# Dialogue / Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs

September 22, 2015

NATIONAL HEALTH COUNCIL

Genetic Alliance

Prepared by Avalere Health

The National Health Council and Genetic Alliance greatly appreciate the educational grants from the following supporters that made this project possible:

- Biotechnology Industry Organization
- Merck & Co.
- Novartis
- Pfizer, Inc.
- Pharmaceutical Research and Manufacturers of America
- Sanofi U.S.

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## Executive Summary

The landscape for researching and developing new medical products continues to become more patient centric. Stakeholders are increasingly demanding that, in addition to demonstrating a product's safety and effectiveness, sponsors of new products more specifically address patient needs and demonstrate how their product will enhance patient outcomes. Furthermore, stakeholders all along the research-to-approval continuum are facing pressures to increase efficiencies and get drugs to patients faster. To keep up and meet this growing demand, drug developers, researchers, regulators, and patient organizations are exploring ways to more thoroughly incorporate the patient perspective into drug research, development, and approval. Although efforts to date among stakeholders have been fragmented and uncoordinated, a path forward is beginning to emerge.

This paper synthesizes the key outputs of a Dialogue Event co-hosted by the National Health Council and Genetic Alliance on March 2, 2015, which sought to convene a multi-stakeholder group of key thought leaders to help guide the transformation of the existing product development paradigm the patient voice an integral part of this process. The full-day event was held at the offices of the Food and Drug Administration and attended by 32 stakeholders, including government officials, patient organizations, industry representatives, and academic researchers.

Discussion at the Dialogue focused on arriving at a common understanding of what it entails to meaningfully engage patients, identifying key gaps in patient engagement methods, determining the critical barriers hindering advancement, and providing tactical next steps.

Participants' perspectives and experiences helped establish some baseline factors for informing a common understanding of what constitutes meaningful patient engagement. Rather than deduce a concise but vague universal definition, participants instead articulated a number of factors that should ideally be considered when designing and executing each engagement strategy. Similarly, through shared learnings, participants identified a need for a set of disease- and condition-agnostic methods standards to enable stakeholders to tailor and optimize engagement, for example, to reach patient populations that may be hard to access.

The Dialogue also allowed participants to discuss and agree on key fundamental barriers that hinder meaningful engagement. The identified barriers fall within three major categories related to culture, communication, and regulatory rules and processes. These

three barriers underlie the majority of challenges associated with achieving meaningful patient engagement in drug discovery, research, and approval. Throughout the Dialogue, participants proposed various actionable solutions that could help move stakeholders forward.

The findings articulated in this report are reflective of the key themes discussed at the Dialogue. They are intended to help provide patients and stakeholders with a viable path forward but should not be considered exhaustive or exclusive. Rather, the contents of this report represent an initial conversation among stakeholders to begin to close the considerable information and communication gap and build consensus around a vision for advancing patient engagement in drug research, development, and approval. A next step may be for stakeholders to prioritize and refine the solutions to make them practical, as needed. Patients and stakeholders will need to collaborate to ensure that future actions and strategies can lead to reduced fragmentation and enhanced coordination. As awareness around these challenges grows, the health care community is continuing to take steps in the right direction to address these gaps and advance meaningful patient engagement.

## Background

Growing pressure to improve the quality of health care and reduce costs has led health care stakeholders to seek solutions that address these seemingly competing demands. One well-accepted approach to achieving these goals is to promote better alignment of treatments with individual patient needs and goals.[1] Research has shown that patients who engage in their own health care tend to have better outcomes.[2] As a result, the concept of patient engagement is gaining momentum as a practical, ethical, and patient-focused way to improve health care outcomes.

Patient input into the early stages of research and development of therapies is increasingly recognized as being just as critical to improving health care as patient engagement after approval.[3] Historically, patients have not played a major role in product development beyond participation in clinical trials; however, this paradigm is changing. Many stakeholders, including researchers, drug developers, and the Food and Drug Administration (FDA), are starting to engage patients before products enter the market with the intent of ensuring not only that they are designed to meet patients' needs, but also that clinical trials, conducted to inform regulatory approval and eventual clinical use, are capturing information that is highly relevant and specific to the patient end users.

