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Incorporating Patient Advocates in Oncology Clinical Development: Lessons Learned From a Novel Pilot Program

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Abstract

The advent of patient-focused drug development (PFDD) has underscored the priority of engaging the "voice of the patient" in therapy development. Industry sponsors are working to enhance engagement of patients early, particularly within decision making for design and execution of clinical trials. This trend is especially significant within oncology, as industry leaders partner with patient advocacy organizations, individual patients, and clinicians to enhance patient-centricity. These partnerships often require a willingness to change attitudes, approaches, and processes to reshape traditional models of drug development. In 2016, Bayer Oncology launched a pilot program called the Patient Advocate Advisory Council (PAAC), to design and execute a program whereby patients join clinical development teams. The PAAC, composed of experienced patient advocates from the US and Europe, worked closely with company leaders to design and execute a pilot in an ongoing clinical development program. The PAAC and Bayer teams have identified important learnings from the first phase of the program, emphasizing earlier engagement of patient advisors, launching the enhanced training platform, and recruiting additional PAAC members to expand the initiative's reach across the cancer community. A critical success factor is having champions for patient engagement within the company to ensure that activities are streamlined and standardized as patient engagement becomes more common. This is particularly important given that patient engagement should be a long-term investment with sufficient and sustained resources. PAAC members and Bayer have committed to sharing learnings, to advance opportunities for successful patient engagement in drug development throughout the oncology therapeutic landscape.

Keywords

patient engagement, patient advocacy, patient centricity, drug development, oncology

Background

The advent of patient-focused drug development (PFDD)[1] has brought into sharp focus the priority of establishing a systematic approach to engaging the "patient voice" in therapy development. This is especially true in the US and Europe, where legislative and regulatory activities are defining, measuring, and advancing patient centricity and empowerment.[2-4] Increasingly, stakeholders are developing processes and systems to embed the voice of the patient into therapy development and treatment decision making.[i]

These trends reflect an environment where patients are seeking a more proactive, dynamic, and central role in their health care, as they move from the role of passenger to one of "co-pilot."[5]

Industry sponsors have worked to enhance patient engagement in drug discovery, particularly within clinical trial planning and decision making.[6-8] This trend is especially significant within oncology, as company leaders partner with patient advocacy organizations, individual patients, and leading clinicians to enhance patient-centricity. These partnerships require a willingness to change attitudes and processes, reshaping models of drug development that have traditionally been somewhat disconnected from patients.[9]

In 2016, Bayer Oncology launched a pilot program called the Patient Advocate Advisory Council (PAAC), whose purpose was to bring patients into clinical development teams. The

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PAAC, comprised of experienced patient advocates in the US and Europe,[ii] worked with company leaders to implement a pilot patient engagement program, taking advantage of a planned Phase 2 oncology trial.

This paper summarizes the PAAC initiative and key learnings, as the PAAC model is integrated across the company's oncology pipeline and its leaders share best practices.

Methods

In 2016, for its annual Patient Advocacy Summit, Bayer leadership challenged patient advocacy organizations to present proposals to help integrate the patient voice earlier in clinical development processes. Diverse teams of patient advocates representing national advocacy organizations brought specific program ideas for review by company leaders and prominent patient advocates.

The review panel assessed the ideas for innovation and creativity, direct benefits to patients, effectiveness of a roll-out plan, ease of implementation, and benefit to the company's portfolio and processes. The winning proposal, to launch a pilot project for systematic integration of patient perspectives into the company's clinical trial process, was granted a 1-year budget for a 5-member Patient Advocacy Advisory Council (PAAC).

Results

In its first phase of work, the PAAC developed a blueprint to systematically engage patient advisors (also known as expert patients or patient advocates) and built an execution strategy using an ongoing Bayer development program as its pilot.

The 2016-2017 PAAC project deliverables included

- defining and developing Patient Selection Criteria and a "role description" for patient advisors chosen to participate in clinical development activities;
- recruiting suitable patient advisors;
- developing a training curriculum framework and platform for patient advisors and relevant audiences inside the company;
- implementing seamless training, communication, and mutual learning with identified patient advisors, including assisting Bayer in establishing appropriate "rules of engagement" between patients and clinical drug development teams; and
- piloting the new framework and approach through an ongoing company development program, including evaluating and adapting the model based on learnings from the pilot.

In late 2016, the PAAC conducted a series of discussions with Bayer leaders in advocacy relations and clinical development to plan the pilot. With access to information about one of the company's clinical development programs under confidentiality agreement, PAAC members provided feedback to the clinical team about specific elements of a trial protocol from a patient perspective and identified models for integration and training to achieve successful engagement of a patient advisor into the program. Discussions focused on ensuring stakeholder agreement about appropriate expectations for the patient advisor's level of experience, time commitment, and compensation. Recommendations recently published by the Clinical Trials Transformation Initiative (CTTI) for stakeholder collaborations in clinical trials were incorporated.[10]

PAAC members worked along parallel tracks to define general patient advisor selection criteria and role description documents (see Figure 1), develop training curricula modules for both patient advisors and audiences within the company, while identifying and onboarding a relevant cancer patient advisor for engagement with the clinical development team working on the study selected for the PAAC pilot. The selected patient advisor began engagement with the project in the spring of 2017, meeting by phone with key members of the study team and providing feedback on the protocol and development program.

