

Patient-Focused Drug Development Meetings

Smart Practices from Patient Community Leaders

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Direct any questions related to externally led PFDD meetings to the FDA's PFDD Program staff.

"Externally led patient-focused drug-development" is often abbreviated as PFDD or as EL-PFDD. In this guide, PFDD refers to "externally led patient-focused drug development" unless it is indicated that FDA led the meeting.

Overview

FasterCures, a center of the Milken Institute, designed this guide for patient advocacy organizations that are interested in hosting, or are currently planning, an externally led patient-focused drug development (PFDD) meeting. We have created other [tools for planning PFDD meetings](#), including the PFDD Meeting Tracker, the PFDD Readiness Assessment, and the PFDD Community Toolbox. We recommend using this guide in concert with those tools and related resources from the U.S. Food and Drug Administration (FDA).

FasterCures gathered the information in this guide from interviews with patient advocacy groups that have hosted PFDD meetings. FDA provided input on the accuracy of the content. This guide, however, represents the perspective of FasterCures alone and does not necessarily reflect the perspectives of the organizations involved or the FDA. This guide does not set standards and will continue to evolve along with patient-focused drug development.

Introduction

Hosting an externally led patient-focused drug development (PFDD) meeting is a weighty endeavor; it requires resourcefulness, fastidious planning, and tactful stakeholder engagement. Perhaps you are a part of a patient organization that is thinking about hosting a meeting or maybe you are planning one now. The litany of considerations can feel overwhelming, but you shouldn't have to begin anew. Organizations that have hosted PFDD meetings have marked a trail before you, some on lean budgets, a few with mostly volunteer staff, and nearly all without prior experience. How have they done it?

The team at FasterCures conducted interviews with veteran hosts of externally led PFDD meetings to gather their insights. During the interviews, we found that many leaders shared a similar experience: while planning, they received guidance from those who had planned meetings before them. Organizations were willing to share advice, but the process wasn't centralized or efficient.

In response, we have taken what we heard from those leaders and synthesized our findings into this guide to provide common practices, lessons learned, and considerations for patient organizations that might host a PFDD meeting or who have already begun the planning process.

This guide is not comprehensive. Planning a meeting is complicated, and you should carefully review FDA's resources. We recommend that you read our [background on PFDD meetings and FDA resources](#) or visit the [FasterCures PFDD webpage](#) for a list of FDA resources. The FDA should be your primary point of contact for any questions related to externally

led PFDD meetings, but you should also consult other organizations for guidance and advice. PFDD hosts rarely plan their meetings alone, and nothing can prepare you better than learning from others.

Since 2013, patients and caregivers have enthusiastically supported PFDD meetings, which have created an empowering opportunity for them to inform medical product development by talking about their experiences and current treatments. These meetings are part of an evolution toward patient-centric medical product development and health care—and this evolution isn't passive. It's driven by patients and caregivers who are ready to have their say. They need you, as a partner, to give them a space to say it.

PFDD began in 2013 as an initiative by FDA to incorporate the patient and caregiver voice into the regulation of new medical products. In late 2015, FDA expanded its PFDD initiative and called on patient organizations to host externally led PFDD meetings. To learn more about PFDD meetings, visit the [FasterCures PFDD resources webpage](#).

Section 1: Steps to Take Before Submitting a Letter of Intent

1.1 CONSIDER FDA'S REQUIREMENTS

Submitting a letter of intent (LOI) to FDA is the first milestone in the PFDD journey. An LOI is not a formal, bounded plan, but a way for you to notify FDA of your interest in hosting a PFDD meeting. FDA recommends submitting an LOI one year in advance of the proposed meeting date. During a FasterCures [webinar on PFDD](#), FDA shared that organizations should use the LOI process to notify the FDA about a meeting as early as possible so that they can receive feedback and input. “While it is important to address all of the key elements of a letter of intent for an externally led PFDD meeting, which are [outlined on FDA's website](#), FDA understands that some elements, such as the meeting date or exact venue, may not be finalized a year in advance. For example, we receive letters of intent that specify a broader timeframe such as ‘September 2019’ or ‘Fall 2019’ instead of an exact date—and that’s fine!”

According to [FDA's guidelines](#), an LOI should communicate “(1) the importance of the meeting in the context of the disease area, and (2) important details regarding the meeting plan.” To address these two points in your LOI, you will need to consider a few topics. Most, but not all, are detailed in this guide. You can also find examples of LOIs previously submitted to the FDA in the [FasterCures PFDD Community Toolbox](#).