Despite efforts to broaden the scope of patient engagement in research and promote earlier patient involvement, a framework for doing so has not emerged. Even as stakeholders continue to craft, implement, and discuss new ideas and experiences, the need to develop consensus-driven methods to objectively evaluate and measure patient engagement persists. At this point, the critical elements required in order to take these steps have yet to be defined. Even though various stakeholders bring different perspectives, needs, experiences, prioritization approaches, and resourcing issues, all stakeholders must agree on a cohesive vision for the future of patient engagement and collaborate to navigate a path forward.

With this vision in mind, the National Health Council and Genetic Alliance, with research and analytic support from Avalere Health, convened a Dialogue Event as part of the broader effort to advance patient engagement in the research, development, and approval of medical products. By providing a forum for thought leaders to share their perspectives, experiences, and expertise, the National Health Council and Genetic Alliance hoped to establish a common vision that would help drive meaningful integration of the patient voice in the product development and approval processes.

## Introduction

On March 2, 2015, the National Health Council (NHC) and Genetic Alliance hosted an invitation-only Dialogue Event at the offices of the Food and Drug Administration (FDA) in Silver Spring, Maryland, including thought leaders involved in patient engagement policy, research, and implementation. Thirty-two participants representing the FDA, patient organizations, academia, and industry gathered to discuss key issues surrounding patient engagement in product research, development, and approval. By providing a forum for these thought leaders to share their perspectives, experiences, and expertise, the hosts hoped to establish a common vision to help drive meaningful integration of the patient voice in the product development and approval processes.

The Dialogue aimed to improve collective understanding of what constitutes meaningful patient engagement; advance sharing of tools, useful learnings, and best practices; and provide a path forward for stakeholders to continue to define, implement, and improve meaningful engagement of patients across the research-to-care continuum. The discussion explored four key issues:

- The absence of a common definition for what constitutes *meaningful patient engagement* in research, product development, and regulatory review
- The need for best practices to inform the choice of appropriate methods for engaging patients
- A fragmented understanding of the barriers to meaningful patient engagement
- The lack of stakeholder consensus and a cohesive set of strategies on how to overcome the barriers

The hosts intended the Dialogue to serve as a starting point to generate discussion, consensus, and more specific, targeted actions. This brief report incorporates key themes from the discussion and summarizes actionable next steps from the Dialogue.

## Building a Framework for Meaningful Patient Engagement

Patient engagement has emerged in recent years as a driving force behind efforts to improve the health care delivery system. Central to this movement is the focus on how therapies can be generated, developed, studied, and evaluated to ensure they better meet patient needs. Initiatives to test innovative patient engagement strategies have increased awareness and are contributing to the understanding and integration of the patient perspective throughout a therapy's life cycle. Despite these advancements, stakeholders broadly agree on the need to move beyond transient involvement of patients in research and develop ways to truly integrate their perspectives throughout a product's generation, development, and approval.

*"We need to develop…various instruments and agreed-upon structured ways of eliciting [patient perspectives]. We know we can't do it all. In fact, we regarded the patient-focused drug development meetings and other work we have done with some of the patient groups as kind of experimental in a way of opening the door to try to figure out what can we do."* – Dialogue Participant

### Defining Meaningful Engagement

While well intentioned, sometimes patient engagement suffers from inconsistency and other pitfalls that can render it suboptimal or even ineffective. Although great interest exists in gathering patient perspectives to inform drug discovery, research, and review, Dialogue attendees agreed that sporadic and transient engagement prevents broader advancement and innovation in this space. In order to move forward, they acknowledged that stakeholders must agree on what constitutes or does not constitute meaningful patient engagement.