The PAAC developed materials to populate an interactive, expandable training platform for patient advisors and company staff. Content was compiled and reviewed by company stakeholders, including legal and compliance, and then incorporated into a web-based architecture built by an external consultant and tested by the PAAC. This platform utilizes the Friends of Cancer Research ProgressforPatients.org regulatory education online learning program.[11] The training platform is flexible, allowing for easy updates and augmentation as needed to incorporate additional content and tools.

Discussion

The PAAC met with company leaders during the 2017 ASCO Innovation Summit to review the project and evaluate its success. All stakeholders were generally pleased with the results of the pilot and agreed to leverage its learnings and expand its reach within the company's development programs. A key learning from the pilot was that patient advisors can and should be engaged with a development program *even earlier* than was possible with the pilot study. PAAC members and their company colleagues agree that, despite the risk that therapy development programs in the earliest stages may not advance to later development, it is nonetheless important to ensure these early programs are designed with direct patient input.

As summed up by Bayer's President of Pharmaceuticals, Americas: "The collective team netted out that the recruitment of patient advisors who can consult on clinical trial design should begin as soon as program discussions around drug administration and target indication are approved."[12]

Additional learnings from the pilot PAAC project included: tackling challenges within existing corporate culture; appropriately defining and clarifying roles and responsibilities for patient advisors and company study team members; anticipating and meeting resource needs; and addressing logistical and communications issues (see Figure 2).

PAAC Recommended Patient Advisor Roles & Responsibilities

When possible, Patient Advisor should be engaged in:

- Discussion of unmet need (definition of ideal drug properties, including IV vs. PO, side effect profile, administration frequency)
- Input into formulation options (ex: coated caplet, dispersible tablets, tablets)
- Draft TPP (Target Product Profile)
- Advisory Board member when appropriate
- PRO development and insight into patient perspectives (including wearables, and apps)
- Draft protocol review (including inclusion/exclusion criteria; frequency of visits, assessment details)
- Site feasibility questionnaire
- Patient recruitment materials (feedback on patient facing documents)
- Informed Consent review
- Study collateral materials (feedback on patient facing documents)
- Development of patient journey (@ time of registration)
- Patient Education materials (@ time of registration) (feedback on patient facing documents and treatment management / shared decision making)
- Evaluation of risk/benefit, QoL and PRO
- Regulatory body interactions if applicable
- Input into design of real life experience studies (RLE)
- Support education of call centers to better understand patient's journey
- Input on 2nd generation drug improvements

Figure 1. PAAC recommended patient advisor roles & responsibilities. A key objective of the Patient Advocate Advisory Council (PAAC) pilot program was to define a "role description" for patient advisors chosen to participate in clinical development activities. The full range of advisors' roles and responsibilities are listed here.

