Joint Meeting of the FDA/CTTI Patient Engagement Collaborative (PEC) and EMA Patients and Consumers Working Party (PCWP)

July 1st, 2021 | 10:00am – 12:30pm ET

SUMMARY

The purpose of this virtual meeting was to facilitate discussion and sharing of ideas between members of the Food and Drug Administration/Clinical Trials Transformation Initiative (FDA/CTTI) Patient Engagement Collaborative (PEC) and the European Medicines Agency's (EMA) Patients and Consumers Working Party (PCWP) around topics related to patient engagement. Meeting discussions highlighted, as demonstrated by the experiences of both the PCWP and the PEC, the importance of collaboration between regulatory agencies, health organizations, health providers, patient groups, and communities. This includes engaging young people in relevant discussions.

Overview of PCWP and PEC

Overview of the PCWP

The first presentation, by PCWP co-chair Juan Garcia-Burgos, reviewed the structure and history of the PCWP, which brings together a community of patient representatives in Europe and facilitates discussions on issues of common interest between the EMA, patients, and consumers. These discussions give the EMA advance insight into the concerns of patients and healthcare providers, enabling early integration. To gather these perspectives, EMA, via the PCWP, collaborates with various non-profit patient and consumer organizations across Europe.

According to the PCWP presenter, the group currently consists of 30 members—22 representatives of patient or consumer organizations, 6 representatives appointed by EMA scientific committees, 1 co-chair elected from the patient representatives, and 1 co-chair nominated by the EMA. There are also observers from the European Commission, EMA's Management Board and the Healthcare Professionals Working Party (HCPWP). PCWP members are nominated for a term of three years, which may be renewed. During their term, members follow the co-developed PCWP mandate, rules of procedure, and 3-year work plan—publicly published on the EMA website. They also attend up to 4 meetings per year, are consulted on specific EMA cases, and contribute to EMA workshops and other public consultations.

The PCWP hosts a variety of workshops and information sessions and facilitates topic group discussions on specific EMA considerations. Members of the PCWP also contribute directly to EMA endeavors and strategies. The presenter from the PCWP noted that the organization collaborates closely with the HCPWP to share information, ideas, and perspectives concerning issues of medicine and public health.

Overview of the PEC

The second presentation, by PEC member Stephanie Monroe, reviewed the development of the PEC, a collaboration between the FDA and CTTI that was modeled after the work done by the EMA's PCWP. The PEC's main purpose is to facilitate discussions about ways to engage patients in medical product development and regulation. PEC representatives include patients, caregivers, and members of patient

advocacy groups. Up to 16 diverse PEC representatives may serve at once, and terms are staggered to ensure continuity. PEC representatives meet virtually on a monthly basis and in person 2-4 times a year to discuss a range of topics related to engaging patients.

The PEC presenter stated the inaugural PEC meeting was in 2018, and it included discussions on increasing patient engagement with FDA activities, filling in patient knowledge gaps, and building trust between patients and the FDA. Sharing another example of PEC discussions, she highlighted a March 2020 meeting topic on patient concerns surrounding the conduct of clinical trials during the COVID-19 pandemic, noting that the insights both informed CTTI activities and were shared by FDA within medical product centers.

Discussion

Discussion between PEC and PCWP members included the following:

Are PCWP members included in discussions of medicine development?

- The PCWP is primarily involved in more general discussions, not related to specific medicines. The EMA has separate procedures for seeking patient input on medicine development and evaluation. Members of the PCWP may assist EMA finding patients to consult.

How does the PCWP raise visibility of patient engagement?

- A PCWP topic group was convened to discuss how to raise awareness of the ways patients can get involved in EMA activities. Potential ideas included: patient organizations to spread awareness directly to their patient communities, reaching out through social media, and providing certificates of attendance for workshops and information sessions. Meeting reports are also shared widely with patient organizations, which helps to stimulate interest in patient engagement.

In addition to planned initiatives, does the PCWP also have unplanned cases that they consult on?

- Yes, sometimes the PCWP is asked for a quick consultation—often in writing—on specific topics. For example, members were asked to test and provide quick feedback on COVID-19 communications.

What responsibilities do the two PCWP co-chairs take on?

- The PCWP co-chairs are primarily responsible for contributing to meeting agendas and chairing the meetings with the members of their respective groups.

What does patient equity mean to the PEC?

- Patient equity means that underrepresented individuals understand the presence of disease and the opportunities for disease prevention, have access to good and early diagnosis and treatment, and are able to enroll in clinical trials. Patients in rural or less populated areas tend to have less access to clinical trials, and PEC members are interested in how medical access and information in these communities can be improved. PEC members are also interested in finding ways to embed equity in clinical trials and directly involve diverse patients in drug development conversations.

Is the PEC formally involved in the public hearings system?

- The PEC is not formally involved in public hearings, but individual members and their associated patient advocacy groups often participate in public hearing advisory boards.

Exchange of Experiences on Patient Information and Communication

Overview of Patient Involvement in EMA Public Communications

Next, EMA's Maria Mavris, discussed the organization's established practices for requesting patient review of certain EMA communications, including medicine overviews, safety communications, package leaflets, and herbal summaries. Patient reviewers are asked to evaluate whether the content of the document is clear and features language that is not overcomplicated or oversimplified. When taking in patient comments, the medical writers consider how the suggested changes would affect the rest of the document. Overall, about 50% of patient comments are incorporated into the final documents.