1.2 OUTLINE GOALS

Before planning your PFDD meeting, you should be able to answer why a PFDD meeting would benefit your community at this time. Leaders we interviewed say it's critical to consider how a meeting

fits within the research, development, and regulatory context of the condition it represents. Are new therapies under development? Are there upcoming clinical trials or looming regulatory decisions? Has a PFDD meeting already happened? If you do not know, do some research and reflect on these questions. They will help you decide if a PFDD meeting is appropriate and what impact it could have.

Next, [review outputs from previous meetings](#) and talk with meeting hosts to understand what a meeting could do for your specific community. PFDD meeting outcomes can be leveraged best in the early stages of medical product development. Remember, though, that your meeting will be different from those that have come before it. As Jill Jarecki, chief scientific officer of Cure SMA, said, “Every community comes in at a different stage of drug development; they have to tailor their message and goals to where they stand.”

Once you have a definite purpose, you can identify goals. Create two lists: one for external goals mentioned in your LOI and another for internal goals. External goals are broad and framed toward regulators, medical product developers, researchers, and other stakeholders. For example, an external goal might be to provide product developers and FDA with patient perspectives on the impact of their condition and therapeutic priorities. Internal goals are for your reference and might include producing a comprehensive report, informing clinical trial endpoints, reaching 2,000 survey respondents, or demonstrating your organization's value to researchers and industry.

Almost every interviewee had the goal of recruiting a pool of participants that reflects the demographic and condition diversity of a patient community. PFDD meetings are an opportunity to present portraits of complex conditions that may affect different types of people. The FDA wants to hear from a representative room of voices. Representativeness is not just a regulatory imperative. Rosángel Cruz, director of research and clinical affairs at Cure SMA, put it simply: it's an ethical responsibility. She made it her personal goal to ensure that any patient unable to attend the meeting could watch the webcast and feel that her or his experience was represented.

Demographic and condition diversity and representation are also crucial for surveys, which are not a requirement of PFDD meetings, but some organizations use them to collect more perspectives. The [Lupus PFDD survey](#) is a good example. Three organizations—the Lupus and Allied Diseases Association, Inc., the Lupus Foundation of America, and the Lupus Research Alliance—built the lupus survey and reached a pool of 2,121 respondents. They now have a collection of data with the potential for use beyond the lupus PFDD meeting and report.

Many interviewees offered the same advice: be flexible. New realizations, therapeutic developments, or partners can require organizations to adjust or reevaluate their goals and objectives.

Demographic and condition diversity and representativeness are critical for PFDD meetings and pre-meeting surveys of your patient community.

1.3 EVALUATE YOUR RESOURCES

Hosting a PFDD meeting can require a sizable effort. Large organizations typically have more capacity for PFDD meetings than small organizations. For example, the complement 3 glomerulopathy (C3G) meeting hosted by the National Kidney Foundation (NKF) had support from internal NKF divisions for creative services, event planning, and website management. Resource and capacity limitations, however, should not preclude your organization from hosting a meeting. In one inspiring example, the Amyloidosis Research Consortium (ARC) hosted an externally led PFDD meeting with only one full-time employee and a volunteer. “We did it on a shoestring, and I think it’s possible to do that. I don’t think financial resources are key,” said Isabelle Lousada, the founder and CEO of ARC.

Regardless of capacity and resources, interviewees concurred that planning a PFDD meeting takes significant time and coordination, especially to recruit and prepare panelists and participants, chart logistics, conduct a survey (though it’s not a requirement), and write a meeting report. You may also need capacity for unanticipated tasks: making lists of participants, arranging travel, managing accommodations, and inviting stakeholders. Organizations often conjoin PFDD meetings with other events, such as annual community gatherings, to maximize the use of their resources and capacity.

Staffing needs can vary from one person’s full-time job (with assistance from other internal departments) to a part-time team with distributed responsibilities. According to Cruz of Cure SMA, the spinal muscular atrophy (SMA) meeting took 75 percent of her time for the first several months, but by the end, “it was a village effort.”

Interviewees stressed that it's critical to go slowly and take time to coordinate, organize, and think through what you want and need for each planning stage. There are "a lot of moving parts—more than you might think," emphasized Molly White, CEO of the Myotonic Dystrophy Foundation. For a successful meeting, plan ahead and try to anticipate the staffing and resources you will need as your efforts progress. Tools such as a timeline, budget outlay, and a communications plan—each discussed below—can help. Check out the PFDD Community Toolbox to find examples of thoughtful planning.

