

**Food and Drug Administration Safety and Innovation Act (FDASIA)
Section 1137:**

**Patient Participation in Medical Product Discussions
Report on Stakeholder Views**

**Food and Drug Administration
Department of Health and Human Services**

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Table of Contents

Introduction.....	2-3
Summary of Stakeholder Comments.....	3-5
Conclusion.....	6
Appendix A Highlights of Current FDA Patient Engagement Programs and Activities.....	7-10
Appendix B Contact Information for FDA Patient Engagement Programs.....	11
Appendix C Food and Drug Administration Safety and Innovation Act (FDASIA), Section 1137.....	12

Introduction

FDA has long involved patients and considered patient perspectives in its work. Recent activities aim to expand and better integrate the role of patient perspectives in regulatory decision-making over the total product lifecycle.

On July 9, 2012 the President signed into law the [Food and Drug Administration Safety and Innovation Act \(FDASIA\)](#) (Public Law 112-144). FDASIA expands the Food and Drug Administration's (FDA or Agency) authorities and strengthens the Agency's ability to safeguard and advance public health in numerous ways, including by:

- Giving the authority to collect user fees from industry to fund reviews of innovator drugs, medical devices, generic drugs, and biosimilar biological products;
- Promoting innovation to speed patient access to safe and effective products;
- Increasing stakeholder involvement in FDA processes; and
- Enhancing the safety of the drug supply chain.

Section 1137 of FDASIA, [Patient Participation in Medical Product Discussions](#), directs the Secretary of Health and Human Services to: *develop and implement strategies to solicit the views of patients during the medical product development process and consider the perspectives of patients during regulatory discussions, including:*

1. *Fostering participation of a patient representative who may serve as a special government employee in appropriate Agency meetings with medical product sponsors and investigators; and,*
2. *Exploring means to provide for identification of Patient Representatives who do not have any, or have minimal, financial interest in the medical products industry.*

Section 1137 strengthens FDA's ability to safeguard and advance public health for patients in the Agency's activities. The statute recognizes the value of patient input by facilitating increased involvement of patients earlier in the regulatory process for medical product review.

To plan for the implementation of FDASIA Section 1137 and summarize FDA's current program functions that address patient participation, FDA formed a work group in the Spring 2013 to discuss current programs and activities and consider strategies across the Agency for building upon current patient participation in accordance with the statute. The group was comprised of representatives from FDA Centers responsible for human medical product regulation and the Office of the Commissioner. Members include:

- Center for Biologics Evaluation and Research (CBER)
- Center for Devices and Radiological Health (CDRH)
- Center for Drug Evaluation and Research (CDER)
- Office of the Commissioner (OC)

The work group identified current activities ongoing within their Center/Office as well as future plans for developing patient engagement activities. Beyond this, the work group sought input from the public and on December 4, 2014, FDA issued a [Federal Register \(FR\)](#) notice establishing a docket ([FDA-2014-N-1698](#)) for public commenters to submit information related to FDA's implementation of FDASIA's Patient Participation in Medical Product Discussions under FDASIA section 1137. The [FR Notice](#) announced FDA's intent to gather input from stakeholders on strategies to obtain the views of patients during the medical product development process and to identify ways to consider patients' perspectives during regulatory discussions.

A summary of FDA's ongoing programs and activities that involve patient participation in medical products is found in Appendix A. Also, contact information for those FDA offices responsible for patient-oriented engagement programs is included in Appendix B.

Summary of Stakeholder Comments

There were 185 comments received representing individual patients, patient advocacy groups, and industry trade organizations.

Five major themes emerged from the stakeholders' comments; these are, in no order of priority or preference:

- Systematic Patient Engagement
- Transparency and Communication
- Clarification of FDA policies
- Clinical Investigations
- Workshops and Partnerships

The comments are summarized under each of these themes below.