**Patient Views Are Not Monolithic:** Developing a concise, workable definition of what constitutes meaningful engagement is challenging, given the variety of methods used, information sought, and ultimate goals of patient engagement. In addition, attendees noted that it is important to recognize that "the patient perspective" is not monolithic, even within a disease or condition. Consumers, patients, family/caregivers, and patient

advocacy organizations are all entities that can potentially provide valuable perspectives; however, those perspectives can vary widely. This concept is reflected in the definitions of "consumer," "patient," and "patient advocacy organization" that NHC uses, listed in **Figure 1** below. Participants also noted that methodological challenges such as limitations in the generalizability of a sampling of patient perspectives and accurately judging the appropriateness of specific patients or patient groups to specific research aims compound this complexity. Furthermore, each stakeholder's informational needs and internal processes will vary as a result of their unique culture, contexts, and circumstances. As a result, many ways exist to qualify whether an engagement is meaningful—reliable, valid, repeatable, or authentic—and each may shape the tone of the relationship differently. Thus, a framework and rubric for meaningful engagement would be helpful in this regard.

### Figure 1. NHC Definitions of the Consumer, Patient, and Patient Advocacy

- A **consumer** is a generally healthy individual who moves in and out of the health care system as his or her needs change over time.
- A **patient** is someone who is dependent on the health care system for the rest of his or her life after the diagnosis of a medical condition or disability. A patient relies on the health care system to feel better and to have a longer, healthier, and more robust life. An individual patient's views on health issues, such as the benefit and risk of new treatments, will vary depending on the severity of his or her condition and personal circumstances.
- A **patient advocacy organization** takes a holistic view of the conditions for the patients it represents and seeks universal support from stakeholders for its mission and programs. Many patient advocacy organizations work through the NHC to address systemic health care policy issues, creating a united voice for the broader patient community and their family caregivers.

## Meaningful Patient Engagement Can Be Characterized:

Although a singular, concise definition of "meaningful patient engagement" remains elusive, Dialogue participants believed that best practices can be identified to ensure that an engagement experience is informative, constructive, and mutually beneficial. Most importantly, patients and stakeholders should thoroughly vet their engagement strategies at the earliest possible stages. When designing a study or process, early planning will help ensure that engagements with patients are systematic. Similarly, patient groups

seeking to participate in research or regulatory processes should also be strategic when agreeing to partner or engage with other stakeholders. Both patient groups and stakeholders should also establish feedback systems to gather data throughout the engagement process to measure its impact. The Dialogue participants identified the following types of questions that both researchers and patients should consider when designing, implementing, and evaluating their engagement approaches:

- **What are we trying to achieve?** Determining specific goal(s) of the engagement
- **Who are we trying to engage?** Understanding who the target population or stakeholder group is and using that assessment to inform selection of who best to engage
- **When should we engage?** Identifying what point(s) along a given process are optimal for each engagement
- **How should we engage?** Selecting the methods most suitable for each engagement
- **What is the expected impact?** Developing or identifying metrics (outcomes or endpoints) that can be assessed to determine whether an engagement achieved the original goal(s)
- **What was the actual impact?** Implementing frequent checkpoints during and following the engagements to encourage communication, collect data on the outcomes/endpoints, and promptly address any issues

Dialogue attendees believed that addressing these questions will assist in tailoring the engagement plan to the goals, information needed, and outcomes desired to help ensure an optimal role for patients. Furthermore, such consideration will offer a greater likelihood that an engagement or engagement strategy will achieve maximum impact and likely be considered meaningful by both patients and stakeholders.

### Addressing Key Gaps in Methods, Resources, and Best Practices

**Engaging Patients:** Attendees noted that patient engagement for every disease area and patient population will have its own unique methodological challenges. As such, resources and tools on best practices for engaging patients across disease areas and patient populations are needed to help promote consistency in how patients are being and should be engaged.

For example, several patient organization representatives highlighted that patient populations from different disease areas will differ in terms of how well organized, well informed, and well represented they are by advocacy groups. Namely, rare disease populations may have limited resources or infrastructure for organized engagement. Given this variation, any best practices or standards for engaging patients should be designed to be readily applicable across many, if not most patient populations, regardless of a population's size, resources, and level of organization. Throughout the discussion, attendees identified several important considerations for organizations interested in engaging patients that could inform a broader set of best practices or standards. (**Figure 2**) Furthermore, participants believed that additional resources, such as a toolkit or a network of partners or collaborators with common interests and goals are needed to help support these efforts and build economies of scale.