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1.4 DETERMINE IF YOU NEED SPONSORS

Many PFDD hosts needed sponsorship to fund their meetings. If you need sponsors, identify them before you submit your LOI.

Consider designating a sponsorship liaison to set expectations and maintain independence from the beginning. It is important to explicitly establish that, despite any contributions, sponsors will not give input on meeting processes or outcomes including the agenda, who participates, or the meeting report. "It's important that [sponsors] don't influence the process or the results," said Diane Gross, national director of advocacy and programs at the Lupus Research Alliance. "For example, we did not share the survey draft with them; we asked them to submit the key insights they would like to get out of [the meeting]. And some points were good, while others were not in the scope of the project. We kept them

updated through periodic emails and calls." Though sponsors shouldn't provide direct input, they may be able to provide other non-financial resources, such as advice from experience as sponsors of other PFDD meetings.

A budget estimate is valuable to have before approaching potential sponsors. A budget can clarify for sponsors how their funding will be used. Expenses can vary depending on meeting size, whether the meeting conjoins another event, and other factors, such as whether the host is providing food. It is also useful to keep a list of budget priorities, such as travel for participants or a writer for your meeting report.

1.5 SELECT A DATE

The meeting goals and the research and regulatory context should inform the selection of your meeting date. Review the therapeutic landscape; PFDD meeting outcomes are most effective in the early stages of medical product development. Check in with stakeholders, especially FDA, to identify meeting dates.

Also consider the burden on patients. Keep in mind that travel and lodging costs can vary with the seasons. For example, if you're hosting your meeting in Washington, D.C., take care to avoid federal holidays and major events, such as the National Cherry Blossom Festival. Consider whether you will conjoin your PFDD meeting with another event or host it independently. Interviewees we talked with were divided on how much this burdens patients. Some hosted conjoined meetings, which simplifies recruitment and makes the most of patients' time. For example, the Friedreich's Ataxia Research Alliance (FARA) started with a standalone meeting but added a small research symposium to maximize participants'

time and travel commitments. Other interviewees, however, felt that stretching participants' time adds burden, particularly for patients who have trouble remaining stationary for extended periods.

1.6 CREATE A TIMELINE

Creating a timeline for your meeting can simplify the planning process and keep your efforts on track. We found that there isn't a one-size-fits-all timeline; some organizations under pressure planned their meetings in two months. Others took nine or more. Ideally, interviewees recommended at least six months for dedicated planning. Keep in mind that the FDA recommends submitting an LOI one year in advance of your proposed meeting date.

Looking beyond the meeting, consider a plan for post-meeting follow-ups, such as writing the meeting report, publishing recordings, or hosting a post-meeting webinar to release the meeting report and highlight notable outcomes.

1.7 COLLABORATE WITH OTHER ORGANIZATIONS

It's common for several organizations to work in the same disease space, each with a distinct mission, capacity, and priority depending on its scope, size, and geography. The FDA encourages collaboration and wants to see a variety of organizations involved in PFDD meetings. Partnerships can strengthen representativeness, prevent duplicate meetings, and produce more broadly relevant outcomes. Before submitting an LOI, consider potential collaborators or at least solicit their feedback. Even if an organization cannot cohost the meeting, it may eagerly contribute in other ways, such as by reviewing content, recruiting

participants, or reviewing the meeting report.

It's also thoughtful to invite organizations that work in a related disease area to support or observe your efforts. They may want to host meetings in the future and apprenticing yours could be a valuable learning experience.

The lupus PFDD meeting is an example of a collaboration among three organizations: the Lupus and Allied Diseases Association, Inc., the Lupus Foundation of America, and the Lupus Research Alliance. These three organizations vary in size, the scope of their mission, and geography, and hosted a PFDD meeting with a comprehensive survey that reached over 2,100 respondents. They offered several suggestions for collaborating:

1. **Consider drafting a memorandum of understanding (MOU)** to divide labor, address competing interests, agree on the use of survey data, and establish a model for making decisions. MOUs prevent discord and provide a concrete reference for the mission and roles of a collaboration.
2. **Establish a steering committee** with diverse expertise and representation from each organization. A steering committee should meet regularly to plan the PFDD meeting and can create greater equity, with each member having equal input regardless of his or her organization's size and structure.
3. **Focus on both internal and external communication.** Draft an external communication plan to prevent overlapping outreach to stakeholders and to ensure you are reaching all of your intended audiences.

The FDA encourages community collaboration and wants to see a variety of organizations involved because partnerships strengthen representativeness, amplify outcomes, and prevent duplicate meetings.

