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Patient Centricity and Pharmaceutical Companies: Is It Feasible?

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Abstract

Various health care bodies (regulatory, health technology assessment, academia, health care providers, scientific journals) request patient input in their decision-making processes. This represents a shift from disease-centered to patient-centered approaches to health care. What does this "patient centricity" mean for the pharmaceutical industry? A panel of senior pharmaceutical industry representatives discussed the following key issues: why the pharmaceutical industry needs to be part of the patient-centric movement; how the industry can become patient-centric; and what a patient-centric company actually does. We summarize the panel's point of view on these key questions. The industry's role has been to develop the science and medicines for prevention or treatment of disease. In response to changes in the current health care environment, the industry should focus its efforts on initiatives that will improve impact and value for patients and carers. True patient centricity requires a change in the industry's cultural mindset, an increase in public trust, clearer roles and responsibilities within pharmaceutical organizations, openness to learn from others, and a framework to measure success. There are examples of industry engagement with patients throughout the drug discovery and development process. Patient-reported outcomes are becoming increasingly important endpoints in trials; they capture information of relevance to patients, identify preferences, and better inform treatment decision making. Understanding the patient experience can improve disease management at critical points in the disease course. The future of patient centricity lies in coordinated efforts by and alignment of multiple health care stakeholders, which can only be achieved through collaborations and consortia, with the industry playing a key role.

Keywords

patient centricity, pharmaceutical industry, patient-centered, patient-reported outcomes, drug discovery

Introduction: Problem Statement

Today, patients are more knowledgeable than ever before. The technological and scientific advances of recent years have enabled easy access to information; patients are online and can connect with peers, exchange experiences and knowledge in real time, take part in advocacy and patient support groups, and contribute to real-time data generation.[1,2] As a result, patients feel empowered and expect their voice to be heard.[3]

Patients are increasingly being recognized as important stakeholders in the health care dialogue: regulatory agencies (Food and Drug Administration [FDA] and European Medicines Agency [EMA]), academia, and health care providers are requesting patient input into their decision making.[4-7] In 2014, the *British Medical Journal* launched a "Partnering With Patients" initiative that requires clinical trials publications to include the patient perspective by having patients as authors, peer-reviewers, guest editors, and contributors to the journal.[8] Moreover, decisions about medicine reimbursement are becoming increasingly dependent on the demonstrated value of medicines to patients. Therefore, payers are seeking evidence in the form of patient-reported outcomes (PROs) and are involving patients in health-technology assessments (HTAs).[9,10]

This shift in the social landscape is accompanied by increased pressure on health care costs,[11] with tension arising between the desire to act in the best interests of individual patients (individualized care) and taking into account the larger economic considerations. This disconnect is limited not only to the pharmaceutical industry but also exists for health care providers and health care payers.

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Patient Centricity—An Appropriate Response to Shifts in the Social and Economic Environment?

Patient centricity can be simply defined as integrated measures for listening to and partnering with patients, and placing patient well-being at the core of all initiatives. In essence, it represents a holistic approach to disease management. Although many agree that patient centricity is a concept to be embraced, there is still little published guidance and, in practice, significant variability[12] exists in its implementation by the pharmaceutical industry. Why is patient centricity coming to the fore today? Is it a reaction to the changing environment of a world in which patients are ever more connected and informed, or is it the realization of an ethical responsibility across the health care system? Is it realistic for a company operating in a commercial environment to place patients at the heart of everything that it does?

The Renaissance Europe Patient Centricity meeting was held on Wednesday, March 16, 2016, in London. An expert faculty (representing a range of committed pharmaceutical companies and including patient advocacy, regulatory, and health care practitioner perspectives) and a dedicated audience of senior industry representatives debated the role of the pharmaceutical industry in the patient-centric movement. The meeting addressed the following key questions:

1. Why does the pharmaceutical industry need to be part of the patient-centric movement?
2. How can the pharmaceutical industry become patient-centric in a real way in a modern business environment?
3. What does a patient-centric company actually do?

Why Does the Pharmaceutical Industry Need to Be Part of the Patient-Centric Movement?

