Step 1. Compile and Organize Information
Step 2. Synthesize Information

Step 1. Compile and Organize Information

Once you have collected the information, organize the data in a way that corresponds to the key questions as identified by the FDA, if applicable (see Stage 1, Step 4). Include a mix of quantitative data and qualitative information.

- Quantitative: data that can be expressed as a number or quantified

  Example: Data and statistics on disease impact and treatment options by subpopulation

  Cataplexy, a striking and sudden episode of muscle weakness often triggered by strong emotion, was reported by 65% of respondents. The three symptoms rated as having the most significant impact on patients' lives were excessive daytime sleepiness (77%), difficulty thinking, remembering, concentrating or paying attention (50%), and general fatigue/never feeling rested (45%).[7]

- Qualitative: data that are descriptive and cannot be expressed as a number

  Example: Personal stories and quotes to provide context and depth to those statistics

  - *"It took me many years to realize that something was wrong – this was my 'normal' but I didn't consider that it wasn't normal at all. Once I decided to see a doctor when I was lucky enough to have insurance, things moved quickly although I've had an assortment of diagnoses over the years."* [8]

  - *"I thought [the chemotherapy] might kill me all on its own… it was the most difficult time in my life and I thought the end was near."*[9]

Step 2. Synthesize Information

Synthesizing information can be the most challenging part of this process, but is the most crucial. The information that you choose to highlight is the information that FDA may ultimately use to inform its processes. As a result, it is not only important to extract information that is reflective

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[7] See Additional Resources section for more information on Unite Narcolepsy's patient-focused narcolepsy survey Interim Analysis as of September 16, 2013
[8] See Additional Resources section for more information on Unite Narcolepsy's patient-focused narcolepsy survey Interim Analysis as of September 16, 2013
[9] http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM379698.pdf

and representative of the range of patient responses, but also to present the information in a way that clearly conveys these important points.

Stakeholders beyond FDA may also find the information you have collected to be useful for educational purposes and/or helpful in informing their own processes. Therefore, translating the information into multiples formats so that it may be communicated in several ways will enhance the dissemination of the findings.

Issue a public report

The report should emphasize the findings from the survey and other information collection activities you decide upon on data collection in Stage 1. Where open text survey questions were used or verbal responses from focus groups or targeted interviews were captured, summarize or include representative quotes.

Draft letters intended for FDA

In addition to the public meetings, FDA will also invite the public to submit responses to what is referred to as the docket during public comment periods. The information gathered could be summarized into brief paragraphs that could be inserted into comment letters. Participating groups, organizations, and individuals should be notified of these opportunities to submit comments to FDA and encouraged to do so.

Develop talking points for organization leaders and other relevant stakeholders

To convey a consistent message to FDA and other interested stakeholders, create a set of talking points to be disseminated to the individuals, organized groups, and networks that represent the patient community.

Stage 3. Sharing Information

In this final stage, there are two remaining steps to ensuring that the information you have collected and translated is effectively shared with the FDA and other stakeholders. These steps are outlined below:

Step 1. Develop Strategies for Sharing Information at PFDD meeting
Step 2. Develop Strategies for Sharing Information Following PFDD meeting

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Step 1. Develop Strategies for Sharing Information at PFDD meeting

At the PFDD meeting, there will be opportunities to present oral or written testimony. Potential speakers or representatives should be identified and prepared for a presentation of your findings at the PFDD meeting.

To ensure a feedback loop to patients who participated in your information collection activities, produce a bulletin or newsletter following the PFDD meeting summarizing the proceedings, recapping the findings, testimonials, and other information shared with FDA.

Step 2. Develop Strategies for Sharing Information Following PFDD meeting

The information you collect through this effort will be useful in settings other than the FDA. There may be several opportunities for you to share the findings from this activity to help inform other important decisions and processes or to educate various audiences and raise awareness on the disease or condition.

Engage in public opportunities

Many organizations hold various forms of public engagement opportunities, such as comment periods or public meetings, to gather external information and input. Oftentimes these organizations will solicit information on a specific topic or feedback on a document they created. It will be important to monitor the environment for relevant public opportunities in which you may be able to engage.

Present findings at workshops, conferences, and other meetings

Workshops, conferences, and other meetings offer the opportunity to proactively share findings with various audiences. Encourage partner organizations to seek opportunities to present at these venues and engage in related activities such as participating on panel of speakers or joining roundtable discussions where participants share their thoughts and expertise on a particular topic.

Target engagement efforts with interested stakeholders

Stakeholders who may be interested in partnering or collaborating with you or your organization to learn more about your patient population may approach you. Consider reaching out to potential stakeholders who you feel may benefit from the information you have collected. Examples of potential targets include researchers or pharmaceutical/device manufacturers who are seeking to inform their own research activity.

Explore options and vehicles for dissemination

Beyond using the established patient organizations networks for disseminating information, you may consider expanding and developing additional pathways and strategies for dissemination. Examples of potential options may be to partner with governmental agencies, industry, and the media to launch an organized dissemination effort.

Request in-person meetings with FDA

FDA is open to meeting with patient groups to engage in dialogue, listen to concerns, and to promote further discussions. Under appropriate circumstances, requesting an in-person meeting with FDA may be an effective way to communicate findings in a more personal and in-depth manner than in public forums.

Additional Resources

About the Food and Drug Administration (FDA)

- FDA Home Page: http://www.fda.gov/
- About the FDA: http://www.fda.gov/AboutFDA/
- Patient Representative Program: http://www.fda.gov/forconsumers/byaudience/forpatientadvocates/patientinvolvement/ucm123858.htm
- Instructions for Using Regulations.gov – How to Search for FDA Federal Registers, Dockets and Daily Listings: http://www.fda.gov/RegulatoryInformation/Dockets/Comments/ucm089193.htm
- FDA Advisory Committee Meeting Calendar: http://www.fda.gov/AdvisoryCommittees/Calendar/default.htm

About the Patient-Focused Drug Development (PFDD) Program

- FDA Patient Network: http://patientnetwork.fda.gov/
- FDA PFDD: http://patientnetwork.fda.gov/patient-focused-drug-development-meetings

- FDA Federal Register Notice for Narcolepsy Public Meeting on Patient- Focused Drug Development (July 19, 2013): http://www.gpo.gov/fdsys/pkg/FR-2013-07-19/pdf/2013-17327.pdf

About Unite Narcolepsy

- Unite Narcolepsy Home Page: http://www.unitenarcolepsy.org/
- Unite Narcolepsy Patient-Focused Narcolepsy Survey Interim Analysis as of September 16, 2013: http://www.unitenarcolepsy.org/wp-content/uploads/Interim-Survey-Analysis-v1.pdf