### Figure 2. Examples of Best Practices for Engaging Patients Identified by Dialogue Participants

- Go to the patient or relevant patient organization first whenever possible
- Maximize patient participation/completion by consolidating the number of steps patients must take to engage (i.e., reduce number of forms to fill out; streamline meetings or encounters to minimize number of physical trips patients must make to site)
- Understand the patient's environment, situation, and state of mind at the point of engagement and how these factors can affect the outcomes and overall experience
- Integrate the data capture process seamlessly into patients' lives
- Build capacity for patients to engage and invest in technical assistance to support them throughout the engagement process
- Set clear expectations for the engagement
- Set boundaries and define what is or is not relevant or appropriate
- Treat patients the same as other participants (i.e., compensate patients for their time as others are compensated for time spent participating in an advisory capacity)*

** Best practices around compensation parity are still emerging, as stakeholders must be mindful of considerations such Federal Assistance Program enrollment thresholds and minimum wage restrictions.*[4]

**Collecting Patient Information:** While many validated methods for collecting patient information currently exist, stakeholders expressed a need for innovative and/or tailored tools and methods that will allow them to collect data that meet their objectives and are sufficiently robust to inform downstream decision-making (e.g., regarding internal business strategies; regulatory decision-making). Participants also believed that a lack of standards governing how to apply validated methods adds to the resources gap, with many calling for consensus-driven tools (and additional research from which to derive them) to help guide optimal selection and use of validated methods for collecting patient information.

*"If we had valid instruments or if we had reliable instruments, we would be able to use the information in a pretty acute sense in a regulatory process related to drugs that we have decided to develop and maybe make a risk/benefit decision."* – Dialogue Participant

Many Dialogue participants agreed that a collaborative effort to develop methodological standards for collecting patient input would be very valuable. Importantly, stakeholders felt that any standards should be adaptable to the rapid pace of scientific advances, reflect the heterogeneity of disease, and recognize the heterogeneity of the patient population.

*"We need valid ways of collecting information that get across the spectrum of both the personal differences that people have, their belief structures, as well as the severity of the disease and the different manifestations of any given disease."* – Dialogue Participant

### Understanding Fundamental Barriers to Meaningful Engagement

The concept of engaging patients in health care and integrating their perspectives and preferences at critical decision points has gained momentum in recent years, especially in the post-market and care delivery space.[5] Until recently, this shift has been slower

to take root in the drug development and approval processes, where patients have historically been very narrowly engaged, most commonly as clinical trial participants or other study subjects.

Stakeholders discussed current barriers to more broadly and more effectively engage patients in the pre-market and approval stages. Dialogue attendees identified that many of the most significant barriers perceived by stakeholders fall into three broad areas of regulatory/legal uncertainty, culture, and communication. (**Figure 3**)

