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Commentary

Using Patient Advisory Boards to Solicit Input Into Clinical Trial Design and Execution

Annick Anderson, MBA; Jasmine Benger; and Ken Getz, MBA

Center for Information and Study on Clinical Research Participation, Tufts University School of Medicine, Boston, MA, USA

ABSTRACT

Academic institutions, pharmaceutical and biotechnology companies, foundations, and associations are routinely implementing patient advisory boards (PABs) to solicit patients' voices and perspectives on a variety of clinical research−related areas, including protocol design, clinical trial execution, informed-consent form design, clinical trial medicine kit design, wearable devices and mobile technologies, and patient-communication materials. Based on experience conducting >50 PABs during the past several years, the authors provide insights into how to best plan and execute PABs and their value in informing improvement in patient engagement. (*Clin Ther*. 2019;41:1408−1413) © 2019 Elsevier Inc. All rights reserved.

Key words: patient advisory boards, patient centricity patient engagement, voice of the caregiver, voice of the patient.

INTRODUCTION

Recent research has shown that the use of patient advisory boards (PABs) to inform clinical trial decisions is popular and widespread, in large part due to their relatively low cost and high perceived value, as well as their ability to be used as a tool for better understanding and minimizing the burden that clinical trials place on study participants.[1] Three in four major pharmaceutical companies report that they have piloted and implemented at least 1 PAB, making it one of the most common patient-engagement initiatives implemented in recent years. A growing number of academic institutions also report soliciting input from patients to inform clinical research planning and ensure convenience in participation.[2]

Organizations are implementing PABs to amplify and gather patient feedback and input on a variety of clinical trial elements, including protocol designs, end-point measures, clinical trial medicine kits, technology solutions, and study communication materials.[3]

Public-sector agencies and institutions have been using PABs to ensure that diverse perspectives are heard. Several senior clinical research executives from major pharmaceutical companies, including EMD Serono, UCB, Biogen, and Janssen, have recommended the use of PABs as a standard practice.[4] Results from a recent CenterWatch study noted that clinical trial study staff, principal investigators, and study coordinators consider patients' input into protocol design and execution a crucial success factor.[5]

Regulatory agencies in Europe and in North America have been soliciting input from patient communities. The European Medicines Agency routinely invites patients and representatives from patient-advocacy groups to share their experiences and perspectives as participants on scientific advisory panels.[6]

The US Food and Drug Administration has been holding meetings among patients and advocacy groups with select rare diseases as part of its Patient-Focused Drug Development initiative and its Patient-Engagement Advisory Committee. Established under the fifth authorization of the Prescription Drug User

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Fee Act, the purposes of the Patient-Focused Drug Development initiative are to hear directly from patients with rare medical conditions to better understand how these illnesses are being managed and to identify meaningful and beneficial outcomes that should be targeted by investigational drugs and biologics.[7]

For the past 7 years, The Center for Information and Study on Clinical Research Participation (CISCRP) has been organizing, hosting, and facilitating PABs on behalf of clinical teams and research sponsors in both public- and private-sector organizations (eg, pharmaceutical, biotechnology, medical device companies, institutions, associations and foundations). In total, CISCRP has conducted ~50 global PABs on a wide variety of therapeutic areas, disease conditions, and other areas of focus, including:

Protocol/study design
Study synopses
Schedules of assessments
Clinical trial execution simulations
Informed-consent form design
Clinical trial medicine kits/packaging
Clinical trial technologies and procedures
Patient recruitment and study or research program communication materials

Despite their growing use, PABs are still relatively new and unfamiliar to many organizations and professionals. The purpose of this article is to share lessons learned and insights from our experience, to inform organizations looking to adopt and implement PABs. It is our hope that public- and private-sector organizations will recognize the substantial value proposition of PABs and that they will consider implementing them as a standard practice to optimize patient engagement in their clinical research activity.[8]

SIZE AND SCOPE

Unlike public meetings, in which a large number of patients and patient-advocacy representatives are present, we have found that the ideal size of a PAB should be no more than 10 participants. Larger panels tend to be unwieldy and make it difficult for some of the panel members to be heard. The ideal panel has patients who are members of an advocacy group, as well as individuals who are nonmembers, to balance and diversify perspectives. In general, most PABs engage individuals who are familiar with what it is like to live with the particular disease of focus, to ensure that the feedback is both relevant and will help to alleviate burdens that are most pertinent to the study population. In some cases, and depending on the specific objectives of the PAB, engaging a mix of clinical trial—experienced panel members and clinical trial—naïve members helps to bring different views to the discussion. Those without prior experience in clinical trials can provide valuable insight into the concerns that the clinical trial—naïve population may have with respect to clinical research, and these perspectives can help researchers to design clinical trials that are more accessible and approachable to these individuals. Conversely, those who have previously participated in a clinical trial can provide feedback based on their experience and established knowledge of clinical trials. Importantly, panel members are not participants in the study that the PAB is targeting. And PABs often include caregivers and family members to solicit their valuable perspectives as partners in the clinical trial

Table. Patient advisory board meeting preferred structure and format.

