SMG 9006

FDA STAFF MANUAL GUIDES, VOLUME IV – AGENCY PROGRAM DIRECTIVES

GENERAL OR MULTIDISCIPLINE

PATIENT AFFAIRS STAFF RARE DISEASE PATIENT LISTENING SESSIONS

Effective Date: 10/11/2019

1. PURPOSE.

The purpose of this document is to describe the procedures for the management of cross-Center Rare Disease Patient Listening Sessions (RDPLS), by the Patient Affairs Staff (PAS) in the Office of the Commissioner. Cross-Center refers to sessions that involve more than one Center, typically to include the Center for Biologics Evaluation and Research (CBER), Center for Drug Evaluation and Research (CDER), Center for Devices and Radiological Health (CDRH) and the Office of the Commissioner (OC). As needed the Center for Food Safety and Applied Nutrition (CFSAN), Center for Tobacco Products (CTP), and others may be included. This SMG outlines the standardized processes for cross-Center RDPLS, organized and executed by PAS, to enable efficient and effective planning for all internal and external stakeholders involved.

2. BACKGROUND.

FDA has made significant efforts to broaden its engagement with patients and ensure that patients and caregivers are actively engaged in medical product development, and their voice is considered as a part of FDA's regulatory work.

Several key programs across the medical product centers support patient-related mandates included in User Fees Acts (UFA) and the 21st Century Cures Act. The PAS' RDPLS builds on these efforts. The Food Drug and Safety Innovation Act (FDASIA) and the fifth Prescription Drug User Fee Agreement (PDUFA V) enacted in 2012, directed FDA to develop and implement strategies to solicit the views of patients to inform medical product development and regulatory decision activities. The 21st Century Cures Act enacted in 2016, the sixth Prescription Drug User Fee Agreement (PDUFA VI), and the Medical Device User Fee Amendments (MDUFA) of 2017 built upon previous efforts by advancing the collecting, analyzing,

interpreting, and integrating patient disease experiences into medical product development and regulatory decision-making processes.

The PAS RDPLS are one of several programs across the Agency that support these patient-related mandates. Through these sessions, FDA staff have the opportunity to ask questions directly to patients and caregivers about their rare disease and/or conditions that are not fully understood, and effective treatments are few or absent. A rare disease is defined as a condition affecting fewer than 200,000 patients in the U.S. RDPLS provide a unique opportunity for the FDA to gain insight to patients' perspectives on the burdens of a disease, current treatments if any, its impact on the patients' and caregivers' daily activities and quality of life, as well as ultimately what is important to patients when new products are to be developed. New medical products are increasingly targeted at rare diseases or diseases with smaller populations and more individualized therapeutics are increasingly the focus of modern medical product development. A key component of modernizing product development is shared learning and incorporating input directly from patients and/or caregivers to help address the challenges.

A RDPLS is a cross-Center, informal, non-binding meeting, (the meeting is herein referred to as a "Session"). The Sessions are not intended to be focused on a specific medical product. The Session is typically 60 or 90 minutes in length and are recommended to have no more than 7 patient perspectives per 1-hour meeting with generally no more than 15 patient/caregiver participating in the Session[1]. Sessions are not open to the public, however high-level summary documents are posted publicly online after it has occurred.

The goals of RDPLS include:

1. To educate FDA review staff about rare diseases or specific segments of non-rare diseases, e.g., to broaden review staff awareness of unmet patient needs, and to better contextualize patient experience;

2. To support regulatory decision-making, e.g., for a specific product or application, benefit/risk trade-offs, responding to shortages;

3. To help provide a starting point to guide or accelerate early stage research and development, e.g., helping to identify endpoints and new outcome assessment tools;

4. To help patients and their advocates understand the FDA's mission and work, e.g., generating awareness around FDA roles in medical product development and review.

[1] The FDA/NORD Rare Disease Listening Sessions are Paperwork Reduction Act (PRA) exempt under 21st Century Cures Act Section 3003.

