

# The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials

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Diane Bloom, PhD, MPH<sup>1</sup>, Joel Beetsch, PhD<sup>2</sup>,  
Matthew Harker, MPH, MBA<sup>3</sup>, Sharon Hesterlee, PhD<sup>4</sup>,  
Paulo Moreira<sup>5</sup>, Bray Patrick-Lake, MFS<sup>6</sup>, Wendy Selig, MSJ<sup>7</sup>,  
Jeffrey Sherman, MD, FACP<sup>8</sup>, Sophia K. Smith, PhD<sup>9</sup>,  
James E. Valentine, JD, MHS<sup>10</sup>, and Jamie N. Roberts, MPH, MA<sup>6</sup>

## Abstract

**Objective:** To identify the elements necessary for successful collaboration between patient groups and academic and industry sponsors of clinical trials, in order to develop recommendations for best practices for effective patient group engagement. **Methods:** In-depth interviews, informed by a previously reported survey, were conducted to identify the fundamentals of successful patient group engagement. Thirty-two respondents from 3 sectors participated: patient groups, academic researchers, and industry. The findings were presented to a multistakeholder group of experts in January 2015. The expert group came to consensus on a set of actionable recommendations for best practices for patient groups and research sponsors. **Results:** Interview respondents acknowledged that not all patient groups are created equal in terms of what they can contribute to a clinical trial. The most important elements for effective patient group engagement include establishing meaningful partnerships, demonstrating mutual benefits, and collaborating as partners from the planning stage forward. Although there is a growing appreciation by sponsors about the benefits of patient group engagement, there remains some resistance and some uncertainty about how best to engage. Barriers include mismatched expectations and a perception that patient groups lack scientific sophistication and that “wishful thinking” may cloud their recommendations. **Conclusions:** Patient groups are developing diverse skillsets and acquiring assets to leverage in order to become collaborators with industry and academia on clinical trials. Growing numbers of research sponsors across the clinical trials enterprise are recognizing the benefits of continuous and meaningful patient group engagement, but there are still mindsets to change, and stakeholders need further guidance on operationalizing a new model of clinical trial conduct.

## Keywords

patient group engagement, actionable recommendations, clinical trials, therapeutic development, best practices

## Introduction

Today's patient groups are not one-size-fits-all: there are small, fledgling groups with limited skills and resources as well as mature organizations with considerable scientific expertise and extensive assets. Historically, industry and academic research sponsors have looked to patient groups primarily to provide a source of participants for large-scale phase 3 clinical trials. However, the more sophisticated, new generation of patient groups seeks more meaningful involvement in the entire drug development process, especially for drugs focused on rare disease conditions with a very defined and limited target population. Some pharmaceutical and biotech companies have responded to the push by patient groups to involve them earlier in the process, when their input can make a difference in

<sup>1</sup> Gillings School of Global Public Health, University of North Carolina, Chapel Hill, NC, USA

<sup>2</sup> Celgene Corporation, Summit, NJ, USA

<sup>3</sup> Duke-Margolis Center for Health Policy, Durham, NC, USA

<sup>4</sup> Parent Project Muscular Dystrophy, Hackensack, NJ, USA

<sup>5</sup> EMD Serono, Rockland, MA, USA

<sup>6</sup> Clinical Trials Transformation Initiative, Durham, NC, USA

<sup>7</sup> WSCollaborative, McLean, VA, USA

<sup>8</sup> Drug Information Association/Horizon Pharma, Lake Forest, IL, USA

<sup>9</sup> Duke University School of Nursing, Durham, NC, USA

<sup>10</sup> Hyman, Phelps & McNamara, PC, Washington, DC, USA

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## Corresponding Author:

Jamie N. Roberts, MPH, MA, Duke Clinical and Translational Science Institute, Durham, NC 27710, USA.