Transformation into a patient-centric organization requires an answer to this key question: why are we doing this?[13] As in any organization, establishing the purpose and vision first will determine the subsequent plan and actions (how and what) and create alignment among employees toward a common cause.[13] Historically, the pharmaceutical industry's role has been to develop the science and medicines for prevention or treatment of disease. Patient centricity involves the patient in this scientific process. In essence, this is an innovative mindset whereby the industry is challenged to engage and collaborate with patients when deciding the best course of action. Therefore, patient-centric initiatives should start with a simple question: how can we make a difference for patients?

Is patient centricity feasible from a commercial standpoint? It is accepted that pharmaceutical companies need to make profit. It is how they get and use that profit that is important, and this must be aligned with any assertion of patient centricity. If the vision is directed at providing value to patients and carers, companies must be seen to be breaking out of the cycle of "recover costs of R&D—make profit—invest in new drugs—make more profit." This commitment, in turn, will help to generate credibility with external stakeholders (patients, regulatory, media, etc.).

How Can the Pharmaceutical Industry Become Patient-Centric in a Modern Business Environment?

Shifting the cultural mindset

Patient centricity demands a shift in cultural mindset within the pharmaceutical industry. At the highest level, it involves listening to and partnering with the patient, and understanding the patient perspective, rather than simply inserting patient views into the established process. Only then can patient-centered outcomes truly be the core of the strategy.

This change in mindset requires leadership from the top and drives the need to redefine core strategy, organizational structure and processes, and capabilities to focus on transparency and value for the patient. A shift from a disease-centered to a patient-centered strategy, and from a product-led to a patient-led development process, requires a change in outlook. A corresponding change in organizational model is also needed, with medical affairs, market access, regulatory, pharmacovigilance, and commercial teams at global and regional levels all collaborating with a common goal—to capture patient perspectives and insights and to respond to their needs. If patient centricity is to be the responsibility of everyone in a pharmaceutical company, key development and performance indicators (and possibly remuneration structures) should be adapted to reflect this change in mindset. These performance indicators could include the proportion of product strategies that are based on true patient input, the number of patients involved, patient feedback based on ratings of patients' involvement (eg, how engaged/involved/satisfied they feel), number of study protocol revisions implemented as a consequence of patient input,[14] the relevance of new product profiles to the self-identified needs of the patients, and improving the information available to patients and prescribing physicians on the risks and benefits of a treatment, so that better decisions for the patient can be made.

Building trust

One thing is clear: if the call is patient centricity, then the pharmaceutical industry has to be earnest about making it happen, not just using it as a means to seem more patient oriented. Indeed, the starting point of placing the patient at the heart of health care initiatives is trust. The industry has started to address questions about past behavior and public perception, and this positive intent, direction, and action is a vital component of rebuilding the industry's reputation and forming the foundation for new trusted partnerships.

Trust between the pharmaceutical industry and the public can be built through clear communication about medicines' risk-benefit profiles and a transparent drug development process. In this context, the relationships between patients, industry, and health care practitioners are essential to building

Table 1. Examples of Initiatives at GSK Aimed at Building Trust With Patients and Physicians.

Engagement with patients can only be possible if there is credibility:
• **Change in marketing practices**: In 2011, GSK eliminated prescription sale targets in the US and introduced a new incentive model for sales and marketing practices based on value and feedback from prescribers; external speakers/ convention travel support was discontinued (2016).
• **Clinical transparency**: Since 2013 GSK has committed to promote transparency of clinical research and is a leading example in the pharmaceutical industry—it was the first company to grant access to anonymized patient data; the *All trials* campaign (2013) commits to publishing all trial data; the GSK patient-level data access site has become a multisponsored portal (2014).

Abbreviation: GSK, GlaxoSmithKline.

credibility: "trust your doctor, trust the medicine, and trust the data."