PAS has developed, piloted, and refined standardized procedures for planning and executing a Session to ensure that they provide value while not adding excessive burden to FDA and patient stakeholders. Based on the outcomes of the pilot and the internal and external stakeholder feedback, the procedures were implemented and are outlined in this SMG.

3. RESPONSIBILITIES.

A. **Patient Affairs Staff (PAS)**: Located in the Office of the Commissioner, the primary FDA staff responsible for executing RDPLS that involve more than one Center.

B. **Lead Review Division**: The FDA division(s) with the primary interest in the RDPLS topic and are typically an expert in the therapeutic area.

C. **Lead Division Point of Contact**: An individual in the lead review division(s) who will serve as PAS' main point of contact for planning division-requested RDPLS.

D. **Interested Staff and Divisions**: FDA divisions other than the lead review division who have an interest in participating in the RDPLS in listening mode only. The patient engagement leads from each Center assist with identifying interested staff and divisions.

E. **Patient Engagement Lead**: A Center representative who will serve as PAS' point of contact for their Center or program.

F. **Patient Community**: The patients, caregivers, or patient advocates encompassing the patient community that will attend the RDPLS and provide insight into their experience and answer questions.

G. **Patient Advocacy Group**: The individual or groups of individuals who are patients, caregivers, or patient advocates who act as the representative for the patient community, when applicable.

4. PROCEDURES.

The procedures for planning and executing a RDPLS in brief:

A. **Evaluation**

1. FDA review staff or the patient community submit a request for a RDPLS to PAS. The request should include:

    a. Rare disease or condition name
    b. Desired meeting goals and objectives
    c. Listening session topics or discussion questions

d. Proposed draft agenda

2. PAS reviews the request and considers whether a RDPLS is the appropriate avenue for communication between review staff and patients, caregivers, or their advocates. Some of the criteria used by PAS to accept and prioritize a request include:

    a. Rare disease area, defined as a condition affecting fewer than 200,000 patients in the U.S.

    b. No prior interactions and no currently planned interactions with the FDA on the same set of agenda topics

    c. Anticipated broad impact from the Session (e.g., Session will be attended by two or more Centers)

    d. Session is requested by review division to aid in regulatory decision-making

    e. There is not another avenue that could better meet needs of the request

3. PAS engages relevant stakeholders, including the lead review division, patient engagement leads, interested staff and divisions, and the patient community to solicit additional context or feedback that can help clarify the appropriateness of the Session.

4. PAS, in consultation with the review divisions, decides if a Session should be scheduled. PAS informs the requester whether a Session is appropriate and potential next steps. If not appropriate or an alternative avenue preferred:

    a. PAS directs the requester to an alternative contact,
    b. suggests alternative timing, or
    c. provides feedback that would enhance appropriateness of request.

B. **<u>Preparation</u>**

1. Requester refines the agenda and participant criteria. PAS seeks input from others, if necessary.

2. PAS hosts preliminary call(s) with the requestor as appropriate. Preliminary calls may occur with relevant FDA staff and/or the patient community. Preliminary calls will:

    a. Help orient stakeholders to the logistics associated with organizing and running a Session

b. Develop the agenda and participant selection criteria

c. Create a facilitation plan for the Session, including what questions or topics will be addressed and their intent

Preliminary calls can occur at different stages of planning with attendance determined based on necessity and availability.

3. PAS schedules a date and time for the Session and sends invitations to all pertinent stakeholders.

   a. PAS provides materials to aide in the preparation for the Session, which could include expectations documents, logistics details, and a refined agenda or discussion questions.

   b. As necessary, PAS schedules additional preliminary calls to refine details of the Session.

4. PAS and the patient community or patient advocacy group identify potential patients/caregivers to participate.

   a. If able, the patient community or advocacy group should identify patients/caregivers to participate in the Session, often through a survey tool. Survey results should be deidentified prior to sending to PAS.

   b. If the patient community or advocacy group is unable to help identify participants, PAS will identify the participants.

5. The lead review division reviews the deidentified list of participants to better tailor their discussion questions. When there are more interested participants than slots available for the Session, the lead review division selects the deidentified participants based on the deidentified information they shared in the survey or other tool.