Email: jamie.roberts@dm.duke.edu

shaping study protocols. Proponents assert that bringing the voice of the patient to the clinical trial planning process can provide insights into the experiences, capabilities, and wishes of the patient population, ultimately leading to more efficient recruitment and shortened timelines. Patient insights can help researchers plan realistic and feasible protocols, define patient-specific clinical outcomes, and refine the inclusion/exclusion criteria, saving sponsors from time-consuming and costly amendments to their protocols.

With growing interest in patient engagement and patient centrality among diverse stakeholders in the clinical trials enterprise, as well as a commitment to patient-focused drug development (PFDD) by the US Food and Drug Administration (FDA), a significant opportunity now exists to improve the development of therapies and enhance participation by patient groups in the work of trial sponsors.<sup>1</sup> The term *patient-focused drug development*, as used here in the broader sense, refers to the meaningful engagement of patients in the development of therapeutic products and the various important roles patient groups can play in improving the entire clinical research enterprise. These include involvement in natural history and patient-preference studies that can inform target selection and clinical research design, selection of study endpoints that reflect outcomes of importance to patients, development of reasonable inclusion and exclusion criteria, study designs that are less burdensome and enable more efficient trial recruitment, engagement in the regulatory process, and more effective post-marketing safety activities. Although several initiatives have been undertaken to enhance the role of patients in clinical research (eg, the European Patients' Academy on Therapeutic Innovation [EUPATI] and Patient-Focused Medicines Development [PFMD]), more guidance is needed about how, when, and with whom trial sponsors and patient groups should engage—and which approaches yield the best results for all parties and the greater public.<sup>2–6</sup> PFDD and patient group engagement efforts around clinical trials should extend the benefits of patient insights and experiences in a capacity that helps accelerate and improve research agendas. However, frameworks and metrics for measuring the value of such engagement are lacking.

The Clinical Trials Transformation Initiative (CTTI)—a public-private partnership whose members include representatives from the FDA, National Institutes of Health, the Centers for Medicare and Medicaid Services (CMS), and academic, industry, and patient advocacy groups<sup>7</sup>—formed the Patient Groups and Clinical Trials project team to help delineate best practices for patient group engagement that ensure the needs of all parties are met. In this article, we use the term *patient group* to encompass patient advocacy organizations, disease advocacy organizations, voluntary health agencies, nonprofit research foundations, and public health organizations. We do not refer here to individual patients or advocates—although patient groups may engage patients or advocates for clinical trial activities with sponsors.

Our intention is to show that by identifying the key factors of successful, effective engagement with patient groups, there

is opportunity for the voice of the patient to be woven into the fabric of the clinical research enterprise, thus enhancing the quality, efficiency, and relevance of therapeutic development.

## Methods

The CTTI project team consisted of engagement experts from patient advocacy organizations, industry, academia, and the FDA. The majority of team members have been instrumental throughout their careers in shaping the evolving field of patient engagement. The team, together with CTTI's Patient Leadership Council, identified a list of recognized leaders in the field who could provide insights into the development of meaningful partnerships, and invited them to participate in a 45- to 60-minute semistructured in-depth telephone interview. The interviews were designed to explore, from different perspectives, the topic of engagement between patient groups and research sponsors around the design and operation of clinical trials. The participants interviewed represented a nonrandom, purposive sample recruited as reflective of the target population. In alignment with CTTI's Strategic Plan, the interview candidates were selected in part for their ability to provide insights relevant to CTTI's primary focus on regulatory submission trials with a US perspective, and all interview respondents were based in the United States. The Duke IRB approved this study as exempt; therefore, participant consent was assumed by a willingness to participate in the interview.

A discussion guide of questions for each of the 3 groups was developed and refined by the project team in consultation with an independent qualitative researcher and professional moderator (Supplement S1). Some of the interview questions were informed by findings from a previously reported survey.<sup>8</sup> The team identified and invited senior-level leaders from patient groups, academia, and industry to participate. Letters describing the study and requesting participation were sent to each of the 41 potential respondents, and 32 interviews were conducted with those who replied: patient groups (n=10), academia (n=10), and industry (n=12). The interviews were conducted from September through November 2014. All interviews were audio-recorded for the purposes of analysis and report writing.

