An Evolved Approach to Advisory Boards in Rare Disease Drug Development: 5-Step Model to Finding and Engaging Patient Advisors

Carlotta Dillon, MBA¹, Joyce Knapp², and Mark Stinson³

Abstract
Nearly all new product development teams at pharmaceutical companies will routinely conduct patient advisory boards. These board meetings will help collect and document the experience of patients and caregivers for medical product development and regulatory decision-making. Recently, in June 2020, The US Food and Drug Administration (FDA) published a final guidance on methodological patient-focused drug development (PFDD) to address, in a stepwise manner, how stakeholders (patients, researchers, medical product developers, and others) can successfully use these patient forums. In the process of developing this guidance, the FDA acknowledged that leading its own PFDD meetings, especially when limited to organized disease advocacy groups, cannot address the gaps in information on the patient perspective. So, it has expressed support for advancing the science and utilization of patient input other means. Because traditional methods of conducting patient advisory boards often do not achieve the full potential of patient centricity, the authors of this article share an approach to consider when selecting patient advisors, in order to gain the most actionable input to a product development team.

Keywords
Patient Advisory Councils, patient activation, patient engagement, patient perspectives/narratives, patient expectations, patient feedback

Introduction
Patient advisory boards have a well-established place in the commercialization process (1, 2, 3, 4, 5). There is a trend of conducting these advisory boards earlier in drug development across all clinical trial phases. They help explore unmet needs, generate hypotheses, create research plans, and design trials. In these groups, patients can advocate for new treatments and advise on clinical study design (6).

Although these initiatives are positive contributors to patient centricity (7), they may be limited to “getting feedback,” or worse, just “checking the box” of an expected tactic (8).

We advocate for a systematic approach to listening and understanding that informs the type of data a study will capture, for example, quality of life metrics, physical functioning, social participation or productivity (absenteeism and presenteeism), or other aspects of patient experiences (6).

Collectively, the authors have planned, executed, and facilitated dozens of patient advisory boards and related group initiatives. Through our experience, we share lessons learned and a practical 5-step model to identify, recruit, and involve patients in drug development.

Description
In the following case study, we applied a systematic, multi-platform, stepwise process (see Figure 1) for a company developing a new therapeutic to treat a rare autoimmune-related disease that led to many actionable outputs, such as clarity of patient language, and a better understanding of the role of patient influencers in this disease. The process is akin

¹ Dillon Consulting, Jupiter, FL, USA
² Knapp Healthcare Communications, New York, NY, USA
³ Bioscience Bridge LLC and 83bar LLC, Boise, ID, USA

Corresponding Author:
Mark Stinson, Bioscience Bridge, LLC, 6310 W Plantation Lane, Boise, ID 83703, USA.
Email: mark@biosciencebridge.com
to the funnel approach used to identify, profile, and recruit candidates for clinical trials.

- **Step 1: Create a patient advisor identification strategy**—begin with the end in mind

What does the client want to learn from engaging patients? What will be produced as a result? The answers to these questions will direct the patient profiles most likely to deliver the required outcome.

In our rare disease case, the development team’s goal was to better understand the overall patient journey, in order to identify care touchpoints at which the new product might make a difference to patients. We also wanted input on a target product profile (TPP) (9).

Examples of other types of goals could include guidance on protocol design, consent forms, patient instructions, and experience with existing products. Each of these may require a different patient profile for optimal results.

- **Step 2: Leverage social media to get real-world patients for initial recruitment**

Identifying the right patient advisors becomes more complicated in a rare disease category because the patient pool is so much smaller. To complicate things further, in our rare disease category, the vast majority of potential patients were still undiagnosed. For this reason, the traditional strategy of targeting an advocacy organization for diagnosed patients was not possible. We first learned this when working with a grassroots patient community leader who was building a database of like-minded patients seeking educational resources—and finding what a limited number of patients would be accessed this way.

Therefore, we cast a wide net across message boards, Facebook groups, and other social media with personal stories and a clear call to action. Multiple campaign platforms were tested for patient feedback to optimize the message and drive qualified responses. For example, we learned that the word “development” was more motivating than “research” or “studies” because it connoted progress. We also formalized a process to ask each qualified patient to provide another referral of someone they might know. As a result of this outreach approach, approximately 100 patients met the criteria to be considered.