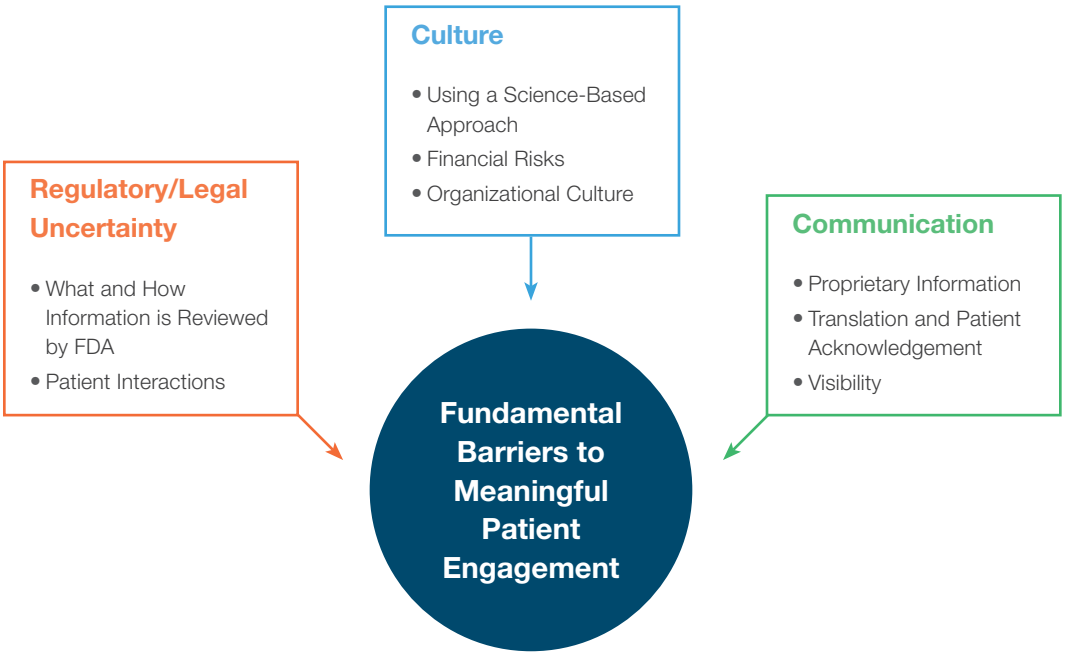


**Figure 3. Broad Areas of Fundamental Barriers**

### Regulatory/Legal Uncertainty

One of the most fundamental barriers to meaningful patient engagement is uncertainty regarding how FDA will evaluate sponsor-submitted patient information during the regulatory review process and the impact such data will have on product approval decisions. Furthermore, many stakeholders described perceived uncertainty regarding what constitutes appropriate engagements with patients and what actions may be seen as violations of certain regulations, including those governing pre-approval promotion. This uncertainty discourages stakeholders from publicly and proactively engaging patients in innovative and forward-thinking ways.

**What and How Patient Information Is Reviewed by FDA:** Researchers and drug developers, in particular, lack clarity about how FDA can or will incorporate patient information into regulatory review. Greater transparency and clarity from the agency will help alleviate some of this uncertainty. For example, with regard to FDA's benefit-risk framework, guidance on the types of information or endpoints of interest to FDA (e.g., informative for the benefit-risk determination), and explanations of how the agency plans to link patient preference data to the framework will provide stakeholders with a better sense of what data or patient-reported outcomes will be most helpful to collect to inform regulatory decision-making. This will in turn inform product developers' and researchers' broader patient engagement strategies.

**Patient Interactions:** Industry stakeholders have concerns that some research-based interactions with patients could be misinterpreted as promotional activities or a violation of other regulations. Without clear guard rails that outline what is considered appropriate and what is considered a violation, industry will not risk implementing or utilizing innovative engagement strategies. Patient groups would also benefit from increased transparency in that they could strategically mobilize and prepare their constituents for engagement opportunities. Patients and other stakeholders are eager for clearer guidance from FDA acknowledging that the agency is supportive of patient engagement efforts to inform drug development.

*"There's lack of clarity now that is dependent on each company's risk tolerance. It's not that [patient engagement can't] be done, but it's the risk tolerance variability and unpredictability now that I think is the challenge we need to address."* – Dialogue Participant

### Culture

The lack of regulatory certainty has impeded significant culture change because patient input has traditionally been difficult to gather and assess due to perceived risks. However, participants noted that the inability to promote cultural shifts and ways of thinking among stakeholders is the result of a multitude of factors, including the perception that gathering patient information does not follow science-based approaches, poses financial risks, is prone to misaligned or lack of incentives, and lacks accountability.

**Using a Science-Based Approach:** Approaches and methods for engaging patients and obtaining their perspectives are not widely regarded by researchers as scientifically robust or methodologically rigorous. Instead, patient perspectives are largely perceived as being anecdotal, emotional, and in many cases subjective as compared to clinical outcomes data obtained in clinical trials. Although validated methods and systematic ways to obtain patient perspectives do exist, the view still persists that due to poor methodological rigor patient information may detract from clinical outcomes data rather than enhance the data package.