6. PAS or the patient advocacy group reaches out to selected and waitlisted patients and/or caregivers via phone to share information about the Session, to determine that the patient or caregiver has first-hand familiarity with the rare disease and the willingness of the patient or caregiver to participate in the Session and share their personal experiences. PAS or the patient advocacy group sets the expectations for what will occur in the Session and addresses any questions or concerns the patient or caregiver may have.

7. PAS organizes logistics around scheduling a teleconference line, conference room reservation/set up, and sends directions for people coming to campus. PAS distributes background documents and updated agendas as appropriate prior to the session. Sessions typically are scheduled for either 60 minutes or

90 minutes, depending on the number of participants and level of detail required to achieve the objectives of the Session.

C. **Listening Session**

1. All participants arrive in person or dial-in via phone. PAS escorts non-FDA in person attendees for the duration of their visit on FDA's campus.

2. PAS opens the conference call line ten minutes early and monitors the line as participants enter the Session to ensure all attendees have joined and can hear. At the start time, PAS does a roll-call of the anticipated patient/caregiver participants. PAS introduces the meeting facilitators and outlines if there are "silent observers" and why they are present.

3. PAS opens the Session, sets objectives, and clarifies expectations. PAS reads the financial disclosure statement and disclaimer and introduces the purpose of the meeting.

4. Following PAS' introduction to the meeting, the requester co-facilitates the meeting with PAS.

    a. In Sessions where FDA made the request and developed the agenda, PAS and the lead review division co-facilitate the discussion. PAS follows a script that was developed with the lead review division, asking questions of each participant in turn, allowing them to answer, then moving on to the next participant. Often, questions are customized to each participant and not every participant will be asked each question. The lead review division interjects with follow-up or probing questions as desired. As needed, PAS interjects if the conversation deviates from the lead review division's intended agenda and to ensure that all intended questions have been asked to all patient and/or caregiver attendees, as per plan. As an alternative, lead review divisions may ask to lead the question and answer session directly, with PAS only facilitating the opening and closing and interjecting, where needed.

    b. In Sessions where the patient community made the request and developed the agenda, they are responsible for leading the majority of the discussion. FDA is expected to listen and encouraged to ask questions. Often, the last 15 minutes of the Session are reserved for an open discussion between FDA and the patient community.

5. When there are 5 minutes left, PAS begins wrapping up the meeting. The FDA has the opportunity to ask final clarifying questions. PAS thanks the participants for their time, reviews next steps and action items, and concludes the Session.

D. **Codification and Communication**

1. PAS thanks the patient community participants for their time and emails a survey for them to offer feedback on their experience with the Session. PAS also emails a survey to FDA participants to obtain feedback and identify lessons learned following the Session.

2. Lead review division and interested staff and divisions follow up on any outstanding questions that were raised during the Session and provide responses to PAS.

3. A summary document is developed, and a link is posted on FDA's website.

    a. In Sessions where FDA requested the meeting, PAS develops a high-level summary document that highlights the key takeaways discussed for each question asked to participants. The summary document is subsequently shared with the lead review division for their input and approval of the content. When approved, PAS posts the summary on FDA's website.

    b. In Sessions where the patient advocacy group requested the meeting, the patient community is encouraged to develop a high-level summary document that highlights the key takeaways discussed during the Session. PAS requests to review the summary before it is made available to the public and provides a disclaimer statement that should be included in the summary. PAS will post the link to the advocacy group's summary on FDA's website.

5. EFFECTIVE DATE.

The effective date of this guide is October 11, 2019.

6. Document History - SMG 9006, "Patient Affairs Staff Rare Disease Patient Listening Sessions"

STATUS (I, R, C)	DATE APPROVED	LOCATION OF CHANGE HISTORY	CONTACT	APPROVING OFFICIAL
Initial	10/10/2019	N/a	OC/OCPP/PAS	Nina Hunter, Director, Office of Clinical Policy and Programs

Back to Agency Program Directives, Volume IV (4000-91000)