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Insights and Best Practices for Planning and Implementing Patient Advisory Boards

Annick Anderson, MBA[1], and Kenneth A. Getz, MBA[1,2]

Abstract

A growing number of organizations—including pharmaceutical and biotechnology companies, foundations and associations—are routinely implementing patient advisory boards (PAB) given their high reported value for minimal relative investment. Organizations are typically implementing PABs to solicit patient voices and perspectives on a variety of areas such as protocol designs, clinical trial medicine kit designs, informed consent form designs, technology solutions, and patient communication materials. The Center for Information and Study on Clinical Research Participation (CISCRP) has planned, executed, and facilitated more than 30 PABs. In this article, the authors share lessons learned and best practices with regard to structure, format, and process for organizations wishing to adopt and implement PABs. The authors also provide metrics on the adoption and impact of PABs.

Keywords

patient advisory boards, patient engagement, patient centricity, protocol design, clinical trial medicine kit design

Introduction

A recent study conducted by the Tufts Center for the Study of Drug Development and the Drug Information Association highlights the wide and growing use of patient advisory boards (PABs).[1] The study found that, as of late 2016, 3 out of 4 major pharmaceutical companies have piloted and implemented at least one PAB, making it one of the most commonly implemented patient centricity initiatives.

During the past 18 months, at major industry conferences, a number of pharmaceutical companies including EMD Serono, Bristol Myers Squibb, Sanofi, Merck, and Biogen have reported conducting PABs to amplify the patient voice and gather patient feedback on a variety of clinical trial elements including protocol designs, endpoint measures, clinical trial medicine kits, and study communication materials.[i]

These findings and recent reports are not surprising given the myriad of initiatives supported by the public and private sector to drive higher levels of patient engagement. The private sector has also noted the ease and low relative cost of implementing a PAB in return for the substantial value that it can generate.[1]

In 2015, Dr Lode DeWulf—formerly at pharmaceutical company UCB—recommended considering PABs as part of a regular arsenal of techniques to gather direct input from patients on clinical trial plans and practices.[2] DeWulf noted that patients are increasingly being invited to both dedicated patient panels and meetings with health care professionals to solicit their opinions and feedback.

Merck's former chief medical officer recently noted the growing role that patient voices are playing, and are expected to play, in protocol design decision making.[3] Results from a recent CenterWatch study agree with this observation. The majority of investigative sites consider patient-friendly protocols to be extremely important clinical trial success factors. However, the majority of investigative site staff (55%) reported in the CenterWatch study that clinical research sponsors are currently failing to provide patient-friendly protocols underscoring an unmet need.[4]

Government and public agencies and organizations have been utilizing a variety of methods to solicit patient input over the past 25 years to ensure that they are incorporating diverse perspectives into new practices and policies that affect the development, administration, and oversight of new medical therapies. The Food and Drug Administration (FDA) has been involving patients since the 1990s through the FDA Patient Representative Program.[5] More recently, the FDA has been holding meetings among patients and advocacy groups with select rare diseases as part of its Patient Focused Drug Development (PFDD) initiative and its Patient Engagement Advisory Committee (PEAC) since 2014.

[1] Research Services, Center for Information and Study on Clinical Research Participation, Boston, MA, USA
[2] Tufts University School of Medicine, Boston, MA, USA

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Corresponding Author:
Annick Anderson, MBA, Research Services, Center for Information and Study on Clinical Research Participation, One Liberty Square, Boston, MA 02109, USA.
Email: Annickanderson@ciscrp.org

Established under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V), the purpose of PFDD is 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.[6] Each patient-focused meeting results in a publicly available summary report. FDA expects to host 24 public meetings by the end of 2017.[7] PEAC was enacted to solicit diverse perspectives from patients on the use and regulation of medical devices.[8]

The new PDUFA VI legislation, to be enacted in 2018, goes even farther in emphasizing the importance of including the patient voice in drug development planning and activity.[9]

The European Medicines Agency (EMA), through its patient engagement department, routinely invites patients and patient advocacy group representatives to share their perspectives as participants in scientific advisory panels.[10] These participants are not expected to be highly knowledgeable in any particular medical field; rather, they provide their opinions on a specific medicine for their condition based on personal experiences.