1.8 SUMMARY

In summary, here are several steps to take before submitting a letter of intent:

- ✓ Review the [FDA's guidelines for submitting a letter of intent](#).
- ✓ Identify the goals of your meeting and how it will fit within the research, development, and regulatory context of the condition it represents.
- ✓ Estimate the internal capacity and resources you will need to host a meeting.
- ✓ Create a budget to understand the funding you will need and seek out sponsorship if needed.
- ✓ Choose a date for your meeting and construct a timeline around that date to keep your planning efforts on track.
- ✓ Reach out to other organizations and search for potential collaborators.

Section 2: Planning Your PFDD Meeting

2.1 DETERMINE WHETHER YOU SHOULD ADMINISTER SURVEYS

Deciding to administer a survey and what it should include will depend on your meeting purpose and your available time and resources. Ask yourself, why do I need a survey? To shape meeting content? To inform my meeting report? To engage my community? Two common reasons for administering surveys are to identify which topics a meeting should prioritize and to gather robust data from a larger and broader group of patients to include in a meeting report.

FDA does not require or necessarily recommend conducting a survey. Organizations may choose not to do surveys because their patient and caregiver populations are too small, because they are already administering other surveys, or because they do not have the time or capacity.

Surveys are not the only way to collect patient experience data. For example, one interviewee worked with an analytics firm to gather information from a condition-specific online group. Other interviewees had information from previous surveys they had conducted. Some, instead, opted for a post-meeting survey to follow up on topics that weren't addressed during the meeting.

If you do have the resources to craft and administer a survey, you should aim high—use rigorous methods that are likely to produce representative and accurate results. Constructing and distributing a robust survey can take four months or longer, so consider your capacity.

Engage with patients, caregivers, researchers, regulators, clinicians, and other care providers to help identify topics and questions. Experts in survey methods can help shape questions and format the structure. The [FDA Guidance Series for incorporating patient input](#) can serve as a starting point for understanding the aspects of rigorous survey methods.

If you are investing in a survey, pilot it with a small group of patients. Jen Farmer, executive director of the Friedreich's Ataxia Research Alliance, said pilot testing of her survey (and other meeting materials) was essential. Pilot testing can help you identify shortcomings, address topical gaps, and construct questions that are more coherent.

FDA does not require nor necessarily recommend surveys for organizations that want to host a PFDD meeting.

Consider your target population. What will make your survey more accessible? Consider factors such as language, literacy, and format (paper, web-based, or both). Be mindful of the limitations your patients may have.

2.2 OUTLINE CONTENT

Every PFDD meeting agenda follows a similar structure. Before you plan your meeting, you should study this structure by attending other PFDD meetings or by watching webcast recordings. The [FasterCures PFDD Tracker](#) is a useful resource for finding upcoming meetings and resources from past meetings, including agendas and recordings.

The agenda for most meetings is separated into two sections. Each section has a patient panel followed by a

facilitated audience discussion that often includes a polling component (which is not required). The first patient panel usually focuses on the symptoms and daily impact of the condition, including physical, financial, and psychosocial. The second patient panel usually focuses on current therapies or approaches to condition management and what patients would like to see from future therapies. Some organizations manage to fit this into a few hours—others, into a full day. FDA-led PFDD meetings are typically half days. As you choose a length for your meeting, first consider the patient burden.

While developing the agenda, work out details such as which discussion questions to ask and when or in what order the panelists will speak. If time permits, ask patients, clinicians, researchers, and other stakeholders for feedback on these agenda decisions. “[We] leaned heavily on disease experts,” said Lousada of the Amyloidosis Research Consortium. “That expertise was really critical in helping us shape what we wanted from this meeting and what would be helpful to the field and the U.S. Food and Drug Administration to know.”

In planning the patient panels, consider ways to bring patients’ stories to life. Several interviewees recommended incorporating pictures, videos, personal items, and medical equipment that illustrate patient testimonies or represent those who are unable to attend meetings. During the myotonic dystrophy meeting, White told us that the Myotonic Dystrophy Foundation decided to show a video with testimonies from patients who were unable to attend. Finally, if panelists’ testimonies don’t address an important topic for the condition or disease, be sure to address it in the facilitated discussion.

2.3 CONSIDER VENUE AND OTHER LOGISTICS

Most PFDD meetings take place at venues in the Washington, D.C., metropolitan area. This location has logistical advantages since it is close to the FDA's offices. On the FDA webpage for externally led PFDD meetings, FDA states it is "open to attending a well-designed and well-conducted meeting held in the DC Metro area (Virginia, Maryland, Washington, D.C.). For meetings conducted outside the DC Metro area, FDA will be open to attending remotely (e.g., webcast)."

