

## Patient Engagement Collaborative Meeting Summary

August 29, 2018  
FDA White Oak Campus  
10903 New Hampshire Avenue, Silver Spring, Maryland

### OBJECTIVES

- Launch the Patient Engagement Collaborative (PEC), an ongoing forum to discuss how to achieve more meaningful patient engagement in medical product development and other regulatory discussions;
- Align on vision and goals for the PEC; and
- Discuss opportunities to enhance communications and resources for interactions between the FDA and the patient community.

### KEY TAKEAWAYS

Over the course of the meeting, PEC members discussed several key takeaways for further consideration:

- Patient engagement can inform the research and development continuum from beginning to end, including the regulatory decision-making process.
- A key challenge for the FDA is a general lack of knowledge among the public and patients about the regulatory decision-making process, the FDA's role in the process, and the agency's structure and organization.
- The patient community is eager to engage with the FDA throughout the regulatory decision-making process. Patients and advocates may struggle with knowing how to engage, what kinds of information to provide, and how to ensure that patients' perspectives are adequately represented.
- Potential goals for the PEC could include (1) enhancing patient access to high-quality information about the FDA, regulatory decision-making, medical product approvals, and clinical trials; and (2) developing strategies for meaningful incorporation of patients and advocates into regulatory decision-making.
- Providing patients and advocacy groups with easy-to-understand resources about the FDA could increase engagement, build trust in the work of the FDA, and enhance the quality of communication between the FDA and the patient community.
- A systematic approach by the FDA to including the patient voice in the regulatory decision-making process will be important.
- Potential approaches to enhancing communication include offering patient-friendly information resources; training patient ambassadors to serve as trusted resources in their own communities; and using diverse communication modes to reach diverse patient populations, including vulnerable and underserved communities.

## BACKGROUND

The U.S. Food and Drug Administration (FDA), in collaboration with the Clinical Trials Transformation Initiative (CTTI), convened the inaugural meeting of the PEC on Wednesday, August 29, 2018, at the FDA's White Oak Campus in Silver Spring, Maryland. The PEC brings together 16 patient advocates representing diverse perspectives to discuss how to achieve more meaningful patient engagement in medical product development and related regulatory discussions. The PEC is being spearheaded by the FDA's new Patient Affairs Staff, which functions as a central point of access to information for the patient community and leads efforts to expand patient involvement in the FDA's regulatory work.

Andrea Furia-Helms, Director of the Patient Affairs Staff, welcomed the members of the PEC and introduced Dr. Rachel Sherman, Principal Deputy Commissioner. Dr. Sherman offered a brief history of patient engagement at the FDA—from the inclusion of patient advocates in advisory committees to, more recently, the FDA's patient-focused drug development efforts in accordance with the 21st Century Cures Act and the FDA Reauthorization Act of 2017. Dr. Sherman expressed optimism about the work of the PEC in helping to ensure that the FDA engages constructively with the patient community and incorporates patients' perspectives into its work.

## SESSION I: MEMBERS' VISION FOR THE PATIENT ENGAGEMENT COLLABORATIVE

### ***Members' Previous Experiences Engaging with the FDA***

The first session began with a discussion of members' previous experiences engaging with the FDA. These experiences included:

- participating in pre-investigational new drug (pre-IND) meetings;
- making presentations to review divisions and panel meetings;
- assisting in the development of clinical trial endpoints;
- sharing patient experience data, including results of post hoc analyses;
- submitting input for Voice of the Patient reports; and
- attending Critical Path Innovation Meetings.

Some members reported that their experience engaging with the FDA was limited while others indicated they had more experience in engaging with the agency.

Members agreed that their experiences with FDA representatives have been positive and that relationships between the FDA and patient advocates have strengthened over time. Discussion of members' previous experiences revealed opportunities for improving engagement and structuring the work of the PEC. In particular, several members highlighted as key challenges a lack of knowledge about the regulatory decision-making process and a lack of knowledge about the FDA's role and structure. Members discussed that providing general education about the FDA for patients and patient advocacy groups could increase engagement with the FDA by the

patient community, build trust in the work of the FDA, and enhance the quality of communication between the FDA and the patient community.