*"We see this perception that this is a quasi-science. That people say, 'we do science-driven, data-driven drug development. We're meeting our p-values and we're going to stand behind our randomized controlled trials…we're not going to approach the FDA with anecdote or emotion.' And they don't realize that what we're talking about is scientifically rigorous. That it's [part of] additional scientific input [put into] the process to round out that totality of evidence of what [we can] consider [in regulatory decision-making]."* – Dialogue Participant

**Financial Risks:** The crux of the business case for engaging patients in the early stages of product development centers around helping to avoid significant patient concerns and potential misalignments between a therapy and patient needs after the product has been launched, such as the types of endpoints or outcomes studied compared to what patients actually care about. But implementing processes and building capacity to meaningfully engage patients will undoubtedly require significant investments from all stakeholders. For example, in the highly competitive marketplace, it is sometimes difficult for pharmaceutical companies to clearly determine the anticipated return on these early investments of conducting patient engagement in product research and development when the downstream uses and value of this type of information are unclear and/or uncertain.

Similarly, patient organizations that are developing programs and initiatives to organize, train, and deploy patients and patient representatives to participate in various engagement opportunities, such as the Parkinson's Disease Foundation's Learning Institute, would ideally also be able to determine the ultimate return on investment of these potentially costly endeavors to justify the expenditures. Although stakeholder participants voiced a clear need for these capacity-building programs, it is difficult to predict the level of impact given the limited and fragmented engagement opportunities for these trained patient advocates.

**Organizational Culture:** More broadly, organizational culture can serve as a barrier to meaningful patient engagement. All stakeholders, including patient groups, pharmaceutical companies, regulatory agencies, and research organizations must embrace a culture that values patient engagement, and commit to ensuring that their organization reflects such a culture throughout all aspects, from organizational structure to day-to-day operations. Some patient representative discussants noted that patient organizations often advocate for greater patient engagement as a policy issue, but that greater focus can also be placed on introspectively assessing how these organizations can more tangibly advance patient engagement, such as investing in capacity building and equipping patients with the tools they need to engage.

On the other hand, skepticism of the benefits of patient engagement due to uncertainties in the environment or simply internal resistance to changes due to organizational pressures can result in a continuation of "business as usual." For example, from the regulatory perspective, FDA reviewers who have not fully bought into the value of incorporating the patient perspective in regulatory decision-making may not give appropriate weight to patient perspectives in the review process. Cultural transformation supporting meaningful patient engagement must occur at all levels of the organization. The most rapid and effective way to influence the culture of an organization is from the top down as well as among influencers at many levels of the organization. For example, in pharmaceutical companies, those at the leadership level need to buy into the concept to elevate patient engagement so it can begin to be integrated broadly into the culture of the organization. This transformation may require effective sponsorship so that a consistent message can be mapped to every major functional department. Leadership communication is also key in ensuring cultural transformation. Top executives in an organization must communicate both internally and externally the importance of patient engagement to organizational decision-making. This lays the foundation for creating incentives to promote behavior change, establishing accountability, and engendering feelings of empowerment in all employees to make strategic and operational decisions incorporating patient perspectives.

*"[There is a natural systemic problem where] part of the company doesn't know what the other part is doing…or they have different cultures within the same company or the person leaves…that's an organizational architecture issue that's really hard to address."*

*"When I talk with CEOs I say, 'you need to inculcate a culture that then pervades the organization. And then you also need major communication strategies to be shared.'"* – Dialogue Participant

## Communication

Information on patient engagement activities in the pre-market space has only just begun to emerge in the public domain. This is partly because until recently such activities were limited, and likely a reflection of organizations acting independently without a clearinghouse for information on patient engagement activities.

**Proprietary Information:** Due to the highly competitive marketplace, attendees explained that organizations treat many of their activities as proprietary, including engagement with patients. As a result, communication and information sharing among stakeholders has been limited. In order to optimize advancement in integrating patient engagement into drug development and approval, Dialogue participants recommended that industry work toward closing the knowledge gap by distinguishing information that is truly proprietary from what can and should be shared, for example, best practices and lessons learned. Until there is a path forward for regulatory use of these tools, methods, and data, this information will likely continue to be viewed as proprietary and used primarily for internal company decision-making.