That said, accommodating participants is the key consideration for selecting a venue. For example, Emily Milligan, executive director of the Barth Syndrome Foundation, decided to hold the Barth syndrome PFDD meeting in Clearwater Beach, FL, to capitalize on the large proportion of the patient, caregiver, and research community that would already be attending the Barth Syndrome Biennial International Conference and reduce the burden of cost and travel. Reaching out to PFDD meeting veterans for advice can help you find hotels best suited for hosting PFDD meetings.

PFDD meetings require the same materials as a typical conference, but it is easy to overlook some PFDD-specific logistics:

- Indicate which participants are patients and caregivers on the name badges.
- Have photo and video-release forms at the sign-in table, if needed.
- Provide printed agendas or programs with speakers' bios so that participants can follow along.
- Include tissues, water, and other items that participants may need at each table in the room.
- Use different colors for tablecloths or signs to indicate which tables are for

patients and caregivers. Have these tables near the front.

- Consider whether handheld, table, or clip-on microphones will work best for your panelists.

Accommodating patient participants is the key consideration for selecting a venue.

2.4 DETERMINE OUTREACH STRATEGIES

Ardent engagement from patients and caregivers drives PFDD meetings. Fostering this engagement starts with effective outreach well in advance of the meeting. Your community members may be unaware of PFDD meetings, and advanced outreach is an opportunity to orient them to the idea of patient engagement and what to expect from the meeting. One smart practice is to draft a comprehensive communication strategy that describes the goals, details, and importance of the meeting in plain language. It should also include a timeline of deliverables for different channels of communication (social media, email, website, brochures) and agreed-upon language and sample messages. Consider branding your PFDD meeting and creating a logo to give it distinct importance.

The central goal of communicating about your meeting should be to recruit patients and caregivers to attend in person or virtually. This can be a difficult task. Even if you sponsor the travel and lodging for your participants, their attendance will require commitments for time off from work, school, or other responsibilities. Communicating about your meeting as early as possible gives participants time to adjust their schedules and plan accordingly. If your meeting conjoins another event, emphasize that the PFDD component is distinct.

Early on, one of the first communication items you may require is a webpage or website where participants can register or can find out how to register. All future communications materials should point back to this web address. On the registration page, consider collecting demographic information, including how each registrant will participate (webcast or in person; as a caregiver, patient, or observer, etc.). Consider keeping your registration page open until the day of the meeting for participants who register late.

What your communication materials look like or what outlets you use to communicate should depend on your community. Where and how are members best reached? Social media and email blasts may be useful for recruiting those in your network. How will you reach those outside of your network? Collaborating with clinicians, researchers, the media, social media influencers, or other patient organizations can amplify your outreach. If your organization has connections to support groups, hosts regular events or webinars, or regularly attends other meetings as a partner, use those opportunities to advertise your PFDD meeting. Regardless of the communication methods you use, be consistent in your messaging and communicate regularly. One interviewee engaged her community through a variety of outlets daily, for three months.

It's important to remember that the FDA does not sponsor or endorse externally led PFDD meetings. Even if you've received feedback or input from the FDA, you should never use the FDA's logo or label your meeting as FDA-endorsed.

Don't forget to include post-meeting communications in your communication strategy to inform those who were unable to attend and to clarify how your

organization will use the meeting information going forward.

The central goal of communicating about your meeting should be to recruit patients and caregivers to participate in person or virtually.

2.5 RECRUIT PATIENT AND CAREGIVER PARTICIPANTS AND PANELISTS

Start recruiting panelists and participants for your meeting as soon as possible. Some organizations begin this process immediately following the submission of their LOI. While regulators, researchers, and industry representatives are important audience members, convening patients and caregivers is essential.

Your primary focus when recruiting should be on convening participants with demographics and experiences that are representative of the disease or condition; your attendees should reflect the population you identified during your goal setting. Time and resource limitations might determine how comprehensive your recruitment process can be, but diverse representation is essential for a successful PFDD meeting and encouraged by the FDA. To convene diverse representation, you may need to find creative ways to connect with traditionally marginalized communities and forge new partnerships. International patients and caregivers may not realize that their participation is equally valued. For rare diseases, it's important to encourage international participants to attend in person or via webcast regardless of their country of origin.