Other challenges identified by members, based on their previous experiences, included (1) a lack of consistency in the inclusion of patients and patient advocates throughout the regulatory decision-making process, especially inclusion by sponsors in pre-IND meetings and other review meetings; and (2) a need for better understanding by patient advocates of when involvement by the patient community is most useful and effective.

Members stressed that patient advocacy organizations differ in their resources, capabilities, and experience. Smaller organizations and those that are new to engaging with the FDA, especially, tend to need more education about the regulatory decision-making process and more guidance in engaging with the FDA.

### ***Short- and Long-Term Goals of the Patient Engagement Collaborative***

Discussion of potential short- and long-term goals of the PEC focused on two areas: (1) patient access to high-quality information about the FDA, regulatory decision-making, medical product approvals, and clinical trials; and (2) meaningful inclusion of patients and patient advocates into regulatory decision-making.

Members discussed that patients and patient advocates value access to credible information about the FDA and regulatory decision-making. Members expressed interest in helping to improve the accessibility of information on the FDA website, especially information about the structure and role of the agency, its advisory committees, and staff contacts. Also, because social media has changed how patients acquire information about diseases, treatment options, and research participation opportunities, members felt it was important for the FDA website to serve as a credible source of clear, up-to-date information prepared for a general audience. They discussed that the PEC could play a role in developing such information resources.

Members also expressed that the patient community is eager to engage with the FDA throughout the regulatory decision-making process. However, patients and advocates may struggle with knowing how to engage, what kinds of information to provide, and how to ensure that patients' perspectives are adequately represented. Some members felt that patients and advocates can play an important role in educating other stakeholders, such as sponsors and academic investigators, about the importance of patient engagement, appropriate inclusion of patient-reported outcomes and real-world evidence in clinical research, and incorporation of patient perspectives in research design and implementation. Members discussed their interest in developing information resources for patient advocates, sponsors, researchers, and others on the role of patient engagement in regulatory decision-making.

## SESSION II: ENHANCING COMMUNICATIONS BETWEEN THE FDA AND THE PATIENT COMMUNITY

### *Communications Challenges*

In the second session, members discussed enhancing communications between the FDA and the patient community and identified several communications challenges.

- Information prepared for a general audience. Members felt that the information currently available on the FDA website is written for an audience with scientific and technical expertise, rather than for a general audience.
- Transparency in receiving and acting on input. When patients and patient advocates provide comments to the FDA, it is not always clear what happens as a result.
- Appropriate access and input opportunities. Members noted that more information about when and how to engage in the regulatory decision-making process would be valuable. For example, patients and patient advocates appreciate opportunities to attend review meetings but do not always know when those meetings will be held.
- Needs of diverse populations. Members noted that different patient populations need different information and use different communications channels. For example, some populations have limited Internet access or limited access to health care services and information about research opportunities.

### *Potential Solutions*

Members discussed several potential approaches to addressing communications challenges, including:

- designing information resources for a more general audience, especially basic information about regulatory decision-making and the role and structure of the FDA;
- developing a single, patient-focused point of access for trustworthy information about the FDA, including opportunities for engagement;
- developing patient-focused information resources, such as an information toolkit and a website designed for easy navigation and accessibility;
- forming a group of trained advocates who can serve as trusted sources of information in their own communities;
- providing more information about FDA staff contacts; and
- improving processes for acknowledging the receipt of input from patients and patient advocates and describing how that input will be used in regulatory decision-making.

FDA attendees noted that the recently formed Patient Affairs Staff can function as a central point of access to information for the patient community. The Patient Affairs Staff is responsible for coordinating agency-wide projects related to patient engagement, facilitating awareness and collaboration with patients and patient advocates, and enhancing external

communication to build awareness about the FDA's patient engagement initiatives and regulatory activities.

Members noted that the FDA's patient-focused drug development guidance series could help to inform the PEC's work. In a four-part series, the FDA is offering guidance on enhancing the incorporation of the patient voice in medical product development and regulatory decision-making.

### SESSION III: ENHANCING RESOURCES FOR INTERACTIONS BETWEEN THE FDA AND THE PATIENT COMMUNITY

In the final session, members discussed what they wish they had known as patient advocates when engaging with the FDA for the first time, then offered ideas for potential resources that could support the patient community in interacting with the FDA.