Systematic Patient Engagement

- Several comments stated that FDA should have a prominent and centralized office to advise the FDA Commissioner on patient engagement activities and systematically implement a coordinated function. The office should work across Centers, be knowledgeable about each Center's processes, and be able to facilitate patient engagement activities based upon the Commissioner's direction. The stakeholders recommended that the Office of Health and Constituent Affairs' role be expanded to serve as such an office so that internal and external processes for patient engagement are better established.
- Multiple comments recommended that FDA establish an external advisory group to provide ongoing counsel about input to and monitoring of patient participation in regulatory processes and policy development. Such an advisory body would be positioned to advise on patient engagement strategies across FDA's Centers and would not be limited to individual product decisions. The advisory body could offer perspectives on how to foster greater inclusion of patient engagement in regulatory decision-making for product review, post-market requirements, direct-to-consumer promotion, risk communication, and safety surveillance.
- One comment stated that early and iterative engagement can influence clinical and regulatory understanding of diseases and conditions, provide a common understanding of the most urgent needs, and inform drug development programs. The commenter stated that it is likely that patient input on clinical trial design may improve patients' participation and engage throughout the drug development process.

Transparency and Communication

Transparency

- Comments recommended that FDA develop a regular report summarizing the patients' participation in the Patient Representative Program since passage of FDASIA. The report should detail the level of patient engagement, the staffing levels within FDA dedicated to facilitating patient engagement, as well as information detailing the progress or challenges identified.

- Multiple comments also stated that FDA should improve transparency on how patient input is evaluated and incorporated into the Agency’s decision-making process for medical products.
- Comments went further to recommend FDA establish a systematic approach to consistently incorporating patient preferences into the regulatory decision-making process.

Communication

- One comment recommended developing a patient data collection tool that systematically organizes issues by disease area and identifies topics to facilitate communications between patient organizations and FDA.
- Comments suggested that FDA should expand the FDA’s Patient Network program to encourage patients to contribute effectively by providing patient-oriented materials.
- A comment stated that FDA should continue to develop multiple modes of communication styles and tools. Some examples were webinars, round tables and tele-townhall meetings to facilitate cross talk among researchers, clinicians, industry and patients to help speed meaningful development programs responsive to patients’ needs.
- Also other comments said FDA should enhance its patient portal allowing greater two-way communication to hear from patients about their experiences with FDA-regulated products, particularly products such as biosimilars.

Clarification of FDA Policies

Manufacturers

- Comments suggested FDA provide a mechanism for interacting with a sponsor early in the product’s development process to discuss patient preferences.
- Multiple commenters recommended FDA develop guidance on interactions between patients and manufacturers to facilitate collaboration in the early stages of research and development and across the lifecycle of product development, clinical trial design, endpoint selection, and patient reported outcomes.
- A comment requested FDA issue guidance describing the appropriate parameters and regulatory/legal safe-harbor for sponsor engagement with patient groups during medical product development. The commenter stated that one perceived barrier to manufacturers’ engagement with patient groups during medical product development is that FDA may interpret such dialogue as promotion of an investigational product. It was stated that FDA could clarify its policies on outreach to patient groups. The clarification would help to understand FDA’s perspectives on the design and conduct of a particular clinical development program and/or its perspectives on whether outreach on benefit-risk and meaningful clinical outcomes constitutes promotion or marketing of an unapproved investigational product or indication thus potentially subject to enforcement.

Patients

- Several comments stated FDA could offer guidance on the types of patient information that would be useful to the Agency.
- Comments noted that access to FDA by patient groups is crucial and a defined mechanism to engage with FDA should be provided that would describe and define patient engagement and outline patient engagement methods.

Clinical Investigations

- Multiple commenters recommended FDA expand use and adoption of measures of patient-centered patient-reported outcomes (PROs) such as physical function and quality of life, much like what the European Medicines Agency has done in its Patient-Reported Outcome (PRO) qualification process.
- Several comments recommended that FDA collaborate with patient organizations to determine methods for conducting shorter, hypothesis-driven, novel trials that evaluate biomarkers as surrogate endpoints to more expeditiously develop effective therapeutics.