Beyond outreach to your community, your primary method of recruitment should be to reach out to other organizations, particularly patient advocacy groups. Working with clinicians and professional societies is also useful for reaching patients with rare diseases. To respect privacy, have clinicians ask patients first if you can contact them about your meeting. A benefit of this method is that clinicians may know patients who are comfortable with public speaking or who have particularly relevant stories. Be prepared to deal with last-minute registrations and the accompanying travel and lodging logistics. Also, have contingency plans for panelists that drop out, including having alternate panelists prepared to speak if needed.

2.6 PREPARE PATIENT PANELISTS

Assembling a diverse, representative, and well-spoken patient panel is important for a successful PFDD meeting. It is also typically a labor-intensive task. You may want to prepare panelists before the meeting to ensure that they can meet the anticipated time commitment. Organizations reported that preparing panelists is a worthwhile investment, though it can take hard work and a bit of time (four months, for one organization). Farmer, of Friedreich's Ataxia Research Alliance, could not emphasize enough: "Preparation, preparation, preparation. The number of practice sessions we held [with panelists] was critical."

The purpose of preparation is to help panelists be clear and comfortable telling their stories on stage. You should not influence or alter panelist stories, and, critically, sponsors or sponsor intermediaries should never be involved.

For PFDD meetings led by FDA, the agency prepares panelists with the lowest potential influence. It selects panelists two weeks before a meeting from those who submitted public comments for meetings with a focus on convening diverse viewpoints. FDA advises panelists to write out their statements and then holds one group call before a meeting to prepare them. At most, during those calls, FDA provides light feedback to help panelists touch on relevant topics.

Many patient organizations take a more involved approach. They regularly communicate with panelists to help them refine and practice their stories. Like the FDA, a common first step is to have patients write their stories as a script. According to White of the Myotonic Dystrophy Foundation, having patients write their scripts is useful. "It is very succinct, makes for very tight, compelling, articulate delivery." To her, having panelists write their stories into a script was a smart practice since it keeps the meeting moving and prevents it from devolving into details that may be less central to its focus.

Next steps can include conference calls or one-on-one calls for patients to practice delivering their statements. For Gross of the Lupus Research Alliance, "The prep phone calls were great because everyone got to hear what each person was going to say, and they helped each other; they provided critiques to each other." Preparing panelists as a group bolsters confidence and creates bonds so that panelists have intergroup rapport when they speak onstage together.

A diverse, representative, and well-spoken patient panel is an important part of a successful PFDD meeting.

When preparing panelists, be careful not to lead their stories. *As an organizer, your influence over what patients say should be minimal.* Some organizations, though, choose the order in which their panelists speak. A crafted lineup can create a narrative that highlights and balances themes. As Jarecki of Cure SMA said, “We had specific goals and information of what we thought would come out of this.” Creating a meeting that conveys the breadth and depth of a condition takes a vision and upfront planning.

Panelists may be nervous before a meeting. There are ways to assuage their nerves and help them effectively present. Prior to a meeting, you can provide informational packets with logistical details, the goals of the meeting, and the role of the panelists. Encourage panelists to bring the scripts that they wrote on stage. Consider hosting a preparatory dinner, social event, or rehearsal the night before. Finally, assure panelists and participants that their input is invaluable. Kathleen Arntsen, president and CEO of the Lupus and Allied Diseases Association (one of the three organizations to lead the Lupus PFDD Initiative), told us that she gave opening remarks as the community representative. To help set the tone of the lupus meeting, she asked people with lupus to stand, thanked them, and urged them to make their voices heard by stating, “Be brave, take a deep breath, dig deep, and go for it—this is your day.”

2.7 FACILITATE AUDIENCE DISCUSSION AND POLLING QUESTIONS

Following each panel is a facilitated audience discussion. A facilitated discussion typically covers several topic areas. Though optional, it is common to weave polling questions into the discussion. These polling questions are not a survey but serve to stimulate

discussion and help the moderator evoke deeper perspectives. They also help engage reticent or remote (via webcast) audience members. Typically, after the audience answers a polling question, the moderator will ask follow-up discussion questions to clarify or explore patients’ viewpoints. Many organizations, though not all, choose to contract with an external moderator for the discussion.

Time constraints can limit the number of topics addressed for each section of the meeting, so carefully craft and review each discussion and polling question. If you have the capacity, consider constructing your questions based on data gathered through a survey or other source of patient input. Some organizations used social media listening to identify topics important to patients. Similar to doing a survey, it may be tempting to ask more questions or more specific questions than time allows. Typically, there are no more than three to four polling questions per topic area. Craft questions broad enough to accommodate most participants and condition states, and take care not to create leading or suggestive questions.