Since 2012, The Center for Information and Study on Clinical Research Participation (CISCRP) has been organizing, hosting, and facilitating patient advisory boards on behalf of pharmaceutical, biotechnology, medical device companies, institutions, associations, and foundations—referred to throughout this article as clinical teams and sponsoring organizations. To date, we have conducted more than 30 PABs across a wide variety of therapeutic areas and disease conditions, and they have focused on an extensive range of areas, including

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

Sponsor companies have been gathering opinions and feedback from patients and patient advocacy groups for some time. However, the practice of systematically soliciting input directly from patients on protocol designs and on other clinical research–specific support areas is still a relatively foreign concept to many sponsor organizations. The purpose of this article is to share lessons learned from CISCRP's experience to inform organizations looking to adopt and implement PABs. It is our hope that pharmaceutical and biotechnology companies will consider implementing PABs as a more standard practice to aid in the development and implementation of patient-friendly and patient-centric protocol designs.

Table 1. Patient Advisory Board Meeting Preferred Structure and Format.

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	• 8-10 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

Abbreviation: PAB, patient advisory board.

PAB Structure

Unlike government-hosted public meetings where 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. We have found that the ideal panel should have both patients who are members of an advocacy group and individuals who are nonmembers to balance and diversify perspectives. 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 additionally helps to bring different views to the discussion. Importantly, panel members are not participants in the study for which the PAB is targeting. And PABs often include caregivers and family members to solicit their valuable perspectives as partners in the clinical trial process. Table 1 summarizes key PAB structure and format elements.

Each PAB typically has one facilitator. Ideally, this individual should not only have experience 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 as an active participant in the discussion. This individual typically helps put participants at ease, lends credibility and trust to the panel discussion, and understands collective patient experience managing a given disease condition.

Pharmaceutical and biotechnology company representatives typically listen without influencing the PAB discussion from a separate observation area. We recommend however 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 recommend that each PAB be structured as part of a series of conversations. One PAB is conducted at a point

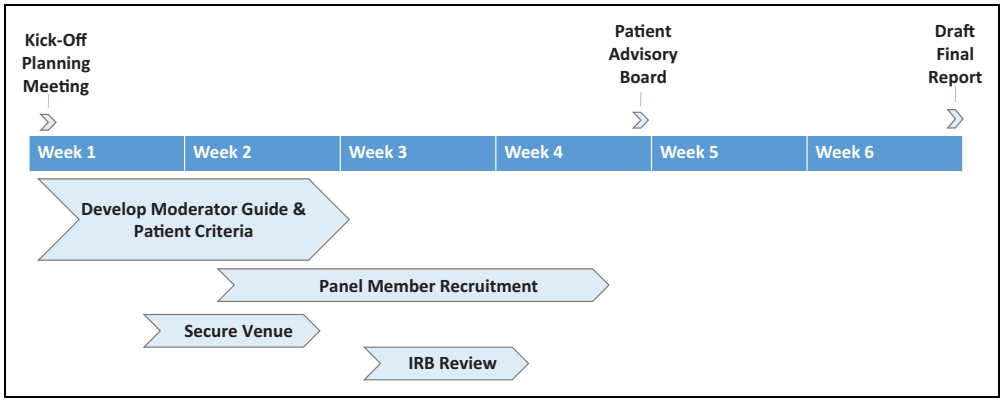

Figure 1. Standard patient advisory board project timeline.

when the clinical team or organization is seeking early input. A second PAB is held at a later date to discuss implementation experience and to solicit input into continuous improvement opportunities.

PAB Format

Ideally, panel discussions should be no longer than 3 or 4 hours 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 conducting patient advisory boards 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 to the advancement of medical knowledge and public health to help panel members better understand the value of their input and participation. With panel member consent, 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 CISCRP has been asked to organize and moderate patient panels over the Internet using social media and video. Virtual formats offer the benefit of reaching diverse patient perspectives from all over the world but, in our experience, they typically only generate broad perspectives rather than generating an in-depth understanding. We generally discourage virtual PABs because they fail to establish a natural dialogue, are far more difficult to probe, and represent a particularly challenging environment to explain complex clinical research concepts.

We have learned 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. Many factors contribute to convenience including ample parking and easy access to the location and the discussion room.

