21 Adelphi Renaissance Forums

Transforming Value, Transforming Trust

Building patient trust and belief in the value of medicines

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Mission

To improve outcomes for all stakeholders, the pharmaceutical industry seeks to deliver outcomes that patients recognize and greatly value. However, health care is exposed to enormous societal pressures with a growing mistrust of traditional systems, including providers. The healthcare environment is also rapidly transforming as it integrates scientific advances with evolving technology.

How can the pharmaceutical industry deliver value and earn the trust of patients?

In this white paper, we summarize the recommendations for providing value and earning patient trust from the unique perspectives of a panel of senior healthcare representatives who met in New Jersey in June 2023.

Executive Summary

Changes and challenges in health care

Value and trust

- Discussions about providing care which patients perceive as valuable and about earning and keeping patient trust have become increasingly pressing across the entire healthcare sector.
- Patient input is critical for determining and communicating value.
- Poor communication of the value of care contributes to the mistrust of the healthcare system.
- Recognizing that underserved populations are still not adequately served by the healthcare system, including the pharmaceutical industry, is vital to building trust in the value of new medicines.
- Implementing a patient-centered approach to medical product development that demonstrates value to and earns the trust of patients requires a change of mindset among all stakeholders.

Technology

- Digital health technology provides new opportunities but may also create new barriers in office-based care, telehealth, and clinical trial participation.
- Technology can empower patients to take ownership of their care, but patients may be overburdened by the need to self-advocate for appropriate treatment.
- Social media-based education by trusted messengers empowers patients to self-advocate for participation in drug development and to receive the best available treatment.
- Interpreting clinical trial data gathered via digital health technology may require standards for cultural debt, cultural adaptation, and linguistic validation to account for diversity in the trial population.

Patient perspective and diversity

- There is often a lack of adequate diversity, equity, and inclusion in medical product development, posing a barrier to patient trust.
- Information about patient experience is predominantly gained through professional patient advocates rather than through patients themselves. Independent third parties or trusted messengers are also important intermediaries for communication between the pharmaceutical industry and patients.
- Direct communication with patients is essential to identify real needs, characterize treatments that are useful to patients, and determine clinical trial endpoints that are relevant to them.
- Early involvement of patients should go beyond drug development to also consider payment and pricing as well as the ultimate accessibility to the treatment.
- While the pharmaceutical industry is sensitive to diversity, equity, and inclusion, this does not translate fully into actions taken.

Calls to action for the pharmaceutical industry

Within the industry, Medical Affairs Departments and Patient Advocacy Departments are uniquely positioned to bring the patient voice into development decisions, clinical assessments, and education, and thereby deliver broadly based and more trusted outcomes by:

- Communicating openly, continuously, and consistently with patients and patient organizations, and challenging compliance barriers to communication.
- Providing patient and community education channels such as Patient Liaison personnel.
- Including a Patient Value Proposition within each Target Product Profile.
- Recruiting diverse and inclusive clinical trial populations.

1. Current challenges

Today, patient belief in healthcare systems is challenged, access to health care professionals is becoming more limited and remote, and patients are faced with multiple sources of complex and often unreliable information.

Patients' perceptions of value and trust in healthcare are intrinsically linked since value is created and trust is either earned or lost at every patient interaction. It is therefore essential to develop a thorough understanding of this dynamic, to develop strategies to improve trust, and to develop concrete recommendations that can be implemented quickly.

1 Value and trust: definitions and "patient" perspectives

In economic terms, value is defined as the cost per outcome achieved. In the context of health care, this economic definition of value is insufficient because it leaves several important questions unanswered. For example, how are outcomes defined? Since patients, their families, and caregivers are all consumers of health care, their perspectives are also all critical for determining value.

Restricting the determination of value to patients, families, and caregivers has inherent problems since it is unclear who can be counted as a patient and a universal definition of value in health care is currently lacking.

In a very broad view, everyone is a patient since everyone will develop a need for health care at some point in their lives. A narrower view that is, for example, taken by some people living with lifelong conditions is that they are patients when they are in a healthcare setting seeking treatment; at all other times, they are not patients but rather people living with their condition. This question of the context in which a person is a patient underpins a wider discussion about an overall approach to health care. Trust will often be built even before the person becomes a patient and is maintained by providing care that the patient perceives as valuable.

2 Trust undermined by societal environment and poor communication

Trust in the healthcare system is also influenced by outside developments. Data from Axios/Harris polls on industry reputation (https://www.axios.com/2023/05/24/axios-harris-poll-100-defensive-health) show that the pharmaceutical industry was not viewed positively by the public before the COVID-19 pandemic. During the pandemic, trust drastically increased but has been declining since then.