Even the most thoughtfully crafted polling or discussion questions may miss the mark, so ask patients, researchers, care providers, or other organizations to evaluate your questions before your meeting. Interviewees who took the time to have their questions evaluated said it was essential to the success of their meeting.

Polling is used to foster discussion. It can be done through a show of hands or with technology. With technology, it is easier to have more response options per question—though you should limit the number of response options to eight or fewer. Interviewees reported that there

are several polling technology products from which to choose. Some are more flexible than others are. For example, if you would like to keep the poll open after your meeting is over for those who later watch the recording, then make sure your software can accommodate. Keep your participants in mind—will they have limitations that might make it challenging to use clickers, smartphones, or computers? More than a few interviewees had polling technology issues. You can't always avoid such issues, but having an IT team on hand can help.

You can encourage audience input in several ways. Some meeting hosts sent discussion questions ahead of time so that participants could prepare their thoughts. It's not recommended, however, to share polling questions ahead of time, since they are meant to foster discussion. Polling results can contextualize survey results, panelist testimonies, and the facilitated audience discussion.

Nearly half of our interviewees wished they had invited more clinicians and clinical researchers to attend their PFDD meetings.

2.8 INVITE REGULATORS, RESEARCHERS, AND INDUSTRY

While your primary objective should be convening patients and caregivers, it is also essential to garner representation from the FDA, the research community, and industry to listen during PFDD meetings. Anyone who might be involved in product development and clinical trials relevant to your condition should hear what patients and caregivers have to say.

As several interviewees emphasized, proactively engage with the FDA early. Early engagement is important since

input and feedback from the FDA can take time. Beyond your LOI, you should directly contact [FDA's Patient-Focused Drug Development Program staff](#). They can assist in answering questions and coordinating communications internally among FDA staff. Some organizations reported that meeting with FDA staff regularly to align objectives, choose dates, and receive guidance was critical to their efforts.

Also, remember to set aside time to reach out to industry and clinical researchers. Nearly half of our interviewees wished they had invited more clinicians and clinical researchers to attend their PFDD meetings.

2.9 SUMMARY

After submitting a letter of intent, puzzling together the details of a meeting can be challenging. The following are a few important considerations:

- ✓ Decide whether you will have a survey, depending on your capacity and if there is an organizational need.
- ✓ Study the structure of previous PFDD meetings before you plan your own.
- ✓ Consider the patient burden when drafting an agenda and choosing a location.
- ✓ Gather background information before organizing meeting content and creating questions. Ask patients and other stakeholders for input and revision.
- ✓ Draft a communication strategy to stay consistent and focused.

- ✓ When communicating about your meeting, prioritize the recruitment of patients and caregivers.
- ✓ Keep your online registration open as long as possible.
- ✓ Work with patients to refine their stories. Before a meeting, take steps to help assuage patients' nerves. If possible, have backup panelists in case some cannot attend.
- ✓ Proactively engage with [FDA PFDD Program staff](#).
- ✓ Patients and caregivers should be your priority, but don't forget to invite other important stakeholders.

patient community and created an online patient platform.

Hosting a meeting is an opportunity to build more formalized relationships with stakeholders in your disease space. For example, PFDD meetings can serve as incubators for finding new collaborators in patient-reported outcomes research and for strengthening connections with decision-makers. It's also an opportunity to demonstrate the value of your organization. "We want to be in the driver's seat," said Annie Kennedy, senior vice president of legislation and policy for Parent Project Muscular Dystrophy. "Patient organizations have worked a long time to have that, [instead of] shifting responsibilities to the U.S. Food and Drug Administration. The onus is still on us to drive implementation once we've created this new engagement with partners. As a patient community, we want to be, get to be, and have the responsibility to be driving that implementation."

Section 3: Preparing for Post-Meeting Outcomes

3.1 SEEK OPPORTUNITIES BEYOND YOUR MEETING

A PFDD meeting shouldn't be your end goal but part of ongoing engagement between your community and medical product decision-makers. Lousada, of the Amyloidosis Research Consortium, noted that a PFDD meeting captures a single point in time. An isolated meeting can have only limited success; you will need to maintain the momentum. Some interviewees used PFDD meetings as a learning opportunity—a moment to reflect on the direction of their organizations. For example, if your organization is involved in research, then a PFDD meeting may help you identify gaps not only in the research that you sponsor but also in the tools and metrics used in that research. Other organizations used the outcomes to refine policy agendas or identify parameters for future meetings. One organization recognized a need for a

An isolated meeting can have only limited success; you will need to maintain the momentum.