Workshops and Partnerships

- A few comments recommended that FDA increase public-private partnerships (PPP) and establish, provide or collaborate via forums among patients, industry, clinicians, the scientific community and FDA. Several existing groups were identified which are doing work which FDA could leverage, including the [Medical Device and Innovation Consortium \(MDIC\)](#), the [Clinical Trials Transformation Initiative](#), [National Health Council](#), [National Organization for Rare Disorders](#), [FasterCures](#), [C-PATH Patient-Reported Outcome \(PRO\) Consortium](#), other C-PATH Consortia, the [Patient Centered Outcomes Research Institute \(PCORI\)](#) or other disease-focused consortia. Focus areas could include:
 - Work towards developing methodologies and study protocols for obtaining patients views of their conditions and benefit-risk assessment. An overarching goal would be to develop a standardized, repeatable, and representative pre-competitive data collection model that could be used across FDA divisions.
 - Work to provide a preliminary framework to facilitate patient involvement earlier in human therapeutic product development. This work could inform key decisions about a particular development program, such as more effective study recruitment and enrollment strategies, the development of surrogate or intermediate clinical endpoints, and the establishment of qualified patient-reported outcomes.

Conclusion

FDA continues to involve patient and caregiver perspectives in its work. We appreciate the comments and concerns submitted by stakeholders, and recognize the significant work reflected in the suggested recommendations to better engage patients in regulatory medical product matters. The range and quality of comments received will assist FDA to consider steps for improving patient involvement. The cross-Agency work group continues meeting to discuss implementation activities. The Centers/Offices continue to develop and implement strategies to include patient and caregiver perspectives in a variety of activities which are increasingly being implemented. Finally, FDA will develop a comprehensive plan for implementation of FDASIA section 1137, outlining the approaches to increase patient involvement across FDA regulatory discussions.

FDA will continue to solicit the views of patients during the medical product development process and during regulatory discussions. FDA expects to maintain open communication with patients, caregivers, patient advocates and other stakeholders, as we further implement FDASIA section 1137, expanding and better integrating patient perspectives in regulatory decision-making throughout the total product lifecycle.

Appendix A

Highlights of Current FDA Patient Engagement Programs and Activities

Over the years, FDA has developed programs and initiatives for patients and families to have a variety of opportunities to provide input into FDA decision-making. The following are highlights of key programs and initiatives.

FDA Patient Representative Program

The [FDA Patient Representative Program](#) is managed by the Agency's [Office of Health and Constituent Affairs \(OHCA\)](#) within the Office of the Commissioner. OHCA coordinates the Agency's recruitment, training, and retention for over 200 Patient Representatives (PRs), who are patients or primary caregivers to patients. These PRs are knowledgeable and experienced in over 300 diseases and conditions and participate on 47 FDA [Advisory Committees \(AC\)](#) and panels, and in review division meetings. These PRs provide direct input to inform the Agency's decision-making associated with medical products for drugs, biologics, and medical devices.

Unlike other AC members, FDA's selection of patients serving involves identifying those with direct experience with the disease. Usually this means that a PR is specific to the AC meeting topic. Also, PRs serve in review division meetings and FDA workshops. Requests for PR involvement in FDA regulatory meetings continues to increase to actively implement FDASIA section 1137. Table 1 lists the number of ACs and review division assignments (DA) where PRs have served by year.

Table 1. FDA Patient Representatives serving on Advisory Committees (AC) and Review Division Assignments (DA)

YEAR	ACs	DAs
2012	27	4
2013	66	11
2014	55	12
2015	42	17

FDA Patient Network

The [FDA Patient Network](#) was created in 2012 as a one-stop-shop of FDA resources to help patients and their families find relevant information from FDA's website. The Patient Network provides patient-oriented educational resources about medical product regulations aimed to help patients learn about FDA, how to interact with the Agency and to become involved more effectively in regulatory decisions related to medical product safety and approval.