One way for pharmaceutical companies to build trust is to collaborate on the development of new patient-centered endpoints. In the context of the Critical Path Institute Patient-Reported Outcome Consortium,[15] 10 diseased-focused working groups composed of representatives of pharmaceutical companies and government/regulatory agencies (FDA, EMA, and the National Institutes of Health [NIH]) are currently collaborating to share data and develop standardized PRO endpoint measures. This precompetitive initiative aims to standardize PRO endpoint measures in order to facilitate comparisons and efficiencies in regulatory and payer decision making (also discussed later). A unified industry approach to collaboration with the appropriate parties is the key to the adoption of patient centricity across the industry, and to improving credibility. In addition, many pharmaceutical companies have transparency initiatives; for example, GlaxoSmithKline has several transparency initiatives aimed at building trust (Table 1).[16,17]

Learning from others and collaboration

Creating a patient-centric culture requires an understanding of its implications for information flow, decision-making processes, and institutional habits. To achieve this, the pharmaceutical industry could gain insights from organizations that already have experience with patient interaction. For example, the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) has a patient-centered special interest working group[18]; the Medical Device Innovation Consortium (MDIC) is a partnership between public and private stakeholders in the medical device industry[19]; the European Patients' Academy on Therapeutic Innovation (EUPATI) is a collaboration between 33 different patient organizations, universities, and pharmaceutical companies across Europe; the International Federation of Pharmaceutical Manufacturers & Associations (IFPMA)[20] brings together regional/national patient associations and research-based biopharmaceutical companies. Furthermore, there is an ongoing collaboration between the FDA and EMA on patient involvement, that is, the FDA/EMA Patient Engagement Cluster, which enables the agencies to share best practices for patient selection and training throughout drug regulatory life cycles. The Patient Engagement Cluster focuses on cancer treatment and orphan drugs, among other topics.[21,22]

Multiple groups, including regulatory bodies (EMA), the National Institute for Health and Care Excellence (NICE),[23] different HTA groups,[24,25] and others[26,27] have already established frameworks for patient engagement. For instance, there is a Consensus Framework for Ethical Collaboration[28] that helps guide interactions between patients, pharmaceutical companies, and health care professionals. The Interest Group on Patient and Citizen Involvement of Health Technology Assessment international (HTAi) has developed a set of values and quality standards for involving patients in HTA.[24] Furthermore, the MDIC has also established a framework for introducing patient preference information into regulatory risk-benefit assessments, specifically on medical devices.[19] It is taking in the lessons learned by these collaborations and consortia that will help pharmaceutical companies reduce barriers to patient engagement.[29]

What are the challenges?

Several challenges exist that need to be overcome before the pharmaceutical industry can adopt the patient centricity concept.[7,10,30] Challenges to address include skepticism about commercial success; lack of process standardization as it relates to involving lay people in a highly regulated environment; lack of sufficient sharing of practices and lessons learned across the industry; lack of understanding of, and respect for, qualitative research methods that often form the foundation of patient centricity research; lack of alignment and measurements of collective progress; internal habits of going back to established information sources ("let's just ask the clinicians"); compliance frameworks and the perception that direct engagement of industry with patients is inappropriate or unauthorized; and last but not the least, the conflict of responsibilities: the perception that industry should not be the party that engages with patients.

Most of these challenges concern the pharmaceutical industry as a whole and can only be solved in a collaborative fashion, as discussed above. Some solutions are beginning to emerge, highlighting the need to share and learn from each other's experiences. For instance, a compliance framework has already been developed and is available to guide that direct engagement with patients by industry.[31]

What Does a Patient-Centric Company Actually Do?

Pharmaceutical companies are at different stages along the patient-centric journey. The following section aims to highlight patient-centric initiatives taken by pharmaceutical companies that have adopted patient centricity as a strategy.

Table 2. Example of Patient-Led Value Strategy at the biopharmaceutical company UCB.

Replacement of " brand strategy" with " patient strategy" is not enough. This process should start with the patient (rather than the commercial product) foremost in mind. Long-term strategy should be based on understanding of the patient journey and critical points (*breakpoints*) in the course of the disease. Typical breakpoints for potential involvement include diagnosis, new symptom development, relapse, start of a new medication, and emergent adverse events.

As an example, UCB has a medication for Parkinson disease:

- Patients with Parkinson disease need to take their medication at the same time each day as their motor function can be very sensitive to changes in this rhythm
- If these patients are hospitalized for a different reason, the hospital rarely has the medication for control of motor symptoms readily available; intake is delayed, the patient's condition deteriorates, and they spend 2-3 days longer in the hospital than if appropriate medication were readily available.
- Understanding this scenario helped UCB to implement relevant solutions in collaboration with treating hospitals, ensuring these medications were available; this in turn led to a decrease in hospitalization duration for patients with Parkinson disease.
- Thus, insights into breakpoints in the disease can help to provide better outcomes for patients.