Respondents demonstrated engagement experience and active cross-sector partnerships (Table 1). Representatives from industry were proponents of patient engagement and were leading business units that were engaging with patient groups around research and development activities. They held positions with titles such as “director of advocacy in the R&D space,” “patient advocacy specialist,” or “vice president in charge of patient advocacy and external relations.” As part of their job responsibilities, all were working on ways to engage more effectively with patient groups and were encouraging their colleagues to do the same. Both small and large companies were represented in the interviews as were rare and common disease groups. Academic respondents were professors, physicians, investigators, clinical trialists, and biostatisticians who have engaged with patient groups in a variety of

**Table 1.** Characteristics of Interview Respondents (N=32).

Sector	Activities	n
<b>Industry representatives</b> were leaders of pharmaceutical companies supporting and funding clinical trials. All were champions of working with patient groups in clinical trial endeavors.	Design clinical trial protocols and materials Work on ways within their companies to engage more effectively with patient groups Promote and encourage patient engagement activities within their companies	12
<b>Academic representatives</b> were experienced principal investigators with responsibility for conducting clinical trials funded by both industry and federal agencies.	Design protocols and materials and implement clinical trials	10
<b>Patient group representatives</b> were founders or executive directors of patient advocacy organizations; disease advocacy organizations; voluntary health organizations; nonprofit research organizations; and public health organizations. Respondents reflected a spectrum of disease conditions.	Support patients and families Educate about disease treatments, risks, quality of life Shape the research agenda Influence policy Fund research Operate patient-powered research networks	10

partnerships surrounding clinical and translational research. Respondents from patient groups were managing research partnerships, funding research, and educating and convening their communities around clinical trial activities.

A comprehensive report of the findings from the qualitative research was presented to a multistakeholder group of experts at a meeting held January 2015 (Supplement S2).<sup>9</sup> This meeting was an opportunity for attendees to provide feedback on the survey and interview results, consider the project team's analysis, challenge assumptions and identify gaps, work in subgroups to refine the factors necessary for successful collaboration, and establish detailed recommendations for best practices for both patient groups and research sponsors. Experts from industry, academia, patient groups, and government presented case studies of successful partnerships, followed by moderated discussions and breakout sessions.

## Results

From analyses of the multistakeholder input and extended discussions, the project team delineated (1) the perceived benefits of and barriers to collaborating with patient groups around clinical trials, (2) three fundamentals that characterize successful patient group engagements, and (3) a set of actionable recommendations for effective collaborations between sponsors and patient groups conducting clinical trial research. We describe these results below.

### *Perceived Benefits of Patient Group Collaborations*

A growing number of pharmaceutical and biotech companies have responded to the push by patient advocacy groups to involve them earlier in the clinical trial process, when their input can help shape study protocols, clinical endpoints, assessments, informed consent documents, patient materials, and eligibility criteria. Per the industry respondents, involving patients in the development of therapeutics is not only “the right thing to do” but there is also a compelling business case

to be made. Engaging with patient groups early in the process can speed up clinical trial accrual and retention by providing insights into the experiences, capabilities, and wishes of patients in the target population. This information is helpful to the clinical trial team in developing more feasible protocols, appropriate eligibility criteria, tolerable event and procedure schedules, and patient outcomes that may be critical to their decision to participate. In addition, “getting it right the first time” helps pharmaceutical companies avoid costly and time-consuming modifications and protocol amendments that can affect development timelines.