The Patient Network website includes real-time information about upcoming open public meetings, current and archived webinars on relevant patient-oriented topics, information on the process for submitting comments to FDA on draft policies and guidances, as well as a patient-friendly portal for patients to input their comments. The Network also has a bimonthly newsletter, *Patient Network News*, with over 50,000 subscribers. The newsletter features current information on product approvals, significant new safety concerns on already approved medical products, and other topics for patients and patient advocates.

Patient Reported Outcomes (PRO)

Patient reported outcomes are used in clinical studies reviewed by the Center for Biologics Evaluation and Research (CBER), the Center for Devices and Radiological Health (CDRH), and the Center for Drug Evaluation and Research (CDER).

A [patient reported outcome \(PRO\)](#) is a direct response from the patient regarding his/her health condition, without an intermediary's interpretation (e.g., clinician or caregiver). A PRO instrument can be developed to measure a patient's outcome such as: symptom severity, sign, or state of a disease status or as a change from a previous measure. In clinical studies, a PRO instrument is meant to measure the effect of a medical intervention on one or more concepts (i.e., the *thing* being measured, such as a symptom or group of symptoms, effects on a particular function or group of functions, or a group of symptoms or functions shown to measure the severity of a health condition). Therefore, understanding and learning from patients' perspectives is an important step towards developing instruments that measure outcomes important to patients. Integrating these measures into the design of a clinical study is one method to enhance a patient's assessment of a medical product's effects.

A description of methods for use and adoption of well-validated PRO instruments are available at:

- [Medical Device Development Tools](#)
- [Clinical Outcome Assessment Qualification Program](#)

Another tool to help drug developers consider the issues and points when planning their drug development programs may be found at [Roadmap to Patient-Focused Outcome Measurement in Clinical Trials](#).

Patient Focused Drug Development Initiative (PFDD)

The Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Review (CBER) are committed to successfully implementing the [Patient Focused Drug Development \(PFDD\)](#) initiative. This initiative has been implemented to more systematically obtain patient perspective on certain diseases and their treatments as part of an FDA commitment under the authorization of the Prescription Drug User Fee Act V (PDUFA V).

FDA disease areas selected were based on comments received in a public docket. Comments addressing over 90 disease areas were submitted by patients, patient advocates and advocacy groups, caregivers, healthcare providers, professional societies, scientific and academic experts, pharmaceutical companies, and others. This public input informed further review and consideration by FDA new drug review divisions to identify the list of diseases for the series of PFDD meetings. After each PFDD meeting FDA publishes a meeting report, [The Voice of the Patient](#), with proceedings and summary analysis of the input received by FDA relevant to FDA's consideration of disease severity and unmet medical need.

Professional Affairs and Stakeholder Engagement Staff (PASES)

The Center for Drug Evaluation and Research's (CDER) [Professional Affairs and Stakeholder Engagement Staff \(PASES\)](#) was created in October 2013 to provide a focal point for advocacy and to enhance two-way communication and collaboration with healthcare professionals, patients, patient groups, and others on CDER issues concerning drug development, drug review, and drug safety. Internal and external outreach enables CDER to better understand stakeholders' perspectives on disease states, which aspects of the disease and treatments matter the most to patients, and the risks patients are willing to accept for access to effective treatments.

Patient Perspectives in Benefit-Risk Determinations for Medical Devices

FDA's 2012 guidance document, [Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications](#), by the Center for Devices and Radiological Health (CDRH), and Center for Biologics Evaluation and Research (CBER), considers patient perspectives on risk and benefit during the premarket review process for premarket approval applications (PMAs) and *de novo* classification requests. It also integrates patient preferences into the regulatory review process when the information meets FDA's standards for valid scientific evidence.

Device Patient Preference Initiative

The [Patient Preference Initiative](#) provides information, guidance, and the framework necessary to incorporate the patient voice into the full spectrum of medical device development and regulatory processes. The initiative aims to advance the science of measuring patient preferences to inform benefit-risk assessments used in regulatory decision-making. In May 2015, a draft guidance [Patient Preference Information - Submission, Review in PMAs, HDE Applications, and De Novo Requests, and Inclusion in Device Labeling](#) was released that encourages voluntary submission to FDA of patient preference information, describes characteristics of quality

patient preference studies which may be considered valid scientific evidence, and includes recommendations for collecting patient preference information and communicating it to patients and healthcare professionals.