- **Step 3: Conduct online surveys and health assessment questionnaires to qualify candidates**

To further evaluate the patients’ qualifications, the 100 potential advisors participated in a series of online surveys related to their current experiences and the emotional impact of their condition. It was useful to reference quality of life instruments, which are available for many disease conditions.
outcome to be in better health, but his journey to reach that
was key for our client to hear the outcomes that the end
and nervous.
To assess their personal health engagement, the survey
asked patients to rate agreement with statements such as:
“I am responsible for taking care of my health,” “I tell my
doctor my concerns even if he or she does not ask,” and
“I can maintain lifestyle changes even during times of
stress.” On the basis of these responses, we narrowed the
field to approximately 50 potential advisor candidates.

- Step 4: Conduct Web-based discussion panels to
assess group dynamics

In this step, we assessed how individuals might interact
together. A high level of interactivity and the ability to com-
municate personal insights in a group setting is critically
important to the success of an in-person patient advisory
board meeting.

This step provided us an opportunity to hear patient advi-
sor candidates define the disease in their own terms, to fol-
low the varied diagnostic pathways, and to understand the
wide range of treatment approaches.

In our rare disease example, we wanted patient advisors
who had varied experiences and who also could relate to
others at different stages of their rare disease journey. Using
webcams with the moderated group allowed for a live plat-
form with participants from across the country. Topics ran-
ged from attitudes about doctor visits to the social,
emotional, and health impact of their condition. At the con-
clusion of this step, we were able to compile the final invita-
tion list of 10 individuals.

- Step 5: Convene patient advisory meeting with client
clinical and commercial team

We facilitated a one-and-a-half-day workshop with
Patient Journey as the centerpiece, using highly interactive
mapping exercises. The emotional language of the patient
was key for our client to hear the outcomes that the end
customer really wants, in contrast to the clinical language
of trial efficacy measures and Food and Drug Administration
definitions—ie, the usual lexicon of the clinical development
team.

Other industry colleagues have published similar exam-
pies illustrating the need to balance medical outcome with
the medical journey and individual aspirations. One case
study reads: “a father with diabetes and heart disease wants
to follow his doctor’s orders to reach his desired medical
outcome to be in better health, but his journey to reach that
outcome is difficult to manage. He is a bus driver and the
prescribed medications make him drowsy. This man’s
aspiration is to make sure he can work to provide a better
life for his children. It is only by prioritizing this aspiration
and altering the journey by finding a treatment that will
allow him to keep working that the patient can achieve the
desired medical outcome” (15).

Results
Taking this different approach yielded meaningful benefits.
Specifically, we gained potentially significant input for
changes in protocol design, suggestions for word changes
for the TPP and labeling, and key patient experiences that
can be shared with health care providers, trialists, guideline
developers, and others. From a patient’s perspective, we
conducted a post-meeting survey in which participants
expressed gaining new knowledge of their disease, a
renewed attitude of empowerment, and a sense of actions
they could take to improvement their own well-being, as
well as contribute to new drug development.

In our work with other companies and contract research
organizations, we also have used patient advisors to seek
perspectives on clinical trial kit designs, a digital diary app,
study outreach communications, and packaging.

Furthermore, one important side benefit emerges within
the company when cross-functional teams are involved:
shared ownership roles that help create continuity from
research and development to launch, including appropriate
outcomes and communications.

Lessons Learned
Investing early in patient advisors is a sound strategic move
that increases a company’s chances of successfully develop-
ing a product with the patient/customer in mind. In addition,
it helps the sponsor team embrace advocacy as its members
work together toward patient-focused goals.

As health care systems continue to evolve and establish
patients as the primary stakeholder in their health care
decision-making, the pharmaceutical industry will need to
be innovative to demonstrate the value of their products
relative to the outcomes experienced by patients. Pharma-
aceutical companies should recognize the value of involving
patients across the entire product life cycle and work to
transform present perceptions and practices throughout their
organizations (16).

Conclusion
The practical 5-step model summarized in the article can
improve the way drug development teams identify, recruit,
and engage patients as advisors and partners in the new drug
development process.

When you reorient the way patients are identified and
engaged, the common everyday tactic of a patient advisory
board takes on a new potential. And true patient-centricity emerges.

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**ORCID iD**
Mark Stinson https://orcid.org/0000-0002-4242-6339

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**Author Biographies**

Carlotta Dillon is principal of Dillon Consulting, with patient-focused experience on commercial and clinical teams at pharma companies including Adare, Endo, AstraZeneca, and DuPont Merck.

Joyce Knapp is president of Knapp Healthcare Communications, specializing in patient and professional meetings and strategic medical communications.

Mark Stinson is principal of Bioscience Bridge, a medical agency veteran in brand marketing, strategic planning and research, and group facilitation. He is also on the management team of 83bar, a patient activation company.