*"Here's where individual companies, I think, have a strong motivation to make a tool they've developed and invested in part of the intellectual property that they have. So how do we provide a way for a tool to become available, an open source, if you will, or just tools that can be used again, and not have to reinvent the wheel, and not have small innovator companies who don't have the resources to do tool development be able to use tools that are available?"*
– Dialogue Participant

**Translation and Patient Acknowledgement:** Discussants noted that a lack of communication exists not only between organizations but also toward patients and the general public. Today, stakeholders rarely engage patients in a continuous manner and engagement usually ends after the data gathering portions of a clinical trial or study. Consequently, these patients often do not have a clear understanding of how their input and data were used. Additionally, the outcomes of the study are rarely communicated and disseminated directly back to the patients who participated with the research organization. The lack of such a "feedback system" to keep patients engaged and informed is perpetuating a sense that patient input is only being collected to "check

a box" and not truly intended to be used. Translating information in a way that is comprehensible to the target population, whether it is patients, caregivers, or patient advocates, and also acknowledging and showing the impact of their contributions can enhance and potentially improve the overall quality of these engagements.

*"Right now the complaints that we get [from patients] all the time is they're only contacted when you want to put them out there to tell how good the drug is and how well they're doing. But after a trial closes there's no follow-up with them. So I think more transparency probably would do very well."*
– Dialogue Participant

**Visibility:** Dialogue participants agreed that patient engagement in drug development and regulatory approval suffers from a large knowledge and information gap. No organized, centralized warehouse currently exists for information related to patient engagement, such as methods, best practices, and success stories. Furthermore, very limited published information about the past and ongoing efforts in this space is available in the public domain. Increased visibility of patient engagement activities or research conducted through avenues such as campaigns and published literature will bring more awareness to this issue and help garner support from a broad audience. Communication on this topic must become a priority so that learnings and best practices will be disseminated beyond each organization and even beyond the health care sector.

*"There is a need for some more information to come out in the form of a publication, an article or [something]…I think it also helps lend some legitimacy because, if you do a PubMed search right now, not a whole lot comes up on patient-focused drug development…I think having some things out there makes it easier for someone to go to their company, to their higher-ups, or to other places and say, look, this is a real thing, FDA is really doing this, it actually really does exist."* – Dialogue Participant

## Steps for Advancing Meaningful Patient Engagement

To begin to address the barriers to meaningful patient engagement, solutions that target specific issues must be identified and prioritized. All stakeholders have an important role to play in moving patient engagement forward. Some of these steps have more direct relevance or can be more readily attributed to a specific group, such as the patient community, academia, industry, or regulators. As a result, some groups may have a greater opportunity to operationalize specific steps by leveraging existing resources. These are not intended to be exclusionary, but rather aim to provide more specific guidance on potential next steps to specific stakeholder groups.

The following tables reflect this concept and contain actionable steps proposed by participants throughout the Dialogue. The "X"'s denote those groups that may be ideally suited to lead an action, however, the absence of an "X" is not intended to indicate that a stakeholder cannot take that action on that particular step. Many of the solutions discussed were identified by stakeholders as a small action with potentially very large impact. If stakeholders take these actions, they could pave the way for longer-term strategies.

| Creating Regulatory Guardrails | Patient Community (P) | Academia (A) | Industry (I) | Regulatory Agencies (R) |
|---|---|---|---|---|
| Prioritize development of one or a series of guidances to set regulatory parameters and/or clarify agency thinking around topics such as:<br>• What type(s) of patient information will be considered by the FDA<br>• Patient data endpoint selection<br>• Appropriate industry interactions with patients<br>• Incorporation of patient information on product labels<br>• Linking patient information to benefit/risk assessments | X | | | X |
| Align stakeholder advocacy strategies to maximize impact | X | X | X | |
| Formalize regulatory asks (FDA action) for negotiation in the sixth Prescription Drug User Fee Act (PDUFA VI), as appropriate | X | X | X | X |
| Generate publications or opinion pieces in high-impact clinical journals or other credible venues to heighten visibility of patient engagement, send a "signal" to broader clinical and scientific community, and enhance legitimacy of patient engagement efforts | X | X | X | X |
| Create more opportunities to collect public feedback and input through public avenues such as requests for information (RFIs), town hall meetings with iterative Q&A sessions, and comment opportunities | | | | X |