PAB Logistics

CISCRP typically requires 3 to 4 weeks to organize a PAB. Figure 1 provides a breakdown of timeline. A 3- to 4-week time frame allows us to obtain ethical committee review and approval; 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.

We identify potential advisory board members through ongoing interactions with patients who have expressed 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 recruiting advisory board members.

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; venue; planning, organization, facilitation, and reporting on the results.

At the end of each PAB, we encourage members of the clinical team or sponsoring organization to enter the meeting room to 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 sponsor organization.

Subsequent to the PAB, we encourage organizations to keep the panel members apprised of next steps and updates either through a follow-up meeting or written communication.

PAB Impact

Every PAB that we have conducted to date has been an emotionally moving experience for clinical research teams and sponsoring organizations. For many organizations, a PAB represents the first time that they have heard the patient voice, learned about real patient experiences managing their specific illness, and received candid feedback about clinical development plans, materials, and activity.

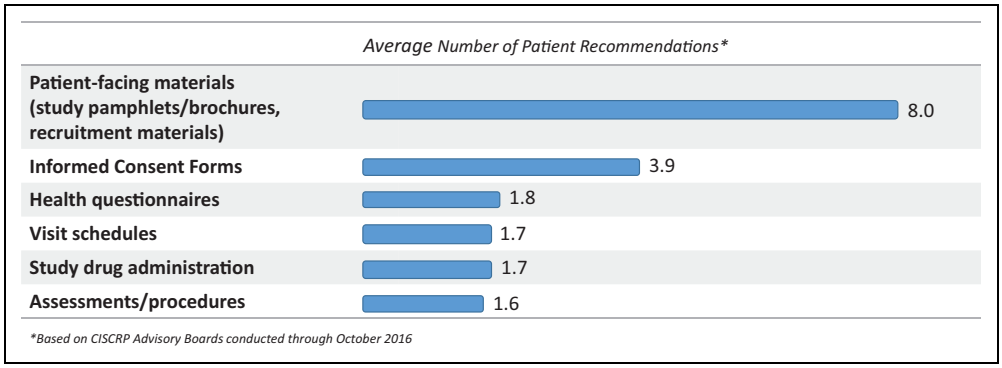

Figure 2. Most frequent categories of patient recommendations.

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 on average two recommendations on ways to improve the visit schedule and on average two recommendations on changes to or a reduction in the number of procedures performed. PABs reviewing informed consent forms, which includes evaluations of content, layout/design and delivery such as eConsent, usually generate an average of 4 recommendations.

One of the most tangible PAB impact areas is the opportunity for clinical research professionals to receive feedback directly from patients: to hear opinions, reactions, and recommendations that are in the patient's own voice and words. For many clinical research professionals, this is a first-time experience and it is 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 teams and between functions supporting a given clinical trial are quickly resolved when the patient has had the opportunity to weigh in and the team gains clarity on the things that matter most to patients.

Conclusions

The following are key takeaways highlighting best PAB practices that will ensure successful engagement with patients, their families and caregivers:

- Treat PABs as **listening** exercises and as natural and genuine discussions; not as market research projects.
- Create a comfortable atmosphere conducive to open conversation—small group size and in-person meetings are preferable.
- Incorporate an educational component on clinical research during each PAB to provide context prior to soliciting feedback on a clinical study or study-related materials. Most patients, caregivers, and family members—including past study volunteers—are not fully informed on the basics, and the educational component offers an opportunity for panel members to warm up.
- Successful PAB discussions must be designed with high sensitivity to the specific health, demographic, and cultural needs of patients. Ideally PABs should be conducted in native languages when conducted abroad.
- Always remember to communicate and show appreciation for panel member participation. At the end of each PAB, thank the participants.
- Employ a collaborative approach with clinical teams and with sponsor organization contacts. Such an approach energizes and engages everyone involved and ensures that the PAB is valued and taken seriously.
- Hold a debrief session among the clinical team or sponsor organization immediately following the meeting to capture the most relevant, top-of-mind insights and discuss next steps.

The wide and growing adoption of patient advisory boards throughout the public and private sectors in the clinical research enterprise is notable and speaks to their value. With ongoing implementation experience, PABs are on their way to becoming a standard and integrated drug development practice with patient engagement at its essence.

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i. Patients as Partners Conference, March 2-3, 2017, Philadelphia, PA.

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