### *Perceived Barriers to Patient Group Collaborations*

Not everyone on industry clinical trial teams is committed to involving patient groups, especially in the early planning stages of trial design. Some industry respondents said their colleagues believe patient groups can offer only a limited amount of information beyond what the clinical trial team already knows about a particular disease, and that having to interact with an additional group would delay their timelines. Industry respondents said that changing mindsets would involve using metrics to demonstrate the value of involving patient groups in the process, but that these kinds of studies would likely never be done. Other barriers include a lack of understanding about how best to engage with patient groups; mismatched expectations and priorities between trial teams and patient groups; a perception that patient groups lack sophistication on scientific questions of study design; and a perception that the “wishful thinking” of patient advocates can cloud their recommendations. Further exploration by the project team revealed that many of these barriers can be overcome.

### *Three Themes Characterizing Successful Patient Group Engagement*

The team's thematic analysis suggests that the following areas of focus are intrinsic to effective engagement efforts between

patient groups and sponsors, and could be instrumental in overcoming some of the identified barriers: (1) establishing meaningful partnerships, (2) demonstrating mutual benefits, and (3) collaborating early and often.

#### **Establishing meaningful partnerships**

There was a recognition among those interviewed that meaningful partnerships depend on choosing the right partners based on complementary interests, capabilities, expertise, and resources. Most patient groups said they work with more than one sponsor at a time and try to pick those with promising products with a high likelihood of making it to market. Most sponsors also tend to work with multiple patient groups in a particular disease area. Among the factors sponsors look for when choosing collaborators are the size of the patient group's constituency and reach; the effectiveness of their website; their social media savvy; their track record in past partnerships; the extent of their assets (including funding); and their level of expertise in trial recruitment.

Respondents from all groups said that before embarking on a collaboration, it is important to manage expectations, set ground rules, and define the roles each partner will play. Identifying shared goals (eg, shortened timelines to bring a therapy to the patients who need it faster) provides the foundation for each partner to be a valued part of the process. Effective communication skills and trust, defined as honoring confidentiality, were also cited as important.

#### **Demonstrating mutual benefits**

The assets of patient groups, both tangible and intangible, can be extremely valuable to research sponsors, and can serve as bargaining chips in exchange for meaningful (as opposed to token) involvement as full partners in the clinical trial process. The most commonly cited assets were patient registries, tissue or blood banks, and a nuanced understanding of the target patient population.

In addition to their traditional role of aiding in recruiting study participants and communicating with patients and their families about the trial, patient groups have intimate knowledge of their constituents and can provide helpful information about disease burden, unmet needs, and outcomes that will make a difference to patients' lives. Patient groups are increasingly recognizing the importance of demonstrating their assets to sponsors in order to make a persuasive case for early participation and recognition as an essential partner. There is opportunity for mutual benefit when patient groups allow sponsors access to their data and sponsors incorporate patient group insights and ideas into target selection, outcomes, and trial design.

#### **Collaborating early and often**

Historically, patient group input was relegated to late in the clinical trial process—after target selection, the protocol, clinical endpoints, eligibility criteria, and trial recruitment materials were established. However, per those interviewed, not having patient groups involved from the beginning of trial

design is one of the biggest barriers to successful partnerships. Those who have engaged in successful partnerships said that patient groups can add a great deal of value to clinical trial accrual and retention by providing insight into the experiences, capabilities, and wishes of their patient population. Some patient groups reported that increased investment in education, not only in the science of the disease they represent but also in clinical trial methodologies, contributes to their ability to offer meaningful input in trial development. They assert that bringing the voice of the patient to the early planning process makes clinical trials more relevant, acceptable, and tolerable for patients, and ultimately leads to shortened timelines in which new drugs can be brought to market. Maintaining regular communication with patient groups throughout all aspects of the clinical trial, even when there is no new study news to report, is also important to the ongoing relationship.