Meeting reports, usually called Voice of the Patient reports, also offer an opportunity for engagement with the FDA, industry, payers, and other influential decision-makers. Most organizations submit their reports to the FDA, and some use the reports for purposes beyond regulation. One interviewee planned to share the report with payers to demonstrate the value of particular therapies. Another intended to leverage the report to support new clinical endpoints for trials.

3.2 EVALUATE OUTCOMES AND OUTPUTS

Most interviewees went into their meetings with specific outcomes that they anticipated; many came out of their meeting with positive outcomes that they hadn't.

Once submitted, meeting reports can be used to inform decisions made by FDA. You can find out how to submit a report by reading this [FDA FAQ](#). Reports can color in the therapeutic context for FDA staff, helping them to conduct benefit-risk assessments and advise medical product developers. Although outcomes can be difficult to track, PFDD meetings undoubtedly influence attendees, including those who attend from FDA. "Anybody that was in the room will never forget what an SMA patient is or looks like," stated Cruz of Cure SMA.

PFDD meetings can also affect product development more directly. One interviewee reported that, as a result of a meeting, she heard from a number of companies that the meeting impacted both their early-stage research and development as well as the endpoints used in clinical trials. And a PFDD meeting might spur the development of new tools and metrics for measuring those endpoints. Another interviewee witnessed a company, in response to a PFDD meeting, reorient its research pipeline.

Beyond regulation, research, and development, PFDD meetings can have an impact on patients and caregivers. Many interviewees didn't anticipate how patients and caregivers would be affected. It is often the first time patients and caregivers meet other individuals with the same conditions or circumstances. After going through what, for many, is an emotional experience, patients and caregivers often build tight bonds. David Feldman, medical project director of the

National Kidney Foundation, "heard so many times, 'I've never talked to another patient with this disease.' It was very gratifying for us to have facilitated such an opportunity for patients. The purpose was [to inform] the FDA, but the patients got a lot out of this meeting." Feldman heard from one person with C3G, "I've felt alone and ashamed because this isn't a 'huge deal' compared to other diseases according to those around me. 'It could always be worse' is a very common phrase for me to hear. Being able to tell my story, be heard, and people empathizing and understanding the struggles this disease faces you with is indescribable." Seeing this impact, Feldman recognized the need for an online C3G patient community and has since created one. Another organization suggested convening focus groups or an informal reception after a meeting to help attendees process their experiences and discuss the challenges they face.

For rare conditions, PFDD meetings can also be an opportunity for patients to meet the limited set of experts and clinicians for their condition.

Finally, meetings often leave all attendees with an appreciation for engagement. "[Patients and caregivers] feel like most people aren't listening to them," said Jarecki from Cure SMA, and they want their voices to be heard.

3.3 SUMMARY

Your PFDD meeting should not be your end goal. Don't forget to plan for post-meeting opportunities:

- ✓ Think of ways to maintain the momentum of your meeting through outreach and future events.
- ✓ Consider your meeting as an opportunity to strengthen relationships with other stakeholders.
- ✓ [Submit your meeting report](#) to the FDA, and consider how to use it in other ways beyond regulatory decisions.
- ✓ Consider how your meeting might affect patients and caregivers and how you can partner with them on new opportunities.

Conclusion

The arrangement of panelists, the cadence of questions, the timing of sessions—it's easy to get flustered by the swarm of details. Each can seem trivial, but together they can have an invaluable effect on medical product development for your community. And that effect will echo beyond the walls of your meeting; it will move us closer to a system that values the voice of the patient. Although planning a PFDD meeting isn't easy, your contribution will make a difference not only for your community but also for all of us.

About Us

ABOUT THE MILKEN INSTITUTE

The Milken Institute is a nonprofit, nonpartisan think tank. We catalyze actionable solutions to persistent global challenges by connecting human, financial, and community resources to those who need them. Guided by a conviction that the best ideas, under-resourced, go nowhere, we conduct applied research, convene luminaries from competing viewpoints, and construct programs and initiatives designed to achieve our overarching mission of building meaningful lives.

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Julien Rashid is an associate at FasterCures, a center of the Milken Institute, supporting the organization's programs. Before he joined the team in the fall of 2017 as an intern, Rashid worked in a mix of health-related spaces while at the University of Wisconsin-Madison. After counting pills in a pharmacy, sequencing DNA in a lab, analyzing the health and economic outcomes of rural communities, facilitating global health educational courses, and listening to people in the most underserved parts of the country and the world, he realized his interests lie in health systems, their inefficiencies, and their potential. Rashid holds a B.S. with a double major in molecular biology and community and environmental sociology. He also has a certificate in global health.

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