Projects on Development and Review of Communications

A member of the PEC, David White, presented the work the organization has done to engage patients. The PEC has discussed the importance of informing, engaging, and empowering patients and communities through education and outreach, and has worked closely with the FDA to help design and disseminate patient-facing educational resources. Social media and public-facing website resources are also an important part of how the FDA communicates directly with patients.

Discussion

Discussion between PEC and PCWP members included the following:

What reading level does the EMA develop their patient-facing communications for? What platforms do these communications come out on?

- The EMA doesn't necessarily aim their materials at any particular age level, but rather they work with the participants to make sure the materials are clear. The EMA has online training materials and an annual hands-on training day for patients interested in engaging with EMA activities.
- The EMA does communicate with patients via social media and direct emailing. The EMA often tries to use a variety of media (e.g., info sheets, emails, videos) to communicate information.

Does the EMA have a formalized structure for soliciting patient feedback on communication documents?

- The EMA's patient review process has been developed and built up over the years. There is a very large pool of eligible patient organizations that are the EMA's first resource for finding patients to review documents.

How does the FDA reach the patients who need this information the most?

- The FDA has the Office of Minority Health and Health Equity that reviews communications and assists with distribution to underrepresented communities.

Does the EMA utilize the PCWP when seeking patient reviews?

- The members of the PCWP are from the eligible patient organizations. When we reach out to these patient organizations to identify reviewers, those patient reviewers are involved as individuals and must sign a declaration of interest form.

Youth Engagement

Young Patient Engagement at EMA and European Patients' Forum (EPF)

PCWP co-chair and EPF Director of Policy, Kaisa Immonen, presented EMA's initiatives to include the voices of young patients, who can often provide their own unique perspectives, in patient engagement activities. In 2017, the EMA published "Principles on the involvement of young patients/consumers within EMA activities" to provide a framework for engaging young patients in discussions surrounding

medicine development and public health. These guidelines highlight that interactions with young patients should be tailored based on age and specific vulnerabilities.

The PCWP presenter also presented how the EPF, which represents patients with a variety of conditions from across Europe, has established an EPF Youth Group that currently consists of 11 members ranging from age 18 to 30. An EPF Youth Board Representative has a formal seat on the EPF Board and represents the interests of the Youth Group in all Board discussions. The goals of the EPF Youth Group are to strengthen the involvement and representation of young patients in patient organizations, promote young patients' rights, needs, and expectations within health policy, encourage better integration of young patients within society, and empower young patients to develop leadership and advocacy skills, self-efficacy, and confidence. Capacity-building is a major focus, particularly through the annual Training for Young Patient Advocates. The EMA often interacts with the EPF Youth Group through workshops and talks that serve to familiarize young patients with the EMA's resources and opportunities for engagement.

Insights from FDA Listening Session with Transgender Youth

FDA Patient Affairs' Susan Chittooran, presented the results of the organization's recent listening sessions with transgender youth. Listening sessions allow patients and advocates to share their health experiences and talk directly with FDA staff. These sessions are non-public and non-advisory discussions meant to facilitate the sharing of patient perspectives. The FDA held listening sessions with transgender adults and transgender teens who are contemplating, currently undergoing, or have already completed transition to their affirmed gender (via hormone therapy or gender-affirming surgery). The goals of these sessions were to understand the barriers to obtaining healthcare, the areas of unmet medical need, and the goals related to gender (medical) transition.

The FDA presenter noted that in planning for these listening sessions, the FDA had to make sure to adapt the language used in outreach communications, especially since the original language was primarily developed for sessions with patients with a specific disease or condition. The language also needed to be age appropriate for individuals as young as 13, and it needed to incorporate gender-affirming terminology (such as using the participant's preferred pronouns). The planning process also involved seeking diverse participants and building connections with organizations around trans youth. There were 5 teens on the youth listening session call who each shared their experiences and concerns. The FDA staff hope to use this information to help in the development of guidance documentation and regulatory decisions.

The presentation concluded with some of the immediate lessons that FDA staff gleaned from this process. In the future, they hope to engage more youth participants by building contacts with schools and youth organizations and expanding their social media reach to include platforms that are more popular with young people. They also plan to be more strategic about the timing of listening sessions and the methods of communication to maximize the number of young people who can get involved. Finally, the FDA is looking at ways to collect parental consent electronically, since paper consent forms require access to printers and scanners.

Discussion

Discussion between PEC and PCWP members included the following:

What is the age of consent for clinical trials in Europe?

- It varies by country.

What has the FDA observed with young patients in terms of their ability to make rational decisions and their willingness to take risks?

- The FDA was specifically interested in including youth in the conversation around transgender health because of their unique perspective with hormone therapy around puberty. However, one thing the FDA observed during both listening sessions was that there were a lot of similar themes between the adult session and the teen session.
- The EMA commented that their experiences with young patients have demonstrated they are actually quite variable in terms of their willingness to take risks.

Was it difficult for the EMA to recruit young people to participate? What recruitment techniques did you find were most effective?

- It has been challenging for the EMA to recruit young people, particularly as specific health perspectives are needed depending on the medicines under evaluation. Often, the EMA reaches out to their network of patient organizations that help to connect them with young patients and youth groups.

Opportunities for Collaboration and Next Steps

Going forward, there is a lot of interest in continued collaboration and communication between the PCWP and the PEC. Members of both groups expressed interest in holding an annual joint meeting where both teams share ideas and experiences around defined topics.