#### **Actionable Recommendations for Successful Collaborations**

The project team synthesized all multistakeholder input into a comprehensive set of final actionable recommendations for successful engagement with patient groups and published it on the CTTI website in October 2015, along with 3 tools to facilitate collaborations.<sup>10,11</sup> Table 2 presents a high-level overview of these final recommendations. The full recommendations report and related deliverables are available at the CTTI website (Supplements S3-S6).<sup>10</sup>

#### **Discussion**

At the same time that a new generation of patient groups is developing diverse skillsets and acquiring assets to obtain an “equal” place at the table with trial sponsors, a growing number of sponsors and investigators are recognizing the benefits of continuous and meaningful patient group engagement. It is notable that the patient group leaders consistently used the term “equal partners,” while the sponsors used the terms “valued” or “important” partners. The sponsors viewed collaborations around clinical trials as a division of labor, whereby each partner group brings a different but complementary strength to the enterprise. Most viewed the patient groups as having a nuanced understanding of the preferences and physical limitations of their patient communities, and that their most valuable contributions lie in bringing the voice of the patient to trial design, recruitment, and retention. The sponsors saw themselves as bringing expertise in science, medicine, and clinical trial design. Ideally, their joint efforts generate a win-win for all involved. The academic investigators said that while they listen to the patient group input, the investigators should retain ultimate decision-making authority since they have more expertise and want science to drive the research.

The sponsors interviewed were, by design, proponents of patient engagement and enthusiastic about the value of this process. However, they acknowledged that not all of their colleagues see the value of involving patient groups, and that those

**Table 2.** Recommendations on Best Practices for Effective Patient Group (PG) Engagement.

Recommendation	Details and Actions
<b>FOR ALL STAKEHOLDERS</b>	
Engage the “patient voice” by establishing partnerships from the beginning of the research and development program to improve trial design and execution	<p>Include the perspective of patients (ie, the “patient voice”) in the early stages of disease targeting. Sponsors benefit by a clearer, more focused understanding of unmet need, therapeutic burden, opportunities for expanding indications, and better targets.</p> <p>Patients benefit by less burdensome study protocols and more meaningful and relevant endpoints, increasing the likelihood they will participate in the trials or potentially help to develop a meaningful treatment for their disease.</p>
From the start, clearly define the expectations, roles, and responsibilities of all partners, including the resources being committed, data being shared, and objectives of the program	<p>It is important to clearly delineate the roles of partnership and clarify the goals and objectives of the collaboration.</p> <p>Expectations about the role of PG consultation and input should be clarified at the start of the collaboration.</p> <p>PG input may be taken into account when determining the objectives of a clinical program or development of a protocol; research sponsors must balance that input with scientific understanding as well as business and regulatory needs.</p>
Build the trust required for successful partnerships by being transparent and trustworthy, following through on commitments, and honoring confidentiality	<p>All stakeholders should be open, transparent, and honor commitments to the development program. Confidentiality Agreements (CAs) and Non-Disclosure Agreements (NDAs) allow sharing of sensitive information with PGs.</p> <p>Expectations about the role of PG consultation and input should be clarified at the start of the collaboration.</p>
Involve the expertise of multiple partners for a broader perspective to mitigate risk and enrich pipeline development	<p>PGs should be involved with multiple research sponsors to increase the pipeline of therapies in development.</p> <p>Sponsors should engage with more than one PG in a particular disease area to ensure that a representative patient perspective is reflected in the input obtained.</p>
Manage real or perceived conflicts of interest by establishing policies that require full disclosure, transparency, and accountability	<p>There are no FDA laws, regulations, or guidelines explicitly prohibiting early engagement with PGs. It is important to clarify which kinds of interactions with PGs are permissible and which ones might violate FDA regulations or fraud, abuse, and other regulations.</p> <p>The bottom line is that research sponsors can engage with PGs in planning and conducting clinical trials.</p> <p>Each type of PG engagement will have its own contractual rules and parameters to mitigate risk.</p>
<b>FOR RESEARCH SPONSORS (ACADEMIC AND INDUSTRY)</b>	
Integrate into your ongoing research and portfolio planning an assessment of PG expertise, assets, and value to your program	<p>The primary drivers for PG engagement are achievement of project milestones, corporate culture, and therapeutic area/vertical business unit interaction.</p> <p>Research sponsors need to develop and execute a comprehensive roadmap for substantive PG engagement.</p> <p>Research sponsors should consider identifying a single point of contact from the company or institution who has a sufficiently broad view of the internal dynamics of the organization.</p>
Match PG expertise and assets to the specific needs and phases of your R&D programs	<p>Research sponsors should recognize differences in the skills, experience, and capabilities of PGs. Currently there are no industry-wide tools used to select a PG.</p> <p>It is imperative to assess PG expertise, interests, organizational capacity, and relationships.</p>
Ensure that PGs are essential partners throughout the R&D process and not token voices	<p>Research sponsors should recognize that the most successful partnerships with PGs are those in which both entities are full partners at the outset, working toward the same goals from different perspectives.</p> <p>The patients’ voice as communicated by PGs is key to understanding the day-to-day effects of the condition and the acceptable benefit-risk tradeoff of treatment.</p>
For consistency, establish guiding principles and clear lines of communication to facilitate a fit-for-purpose process for collaborating with PGs	<p>Sponsors should establish and document best practices for engaging with PGs, including how to approach them, the legal requirements for working with them, and a template for master services agreements.</p> <p>Elements of the work practice may include a database of previous collaborations, required documents, and clear lines of communication.</p>