Several members wished they had received training or information resources on engaging with the FDA, such as a toolkit or slide deck providing basic information about the agency and its role in regulatory decision-making. One idea that resonated with several members was to produce an equivalent of the *Schoolhouse Rock!* "I'm Just a Bill" video segment to explain the regulatory decision-making process for medical products. Other members mentioned that it would have been helpful to see case studies of effective engagement with the FDA by patient advocates, including lessons learned. FDA attendees showed the current "For Patients" section of the FDA website, and members further highlighted how the website and social media could become more accessible to a general audience.

Finally, FDA attendees presented information about a potential video series for patients and patient advocates. The individual videos would include an introduction to the series from FDA leadership and information about how patients and patient advocates can engage with the FDA. Members offered ideas for video content that would be helpful to the patient community, including case studies from patient advocates, a look inside the FDA with profiles of leadership and staff, and information about advisory committee meetings and other opportunities to participate.

### WRAP-UP AND NEXT STEPS

The meeting ended with a discussion of next steps and key takeaways. Members conveyed a sense of excitement and optimism about the opportunity to engage in a true partnership with the FDA to enhance patient engagement.

## DISCLAIMER

The views expressed in this meeting summary represent the individual perspectives of the attendees and do not necessarily represent the official views of the FDA or CTTI or of any organization with which the attendees are affiliated.

## MEETING ATTENDEES

### Patient Engagement Collaborative

Ron Bartek	Friedreich's Ataxia Research Alliance
Karen Erickson	Alpha-1 Foundation
Jeff Goldstein	Lung Transplant Foundation
Anne Hall	Independent patient advocate
Melissa Hogan	Project Alive
Dawn K Aldrich	SOLUTIONS Cancer Resource Center, Inc.
Nancy Lenfestey	St. Baldrick's Foundation
Isabelle Lousada	Amyloidosis Research Consortium
Stephanie Monroe	African Americans Against Alzheimer's, a network of UsAgainstAlzheimer's
Rick (Lawrence) Phillips	Independent patient advocate
Phil Posner	Independent patient advocate
Adrienne Shapiro	Axis Advocacy
Theresa Strong	Foundation for Prader-Willi Research
Dave White	Kidney Health Initiative
Elizabeth Joniak-Grant*	Independent patient advocate

### Food and Drug Administration

Meghana Chalasani	Center for Drug Evaluation and Research
Susan Chittooran	Office of the Commissioner
Andrea Furia-Helms	Office of the Commissioner
Nina Hunter	Office of the Commissioner
Karen Jackler	Center for Biologics Evaluation and Research
Laura Lee Johnson*	Center for Drug Evaluation and Research
Diane Maloney	Center for Biologics Evaluation and Research
Kristen Miller	Center for Drug Evaluation and Research
Lisa Miller	Center for Devices and Radiological Health
Theresa Mullin	Center for Drug Evaluation and Research
Lei Nie	Center for Drug Evaluation and Research
Katie O'Callaghan	Center for Devices and Radiological Health
Chinyelum Olele	Center for Devices and Radiological Health
Annie Saha*	Center for Devices and Radiological Health
Samir Shaikh*	Office of the Commissioner
Rachel Sherman	Office of the Commissioner
Lauren Spicher	Office of the Commissioner
Michelle Tarver	Center for Devices and Radiological Health
Pujita Vaidya	Center for Drug Evaluation and Research

### Clinical Trials Transformation Initiative (CTTI)

Zachary Hallinan	CTTI
Damon Seils	Duke Clinical Research Institute
Pamela Tenaerts	CTTI

\* Participated by telephone.

## MEETING AGENDA

9:15 am	Welcome and Background
9:30 am	Background and Introductions
10:00 am	Break
10:15 am	Session I: Members' Vision for the Patient Engagement Collaborative
11:30 am	Lunch
12:30 pm	Session II: Enhancing Communications between the FDA and the Patient Community
1:45 pm	Break
2:00 pm	Session III: Enhancing Resources for Interactions between the FDA and the Patient Community
3:30 pm	Wrap-up and Next Steps
4:00 pm	Adjourn