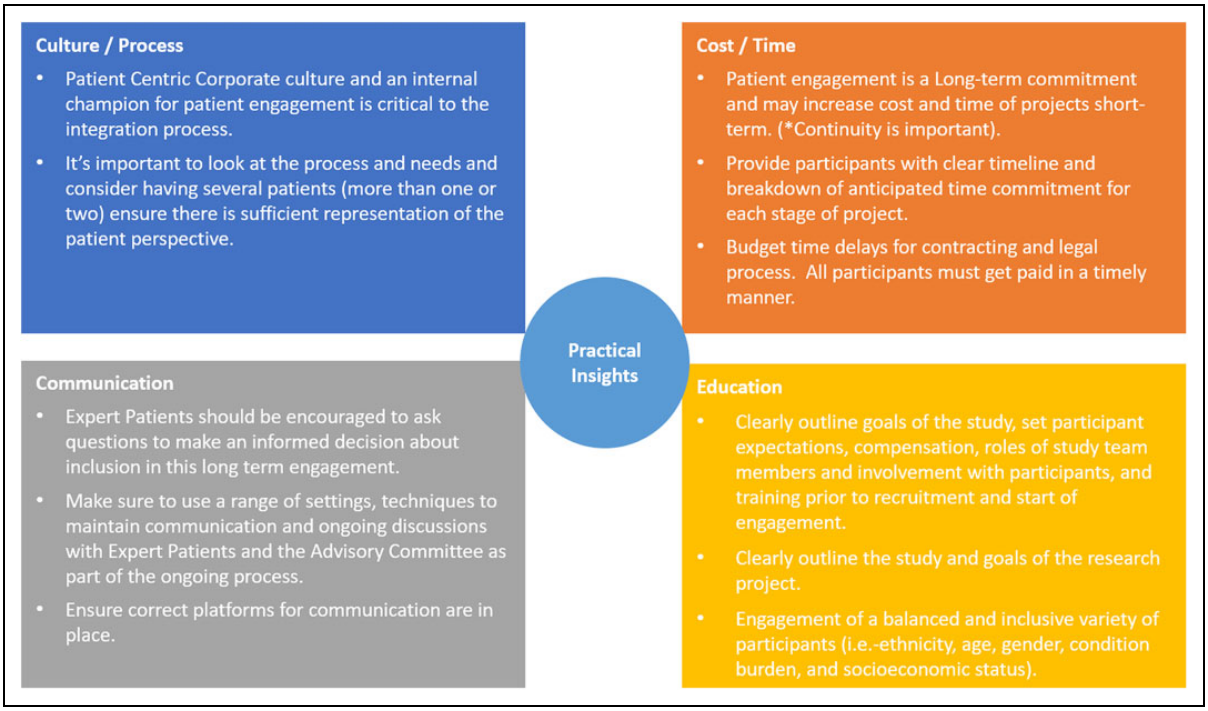

Figure 2. Lessons learned from PAAC Pilot. Key lessons drawn from the PAAC initiative encompassed several thematic areas, including tackling challenges within existing corporate culture; appropriately defining and clarifying roles and responsibilities for patient advisors and company study team members; anticipating and meeting resource needs; and addressing logistical and communications issues.

Specific challenges and solutions identified by the PAAC and company stakeholders were:

Challenge: Ensure seamless and sufficient, ongoing two-way communication, and prioritize efforts to coordinate necessary discussions involving the patient advisor.
Solutions: Identify and onboard patient advisor early in the trial process to allow for regular communication touch-points and appropriate inclusion in key discussions. Use a range of settings and techniques to facilitate appropriate inclusion of the patient advisor and PAAC members.
Challenge: Address barriers within existing corporate culture that impede patient engagement.
Solutions: Develop an appropriate compensation structure for patient advisors, streamline necessary legal and contractual processes, and establish common expectations between the team and the patient advisor.
Challenge: Carefully select the right patient advisors.
Solution: Consider involving more than one patient in a study team to enhance representativeness of perspectives and skill sets.

A critical success factor is having champions for patient engagement within the company to mitigate obstacles and streamline activities as patient engagement becomes more commonplace within the company. This is particularly important given that patient engagement should be viewed as a long-term investment with sufficient and sustained resources.

Patient engagement in drug development programs is expanding quickly as the field evolves and best practices are developed. Stakeholders are actively exploring novel methods for measurement of success and returns on investment. The PAAC and Bayer team are committed to evaluating relevant metrics from the pilot program, including reviewing the number of amendments to the study, quantity and type of suggestions from the expert patient included in the study design, number of additional study teams that adopt this approach, and ability to engage patients even earlier in the process by those study teams.

While the pilot is still ongoing, there have already been positive effects from the program. For example, there were important insights from the expert patient relating to crossover design considerations and type of information provided to patients. In addition, the expert patient was involved in discussions about feasibility for capturing regular data from patients and in formulating informed consent materials.

The study team will ultimately evaluate how these insights impact protocol execution or result in savings (in time, money patient resources etc.). While this process continues, there has been significant interest expressed within Bayer among other study teams (and even other therapeutic areas beyond oncology) to adopt the program.

The pilot program has enabled creation of a robust training platform for expert patients and internal company audiences, providing an opportunity to seamlessly integrate patients into Bayer's clinical development program, rapidly expand the program to additional study teams, and elevate understanding of best practices for patient engagement within Bayer.

Conclusion

Based on successful execution of the Pilot program, Bayer has committed to continuing the PAAC initiative and expanding its application. The PAAC and Bayer teams have identified important learnings from the first phase of the program and are incorporating them into the program's next phase, emphasizing earlier engagement of patient advisors, launching the enhanced training platform, and recruiting additional PAAC members to expand the initiative's reach across the cancer community.

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Declaration of Conflicting Interests

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Supplemental Material

Supplemental material for this article is available online.

Notes

i. For example, the ECCO summit in Sept 2018 (Vienna) has the chair of its patient advisory committee as co-chair of the meeting. More to the point, there is *no* patient track, the patient voice is embedded in every single facet of the event with speakers, panellists, and chairs. The importance of this cannot be overstated as the summit will be a decision-making process based on recommendations formulated by the summit discussion and work preceding the event to ensure that patients are no longer a passive recipient of decisions made in their absence.
ii. Ian Banks, MD, European Men's Health Forum; Anjelica Davis, MPPA, Fight Colorectal Cancer (FightCRC); Ryan Hohman, JD, Friends of Cancer Research (Friends); Lisa Schlager, Facing Our Risk of Cancer Empowered (FORCE); Wendy Selig, MSJ, WSCollaborative (former CEO, Melanoma Research Alliance).

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