Parameter	Description
Format	Structured and facilitated in-person meeting
Timing/ location	Half-day meeting in easily accessible metropolitan location
Frequency	Ongoing series of meetings to maintain engagement
Composition	Eight to ten panel members; representative of patient advocacy group (if applicable); member of sponsoring organization; independent facilitator
Member profile	Patients/caregivers/family members; mix of members and nonmembers of patient advocacy groups; mix of clinical trial-experienced and clinical trial naïve; diverse demographic profiles; generally representative of clinical trial population (if applicable)

process. The Table summarizes key elements in the structure and format of PABs.

Each PAB typically has 1 facilitator. Ideally, this individual should have not only experience in moderating panel discussions but also familiarity with the clinical research enterprise and the clinical trial process. We have found it valuable to include a representative from the advocacy group in the discussion. This individual typically helps participants to feel at ease, lends credibility and trust to the panel discussion, and understands collective patient experiences in managing a given disease condition.

Representatives from pharmaceutical and biotechnology companies typically listen, without influencing the PAB discussion, from a separate observation area. We recommend that a clinical scientist or a clinical team member intimately familiar with the protocol or tested concept participate on the panel. Past experience has shown that these individuals bring scientific authority and gravitas to the advisory panel discussion and convey a deeper commitment to the PAB.

We also recommend that PABs are designed as a part of a series of conversations. For example, an initial PAB may be conducted at a point when the clinical team or organization is seeking early input. A second may be held at a later date to discuss implementation experience (meaning execution of clinical trial). So first meeting is about seeking input into clinical trial design (before launching trial), second meeting could be about ways to improve execution of clinical trial and to solicit input into continuous improvement opportunities.

DURATION AND FLOW

Ideally, panel discussions should be no longer than 3 or 4 h in duration—much longer and participants begin to tire and to lose focus. Panel discussions should be structured as lively conversations with an opportunity to listen to diverse and candid opinions. Our experience in conducting PABs has shown that panels designed and implemented as focus groups or market-research studies tend to come across as disingenuous and fail to engage participants as valued advisors and discussants.

We begin every PAB with a short general discussion about the clinical research process and the importance of clinical research in the advancement of medical knowledge and public health, to help panel members better understand the value of their input and participation. It is crucial to include ample time for each panel member to share his or her personal journey as a means to build rapport. With consent from panel members, discussions are audio-recorded and reviewed afterward, when key themes can be captured and shared with clinical teams.

In addition to organizing and facilitating live events, over the years we've been asked to organize and moderate patient panels over the Internet using social media and video. Virtual formats offer the benefit of reaching diverse patients and perspectives from all over the world, but, in our experience, they typically generate only broad perspectives rather than an in-depth understanding. We have found that virtual PABs fail to establish a natural dialogue, are far more difficult to probe, and represent a particularly challenging environment in which to explain complex clinical research concepts.

We believe that it is extremely important to hold PABs in venues that are conducive to relaxed and natural discussions and in locations that are comfortable and convenient for patients and their family members and caregivers. Depending on the population of interest to be included in the PAB, panel members who would provide relevant and valuable insight into the disease of interest might not be local (particularly if the disease is rare), and in order to accommodate individuals who would need to fly in, holding a PAB in a metropolitan area near an airport is suggested. Many other factors contribute to convenience, including ample parking and easy access to the location and the discussion room.

PLANNING AND EXECUTION

In the United States, a PAB typically requires 3–4 weeks to organize. Figure 1 provides a breakdown of the timeline. A 3- to 4-week turnaround time allows us to obtain review and approval from the ethics committee; to develop a discussion guide and materials to share with the panel; to establish and coordinate relationships with patient-advocacy groups; to identify, select, and secure a venue for the panel; and to recruit and engage panel participants.