Table 3. AstraZeneca's Approach to Patient Centricity.

- AstraZeneca has a focus group to internally facilitate patient centricity (PaCe): according to AstraZeneca ("put the patients first"), patient centricity is the open and sustained engagement of the patient to respectfully and compassionately achieve the best experience and outcome for that person and their family; in this conceptual model, patient insights drive innovative solutions that in turn create positive effects for the patient.
- The PaCe approach engages with patients through several research and therapeutic areas collaboration with PatientsLikeMe; more than 30,000 patients are now connected with AstraZeneca research projects.
- AstraZeneca is also setting up disease-specific networks and patient/carer networks (eg, Patient Expert Network) to advise the development/brand teams on the co-creation of patient solutions.

Moving from a brand focus to a patient-value focus

A shift in the industry environment and patient empowerment is dictating a change in how health care solutions are designed. We recognize that patient expectations and needs can differ considerably from the aims and objectives of health care providers, at both the policy and delivery levels. In fact, to be accepted by patients, the patient experience should be the key driver for the development of solutions (eg, medicines, devices, information, support programs, apps). A better understanding of patient experience at critical points (*breakpoints*) in the course of the disease will help to determine breakpoints that would benefit from pharmaceutical industry input (Table 2). Roundtable discussions involving patients, or "patient value tables," can be used to validate patient needs and understand the solutions patients seek, or assess the effects of solutions already in place. UCB has involved patients in all internal meetings since 2002 and uses patient value discussions between patients, health care providers, regulators, and payers.

AstraZeneca engages with patients through research and therapeutic area collaborations using online patient platforms and networks (Table 3). Essentially, capturing information about the patient journey and gaining an understanding of patient needs will help to develop a true patient-led strategy within the industry and design solutions that treat the patient and not just the disease.

Involving patients in research and development

Another approach to patient centricity is promoting patient involvement throughout the drug discovery and development process (Figure 1).[5,32,33] Patient advisory groups already provide input on unmet needs, protocol design, trial conduct, data interpretation and dissemination, risk-benefit discussions, risk management plans (risk evaluation and mitigation strategies), and the interpretation of value of treatment relevant to conversations with payers. However, the degree of input from patients, the frameworks used to gain input, and the implications of their input are not consistent across the industry. Patient engagement in research and development is expected to provide benefits at various stages of the drug development process, which include

- identification of unmet patient needs, enabling better-informed decision-making about target choices and the required asset profiles for go/no go decisions;
- study designs and endpoints that are more relevant to patients, which in turn improves recruitment and retention and provides regulatory and reimbursement decision makers with clear patient-relevant evidence;
- shorter trial cycle time, so medicines can move more quickly to regulatory submission;
- a greater range of products that are of greater value to more patients;
- improved patient adherence to drug regimens, leading to improved outcomes for patients; and
- a growing body of evidence of patient preferences and unmet needs that will help focus future research.

There is emerging evidence supporting the value associated with patient engagement in the drug development and discovery process.[33,34] Considerable progress has already been made in this direction, led by the Clinical Trials Transformation Initiative[35] and a number of concrete examples of engagement between pharmaceutical companies and patients.[36,37] However, the degree of input from patients, the frameworks used to gain input, and the implications of their input are not consistent across the industry.[12] Therefore, the industry as a whole should aim to standardize and increase patient involvement in research and development as a priority if its goal is patient centricity.