Also in May 2015, a report was released by the MDIC which outlined how patient preference information can be used throughout the total product lifecycle, and which catalogued methodologies that may be used to assess patient preferences and incorporate this information into product development and assessment. The framework report and catalog can be found at <http://mdic.org/pcbr/>.

Patient Engagement Advisory Committee (PEAC)

In 2015, the PEAC was established to help assure the needs and experiences of patients are incorporated into FDA's work, by providing advice to FDA on complex issues relating to medical devices, the regulation of devices, and their use by patients. The PEAC may consider topics such as: Agency guidance and policies, clinical trial or registry design, patient preference study design, benefit-risk determinations, device labeling, unmet clinical needs and available alternatives, patient reported outcomes and device-related quality of life or health status issues, and other patient-related topics. The PEAC will advise CDRH on ways to include and foster participation of patients where appropriate throughout the total product lifecycle, and on will provide perspectives about current and new approaches or policies for integrating patient input in regulatory decision-making. The PEAC will also serve as a resource to CDRH as a body of experts in patient experience, needs, and the activities of the patient community.

Appendix B

Contact Information for FDA Patient Engagement Programs

Office of Health and Constituent Affairs
FDA Patient Network
Phone: 301-796-8460
Email: PatientNetwork@fda.hhs.gov

Center for Drug Evaluation and Research
Division of Drug Information
Phone: 855-543-3784
Email: druginfo@fda.hhs.gov

Center for Biologics Evaluation and Research
Consumer Affairs Branch
Phone: 800-835-4709
Email: ocod@fda.hhs.gov

Center for Devices and Radiological Health
Division of Industry and Consumer Education
Phone: 800-638-2041
Email: DICE@fda.hhs.gov

Appendix C

Food and Drug Administration Safety and Innovation Act (FDASIA), Section 1137

SEC. 1137. PATIENT PARTICIPATION IN MEDICAL PRODUCT DISCUSSIONS.

Subchapter E of chapter V (21 U.S.C. 360bbb et seq.), as amended by section 1123 of this Act, is further amended by adding at the end the following:

“SEC. 569C. PATIENT PARTICIPATION IN MEDICAL PRODUCT DISCUSSION.

“(a) **IN GENERAL.**—The Secretary shall develop and implement strategies to solicit the views of patients during the medical product development process and consider the perspectives of patients during regulatory discussions, including by—

“(1) fostering participation of a patient representative who may serve as a special government employee in appropriate agency meetings with medical product sponsors and investigators; and

“(2) exploring means to provide for identification of patient representatives who do not have any, or have minimal, financial interests in the medical products industry.

“(b) **PROTECTION OF PROPRIETARY INFORMATION.**—Nothing in this section shall be construed to alter the protections offered by laws, regulations, or policies governing disclosure of confidential commercial or trade secret information and any other information exempt from disclosure pursuant to section 552(b) of title 5, United States Code, as such laws, regulations, or policies would apply to consultation with individuals and organizations prior to the date of enactment of this section.

“(c) **OTHER CONSULTATION.**—Nothing in this section shall be construed to limit the ability of the Secretary to consult with individuals and organizations as authorized prior to the date of enactment of this section.

“(d) **NO RIGHT OR OBLIGATION.**—Nothing in this section shall be construed to create a legal right for a consultation on any matter or require the Secretary to meet with any particular expert or stakeholder. Nothing in this section shall be construed to alter agreed upon goals and procedures identified in the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2012. Nothing in this section is intended to increase the number of review cycles as in effect before the date of enactment of this section.

“(e) **FINANCIAL INTEREST.**—In this section, the term ‘financial interest’ means a financial interest under section 208(a) of title 18, United States Code.”.