The 3- to 4-week planning period also accounts for the time typically required for identifying advisory board members on an ongoing basis until about a week before the PAB. We identify potential members

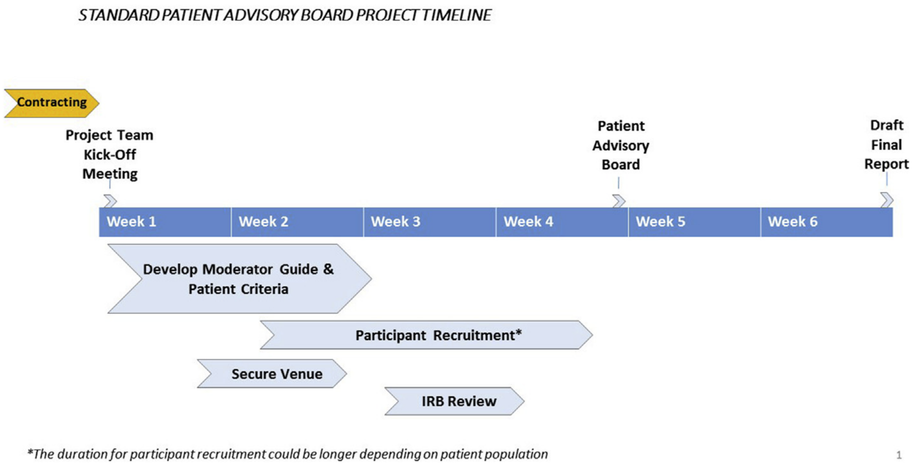


Figure 1. Standard patient advisory board timeline. *The duration of participant recruitment could be longer, depending on patient population. CISCRP = Center for Information and Study on Clinical Research Participation; IRB = institutional review board.

through ongoing interactions with patients who have expressed an interest in participating on future PABs and through routine outreach efforts to notify patients and the public about upcoming educational programs and events. We often work closely with advocacy groups for help with recruiting advisory board members.

Depending on the objectives of the clinical team, prework or prereads may be sent to the advisory board members in advance of the meeting; however, it is important to consider the advisory board members' level of familiarity with the clinical research enterprise, so as not to overwhelm or confuse them with medical jargon.

The cost of running each PAB varies widely depending on the geographic area, language translations, and the effort required to identify and engage participants. PAB sponsors typically cover board member participation; travel expenses; venue rental fees; event planning; and organization, facilitation, and reporting of the results.

At the end of each PAB, we encourage members of the clinical team or sponsoring organization to enter the meeting room, introduce themselves, and express their gratitude. This act affirms the sense that PAB members are valued partners and advisors. It also conveys a commitment to transparency and openness, and, importantly, puts a face on the sponsoring organization.

Subsequent to the PAB, we suggest that organizations keep the panel members apprised of next steps and updates, through either a follow-up meeting or written communication. Because information on advisory board members is held confidential and is not shared with the sponsor, CISCRP typically moderates such discourse. While it would be valuable to the advisory board members to be notified of the changes they helped to make to the protocol, we understand that not all changes can be implemented. As such, the use of written communication to express thanks and to reiterate the learnings back to the members, in order to communicate that their ideas were heard, can be a valuable way to express appreciation.

SPECIAL CONSIDERATIONS WHEN EXECUTING A PAB ABROAD

Conducting a PAB in countries outside of the United States, particularly in countries where English is not the native language, requires special considerations. The concept of clinical research is often unfamiliar to PAB participants. Dedicating sufficient time to clearly explain and set expectations well in advance of the meeting is imperative. Building extra time into the project timeline for the ethics review process is also important, as the process differs from country to country, and there may be variances in local laws about which researchers should be aware so that they are in compliance with these requirements. Additional time for translating materials into the local language may also be needed.

To overcome some of these linguistic and logistical challenges, PAB discussions should be held in a country's native language to create a comfortable atmosphere for participants to freely and easily participate in the discussion. A qualified simultaneous translator is a valuable asset in helping project team members to follow the discussion.

RESULTS AND IMPACT

Every PAB that we have conducted has been an emotionally moving experience for clinical research teams, sponsoring organizations, and the advisory board members who are invited to participate.