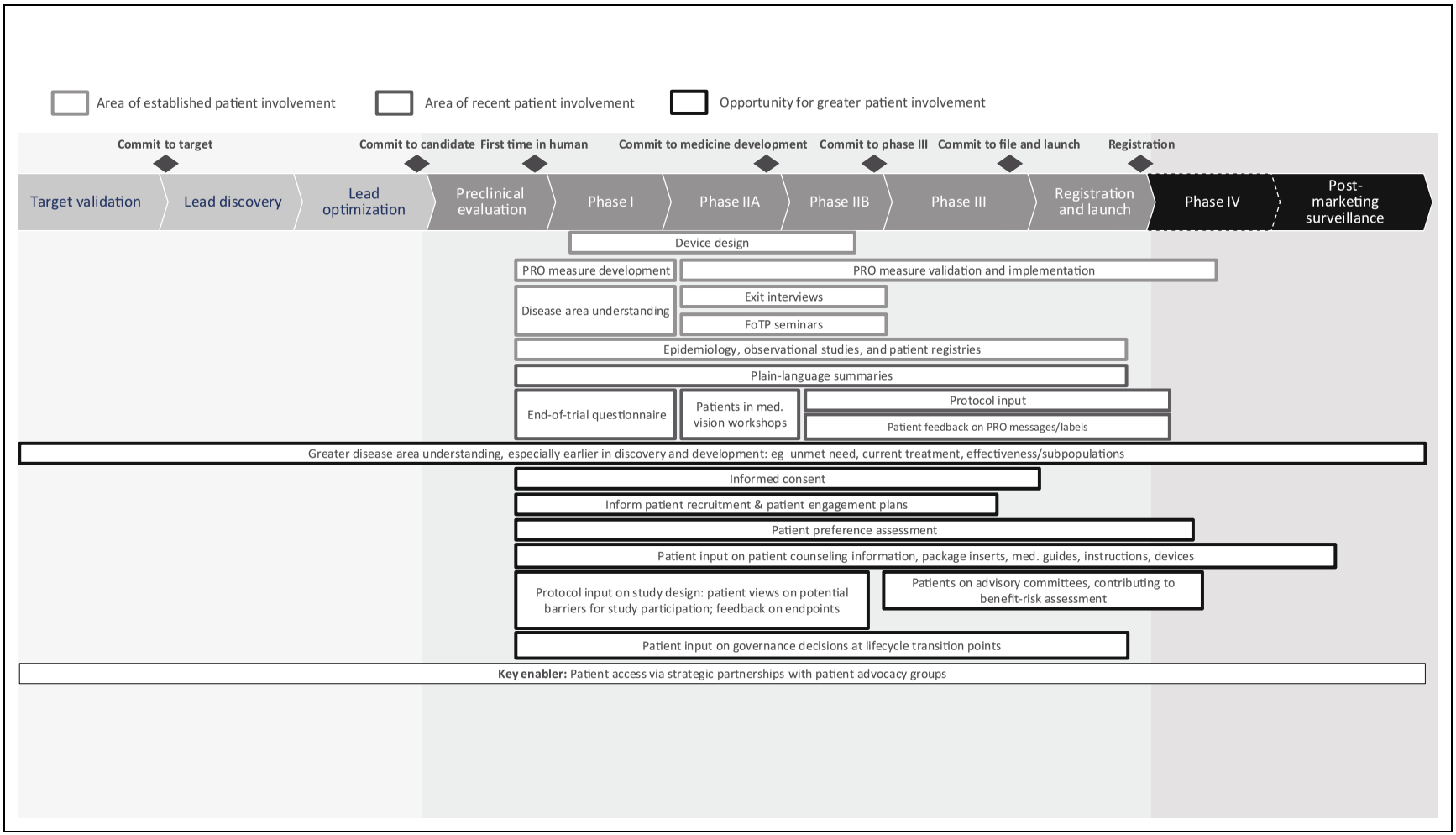

Figure 1. Patient involvement through drug discovery and development. FoTP, focus on the patient; PROs, patient-reported outcomes.

Standardizing PROs

The industry has a responsibility to provide information about medicines that will be relevant to patients. But what information are patients looking for? A patient would want to know, What can I expect in terms of symptoms/side-effects? How will I feel? and What will I be able to do?

Measuring treatment benefit versus risk from patients' perspectives is important because it highlights the value of treatment. The FDA defines PRO measures as "any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else."[38] As only patients can report how they feel and function, this generates a need to define endpoints that are important and relevant to patients.[39,40] PRO assessments are increasingly becoming the primary trusted evidence for all stakeholders, including payers, regulators, and patients.