| Creating Regulatory Guardrails | P (Patient Community) | A (Academia) | I (Industry) | R (Regulatory Agencies) |
|---|---|---|---|---|
| Enhance FDA division alignment on the use of tools for evaluating patient information at the reviewer level | | | | X |
| Increase transparency on how information is used and incorporated in each engagement/into each decision-making step | | | X | X |

| Promoting a Culture Shift | P (Patient Community) | A (Academia) | I (Industry) | R (Regulatory Agencies) |
|---|---|---|---|---|
| Generate buy-in and sponsorship for patient engagement at the executive and senior leadership levels | X | | X | |
| Create accountability at all levels within an organization for collecting, understanding, and integrating patient perspectives by establishing expectations and measuring the impact | | X | X | X |
| Organize internal infrastructure and staffing to be coordinated around patient engagement activities and to prevent information silos | X | | X | |
| Train and educate researchers on patient engagement | X | X | | |
| Develop methods standards that can be applied across multiple disease areas | X | X | X | X |
| Catalog validated methods for gathering patient information | X | X | X | X |
| Establish processes or models to systematically engage patients at any point in the research-to-approval continuum | X | X | X | X |
| Develop and implement tools and resources that complement methods for patient engagement and facilitate implementation | X | X | X | |
| Develop and test metrics to evaluate patient engagement | X | X | X | |
| Develop a platform, repository, or system for sharing best practices, research, examples of impact (e.g., public-private partnership or “center of excellence”) | X | X | X | |
| Establish a public-private partnership to serve as a central clearinghouse for patient-centered studies in the pre-market space, build capacity and infrastructure, and advance scientific methodologies for patient engagement | X | X | X | X |
| Direct funds from public and private research funders through patient groups to provide patient groups with the opportunity to solicit, evaluate, prioritize, and even directly fund patient-centered studies or projects | X | X | | |

| Facilitating Open Communication | P<br>Patient Community | A<br>Academia | I<br>Industry | R<br>Regulatory Agencies |
|---|---|---|---|---|
| Translate all information provided to and communications with patients to an appropriate level such that it is comprehensible | X | X | X | X |
| Create a feedback system to inform patients about how their contributions impacted decision-making and outcomes | | X | X | X |
| Manage patient expectations at the outset of each engagement through clear and transparent communications | | X | X | X |
| Consistently document the impact of patient perspective studies or other outcomes and publicize them | X | X | X | X |
| Proactively conduct media and press activity to publicize successes or give periodic updates on new developments | X | X | X | |
| Publish studies and editorials in high-impact clinical and scientific journals to raise awareness among the research community and enhance credibility | X | X | X | X |
| Coordinate publication strategies with other stakeholders for relevant pieces to be published at the same time or sequentially through various outlets | X | X | X | X |
| Publish or make publicly available experiences, advice, best practices, lessons learned, and other resources not considered proprietary or intellectual property | X | X | X | |
| Create partnerships and collaborations among private companies that encourage and incentivize information sharing and building economies of scale to accomplish a shared goal | | | X | |
| Utilize the open source production model whenever possible to promote continuous refinement, improvement, and open access to an engagement “blueprint” or “design” | | X | X | |

## Conclusions

Stakeholders across the health care industry are striving to make health care more relevant and responsive to patient needs and preferences. Considerable progress has been made in recent years to bring awareness to the importance of integrating patient perspectives earlier into the process of developing and making new therapies available to patients. However, there remains a growing need to understand and address the fundamental barriers that are preventing a larger paradigm shift that would further integrate patients as a central part of this process. Driving consensus on the vision for patient engagement in this space is critical for drawing alignment on the direction in which all stakeholders can collectively move.

This brief report summarizes key discussion points and outputs of the Dialogue Event, where 32 expert participants from across the health community shared their experiences and aspirations for the future of meaningful patient engagement in drug research, development, and regulatory review. Many participants offered potential actions to help address the challenges discussed. It will be important to continue to refine, prioritize, and act on these recommendations so that the benefits of meaningful patient engagement can be realized as fully as possible.

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March 2, 2015

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