(continued)

**Table 2.** (continued)

Recommendation	Details and Actions
Measure the impact of PG engagement	Though no standard metrics exist for PG engagement across industry, it is recommended that research sponsors establish expectations up front on how to measure the effectiveness of the partnership. A regular assessment of satisfaction related to objectives, expectations, and success of strategies is recommended.
Establish ongoing relationships with PGs and communicate openly with them on a regular basis	Study teams should communicate with them regularly throughout in the development program. It is also important to maintain regular communication with PGs even when there is no study news.
<b>FOR PATIENT GROUPS</b>	
Proactively identify, engage, and bring the patients' voice to stakeholders relevant to your R&D interests	Recognize that there are limits to what any one PG can accomplish alone. To be successful in partnerships, you must build and sustain that trust to maintain your credibility among the constituents who rely on your group for dependable information.
Promote your value as an essential partner by maximizing and articulating your expertise and assets	PGs should know what they can offer research sponsors and have information and/or data that clearly articulates their value proposition. Through active, continuous engagement in the development program, PGs can demonstrate a unique value to their academic and industry partners.
Deliver your expertise and assets to sponsors throughout the entire R&D process	PGs should express the patient perspective as early as possible and throughout the development process—during basic and translational research, preclinical and clinical trial planning and implementation, the regulatory process, and the postmarket period. The degree to which the PG can provide grants to selected academic investigators and participate in a variety of forms of funding with industry partners and even well-vetted venture philanthropy partners will help position the PG as a key player in the field.
Select sponsors who have a product or program with significant promise for your constituents and who are committed to engaging in a meaningful way	PGs should ensure that they have a “finger on the pulse” of the preclinical landscape in order to maximize opportunities and ensure that they are viewed as valuable partners for sponsors. The PG should consider establishing a scientific review process in order to have an independent ability to evaluate the science being presented.
Manage real or perceived conflicts of interest by establishing policies that require full disclosure, transparency, and accountability	PGs should create written policies to clarify their position on accepting funds from industry sponsors, purchasing company stock, and other activities that might be perceived as generating a conflict. To manage internal and external conflicts of interest (COI) effectively, PGs should fully disclose relationships with industry sponsors. To help PGs navigate the complex web of decisions and opportunities, it is recommended that they prospectively develop a “Guiding Principles” document.

who are considering patient engagement collaborations need further guidance on operationalizing this new model. Our study results are consistent with those reported in Europe; specifically, uncertainty around the engagement process, the public's lack of knowledge and interest in research and development (and the need to be proactive in education efforts), and industry's lack of knowledge, interest, and receptivity to patient engagement.<sup>12,13</sup>

We gathered the in-depth opinions of 32 stakeholders with extensive experience collaborating successfully on clinical trials. The comprehensive analysis of our multistakeholder experts culminated in the development of actionable recommendations associated with effective patient group collaborations.