The use of PRO measures in the industry began many years ago, as health-related quality-of-life instruments included in clinical trials. Although many expert centers develop and validate PRO tools, there remains a gap between ensuring that adequate patient input has been sought at the beginning of the development of the tools (eg, truly reflecting the patients' experience of a disease) and the experts' view of the patient experience. For example, a recent report showed low and variable rates of patient involvement in the development of PRO measures, with only 6.7% of patients involved in all stages (item development, item testing, item selection).[41]

Patient-centered drug development is expected to change the way the pharmaceutical industry operates. PRO tools and measures are ideally developed in a precompetitive collaborative way, to set standards across all medicines.[15,42,43] A standardized approach will provide easier, more straightforward assessment by all key stakeholders, including regulators (FDA[38] and EMA[44]), patients, prescribers, payers, and everyone who reviews the data.

Expert Patients

Regulators (FDA, EMA), HTA bodies, NICE,[45] and consortia[46] all seek to involve *educated* patients as advisors (expert patients) in drug development, evaluation, and regulation.[47] Expert patients have in-depth understanding of the disease context, and in most cases they function on patient advisory panels to provide insights relevant to the patient journey.

The pharmaceutical industry needs to understand the conflicts of interest as perceived by other health care players, such as regulatory bodies or HTA groups, in order to address them when involving expert patients or know when to step away. Currently, there are very few expert patients across a small number of well-represented diseases. A need exists to expand the pool of expert patients across a wide range of diseases.

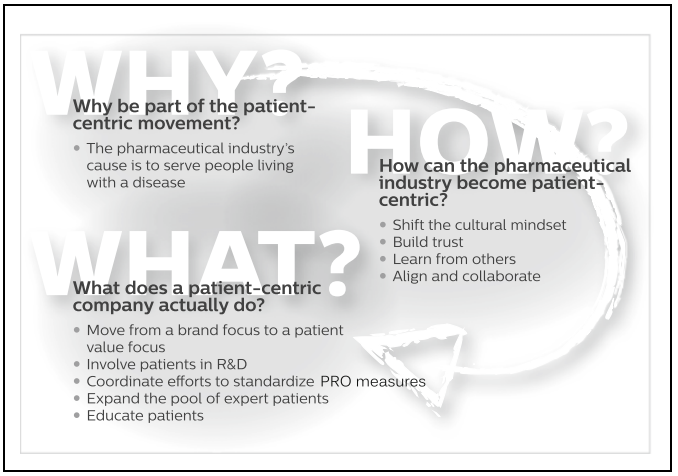

Figure 2. How patient centricity can be achieved in the pharmaceutical industry (conceptual framework). PROs, patient-reported outcomes.

Concluding Remarks

Bringing patient centricity to life—role of the pharmaceutical industry

Bringing patient centricity to life requires a vision shared by all stakeholders. We believe that the key steps the industry needs to take in order to make this happen are (1) change the mindset—make patient centricity a company-wide and industry-wide vision; (2) collaborate—involve patients and other health care stakeholders to develop patient-centric solutions; (3) share the learning—challenge the status quo, record the experience, learn from it, and share it in order that the industry may get better at being truly patient-centric. Therefore, we encourage colleagues to publish case studies of what is working well in order to keep the discussion alive and advance our collective learning.

In summary, a transformation into a patient-centric organization requires defining the answers to the following key questions: Why are we doing this? How should we do it? and What are the results we aim to achieve? (Figure 2). Historically, the pharmaceutical industry's role has been to develop the science and medicines for prevention or treatment of disease. Patient centricity is an innovative mindset whereby we are challenged to engage and collaborate with patients to get critical input for determining the best strategy to improve patient outcomes. This change from a brand focus to a patient-value focus requires leadership from the top and drives the need to redefine strategy, organizational structure and processes, and capabilities to focus on transparency, credibility, and value for the patient.

The industry is a major stakeholder in making patient centricity a reality, but cannot bring this about alone, or set the agenda. Equally, however, neither can medical professionals, government, or policy makers. How and with whom to partner is the key challenge. The future of patient centricity lies in coordinated efforts and alignment of multiple health care

stakeholders. Patient centricity can be achieved only through collaborations and consortia that include all health care stakeholders, in which the pharmaceutical industry has a key role.

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