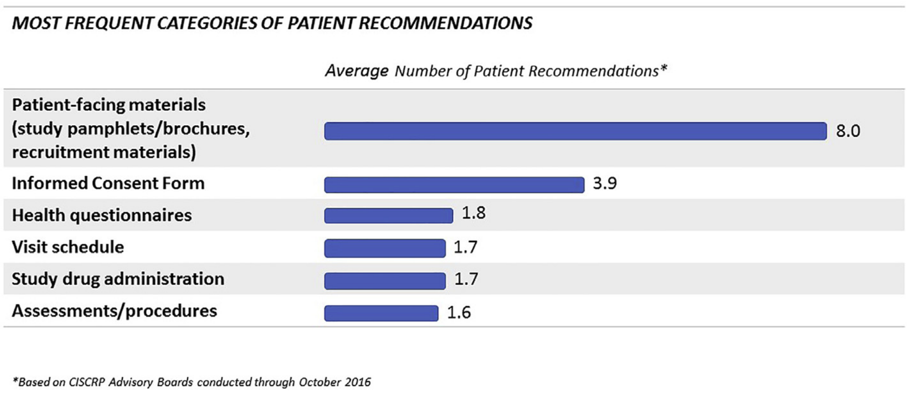
For many organizations, a PAB represents the first time that they have heard the patient's voice; learned about patients' experiences in managing the specific illness; and received candid feedback about clinical development plans, materials, and activity.

For some advisory board members, a PAB may be the first time they have met other people living with the disease, particularly if it is rare. In these scenarios, the PAB offers these patients the opportunity to learn about other patients' personal journeys with the same disease, to feel less alone, and to make meaningful connections.

Every PAB has generated valuable insights and resulted in concrete improvements, such as adjustments in assessments and visit schedules, enhancements to the content and presentation of patient-facing materials (eg, study pamphlets, patient recruitment posters), and modifications to the user interface for specific clinical trial technologies.

Figure 2 provides a summary of the average number of patient recommendations per panel, by discussion topic area. The typical PAB focusing on protocol design, for example, generates 2 recommendations on ways to improve the visit schedule, and 2 recommendations on changes to or a reduction in the number of procedures performed. PABs reviewing informed-consent forms, which include evaluations of content, layout/design, and delivery, such as electronic consent, usually generate an average of 4 recommendations.

One of the most tangible areas of impact is the opportunity for clinical research professionals to receive feedback directly from patients—to hear opinions, reactions, and recommendations that are in each patient's own voice and words. For many clinical research professionals, this is a first-time experience that can be both moving and enlightening. It is not uncommon for clinical teams to be inspired and energized by PAB discussions. In many cases, issues that were in protracted debate among clinical



*Based on CISCRP Advisory Boards conducted through October 2016

Figure 2. Most frequent categories of patient recommendations. *Based on Center for Information and Study on Clinical Research Participation Advisory Boards conducted through October 2016.

teams and between functions supporting a given clinical trial are quickly resolved when a patient has had the opportunity to weigh in, and the team gains clarity on areas that matter most to patients.

KEY RECOMMENDATIONS

To summarize, we recommend the following key practices to ensure a successful and valuable PAB:

- Treat each PAB as a listening exercise and as a natural and genuine discussion, not as market-research project;
- Create a comfortable atmosphere conducive to open conversation;
- Use a small group size and in-person meetings;
- Incorporate an educational component on clinical research during each PAB to provide context prior to soliciting feedback on a specific clinical study or study-related materials (most patients, caregivers, and family members—including past study volunteers—are not fully informed on the basics of clinical research, and the educational component offers an opportunity for panel members to feel more confident with the discussion topics);
- Design a successful PAB discussion with high sensitivity to the specific health, demographic, and cultural needs of the patients (ideally, PABs conducted abroad should be in native languages);
- At the beginning and end of each PAB, communicate and show appreciation for panel members' participation by thanking each participant for his or her time and willingness to share his or her experiences;
- Employ a collaborative approach with clinical teams and with contacts from the sponsoring organization, using regularly scheduled meetings leading up to the PAB with key team members, to help to ensure that (1) all parties are involved and engaged throughout the process and (2) the PAB is valued and taken seriously; and
- Hold a debriefing session among the clinical team or sponsoring organization immediately following the meeting to capture the most relevant, top-of-mind insights and to discuss next steps.

Growth in the popularity and use of PABs by both public- and private-sector organizations throughout the clinical research enterprise highlights their value. We anticipate that over time PABs will become a standard and integrated practice to ensure higher levels of patient engagement.

CONFLICTS OF INTEREST

The authors have indicated that they have no conflicts of interest with regard to the content of this article.

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Address correspondence to: Annick Anderson, MBA, Research Services, Center for information and Study on Clinical Research Participation, One Liberty Square, Suite 1100, Boston, MA 02110, USA. E-mail: annickanderson@ciscrp.org