### Limitations of the Study

We acknowledge some limitations of this study. First, qualitative research techniques such as those used in the in-depth

interviews can provide useful insights into a target audience's perceptions and beliefs. However, data gathered qualitatively are, by definition, not as objective or clear-cut as quantitative data and may not necessarily be generalizable to a larger audience. Rather, these findings are intended to give a flavor of the thinking of the target audience.

In this study, the research provided the multistakeholder group with a sense of how patient groups and sponsors think about the elements necessary for successful collaborations around the clinical trials enterprise. We also acknowledge limitations in our sampling methodology. The experts interviewed were a nonrandom, purposive sample, defined as respondents who are similar to those of interest to the researchers. It is possible that those who agreed to take part in this research may be different from those who did not, which may also affect the generalizability of the findings. That said, this is a standard methodology for recruiting participants for qualitative research.

Despite these limitations, the interviews yielded strong, consistent themes. The participants interviewed from academia, industry, and patient groups are leaders in their field who have spent much of their professional careers exploring the intricacy of these partnerships. We believe that their insights will shed light on the fundamentals of successful collaborations between sponsors and patient groups around clinical trials.

### Future Work

One critical barrier to engagement of patient groups in clinical trials is skepticism on the part of some industry sponsors about the financial benefit of these collaborations. Industry sponsors interviewed said some of their colleagues would need to see research that shows the value of these collaborations before they would commit to such a partnership. The sponsors interviewed, however, were doubtful that studies involving metrics to demonstrate value would be funded by industry given more pressing research priorities.

The CTTI Patient Groups and Clinical Trials project team, however, has developed a framework using risk-adjusted financial models to assess the potential financial impact of patient engagement in the design and conduct of clinical development programs.<sup>14</sup> Additional work to test such models is still needed. We hope that this framework will be useful in harmonizing expectations between patient groups and research sponsors and thus lead to more efficient and effective collaborations to accelerate therapeutic development, as well as help to define the value proposition for such engagement activities. Additional project work is also underway to more broadly characterize the types of benefits and investments—beyond financial metrics—that sponsor organizations and patient groups associate with engagement. There may also be value in developing additional resources with which patient groups can better evaluate which sponsors to work with, especially as the value of engagement is more broadly recognized and demand for patient group involvement grows.

### Conclusion

The multistakeholder group suggested that one of the biggest barriers to sponsor engagement with patient groups is a lack of well-defined best practices and guidelines. Often there are no guidelines for engagement activities within a particular company, and charting new waters can be time-consuming and compete with other “must-do” study priorities. The themes generated from the qualitative research study augmented the literature review and survey to inform the development of actionable recommendations to enhance collaborations. The ability to establish meaningful partnerships relies on choosing the right partners and defining a mutual benefit. Engaging from the planning stage onward is the best way to build the relationship and ensure that therapeutic development is aligned with patients’ needs, which, among other positive outcomes, is critical to avoiding the wastes and inefficiencies identified in the past.<sup>15</sup> Patient-relevant, patient-centered outcomes—rather than “academic” endpoints—are more likely to be considered

clinically meaningful by global regulatory agencies including FDA, which requires clinical trials to provide clear evidence of a drug’s safety and efficacy, although the standards are becoming more inclusive of the patient experience. We hope our work leads to increased patient-focused drug development and tools that can drive value for both patient groups and research sponsors.

### Authors Note

Since the time this article was written, author Bray Patrick-Lake has changed affiliation. Patrick-Lake is now affiliated with Duke Clinical Research Institute.

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### Supplemental Material

The six online supplements are available at <http://journals.sagepub.com/doi/suppl/10.1177/2168479017720247>.

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