This lack of trust is exemplified by the results of a study on the views of Americans on childhood vaccines conducted by the Pew Research Center. The study assessed the percentage of Americans who agree with one of the following statements (pertaining to measles, mumps, and rubella [MMR] vaccines):[1]

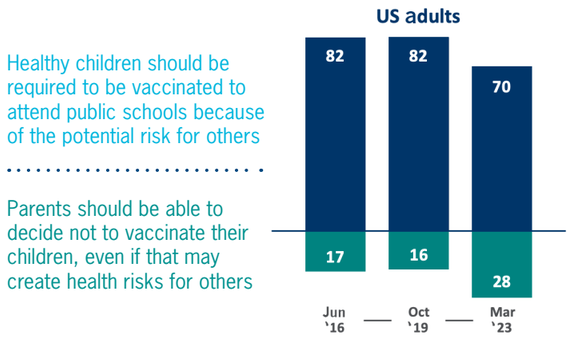
1. "Healthy children should be required to be vaccinated to attend public schools because of the potential risk for others."

or

2. "Parents should be able to decide not to vaccinate their children, even if that may create health risks for others."

The study showed a notable change in attitudes toward vaccines—likely a result of the skepticism toward the COVID-19 vaccines—and associated vaccine mandates that may have carried over to vaccines in general (Fig 1).

% of US adults who say the following about childhood vaccines for MMR



This lack of trust is paralleled by widely held negative views of prominent healthcare figures such as the former Director of the National Institutes of Health, Dr. Francis Collins, and the former Director of the National Institute of Allergy and Infectious Diseases, Dr. Anthony Fauci. These negative views of figures of healthcare authority appear to be driven by beliefs and political views as well as by poor communication of the value provided by the healthcare system.

Recognition of the breadth of challenges to value and trust in health care has led to the recent formation of the Coalition for Trust in Health and Science (https://trustinhealthandscience.org). This partnership of more than 50 organizations includes basic and applied science organizations, health services researchers, pharmaceutical manufacturers, doctors, nurses and pharmacists, foundations and policy organizations, among others. The objective of the Coalition is to build trust in health care, public health, and science across all segments of society.

3 Lack of diversity, equity, and inclusion: a barrier to patient trust

Other barriers to trust include a lack of full diversity, equity, and inclusion in drug development and healthcare systems. The historic treatment of underrepresented minorities still affects their trust today. For example, sickle cell disease affects predominantly people with African ancestry. Sickle cell is also one of the most painful diseases and requires intensive pain management. Since these patients are predominantly black, their demands for pain management are often misconstrued as drug seeking and the statements they make about their level of pain are not entirely believed by some clinicians.

Due to the limited availability of specialists, patients with sickle cell disease are often treated in emergency departments by non-specialist physicians who lack the knowledge to appropriately manage the level of pain and thereby contribute to a lack of trust among this patient group. A less visible but important set of barriers to trust is created by real-life challenges, like the hours of operation of healthcare facilities and the limited availability of specialists. Such limited access to care undermines the value to patients, which in turn leads to a lack of trust.

4 Relationships suffer from a lack of patient input and patient education

For many in the industry, patient engagement means approaching patient advocates. While they are a useful starting point, they cannot substitute for a dialog with real individual patients. Patient advocates speak for entire patient groups which results in generalizations that can disregard individual patient experiences. The lack of first-hand patient input is particularly important because the experience of patients with complex conditions from disadvantaged communities, such as a patient from the rural US South with metastatic breast cancer, cannot be adequately represented by people who don't belong to these communities.

Patients can also feel stigmatized by medical terminology. For example, patients with sickle cell disease perceive labeling their condition as a disease as detrimental and it impedes conversations about important aspects of their condition such as pain crises and anemia.

The pharmaceutical industry in particular has not widely recognized that the trust of patient communities cannot be gained through short-term episodic engagement. What patients want is reliable and consistent engagement over time including active participation in community activities. Once trust between established patient communities and a pharmaceutical company is lost, it is extremely difficult or impossible to regain. Medical Scientific Liaison personnel have proven to be effective at providing trusted ongoing education and communication with physicians. So one of the panel's recommendations is to consider the adoption of patient liaison personnel to provide reliable continued relationships with patient communities.

5 Are we overburdening patients?

Following a treatment plan is a significant burden for many patients. Simply getting medication from the pharmacy can involve multiple interactions with the pharmacist, insurance, and healthcare provider. These interactions may take several days and must be coordinated with the patient's real-life obligations. When patients are unable to follow a treatment plan they may be labeled as noncompliant. However, the use of "noncompliance" puts the onus on the patient and implies the patient does not want to follow a treatment plan, which can be a barrier to both the value of care and patient trust.

Patients with rare diseases often find themselves in a situation where they must educate healthcare providers on their condition so they can receive appropriate treatment. Many patients are now in a situation where they must advocate for themselves, for example by organizing a phone conference with the emergency department physician and their specialist even as they seek help for a pressing medical problem. This growing systemic expectation may work for educated and health-aware patients, but it exacerbates other problems of health equity.

6 Clinical trials by name and by nature

Patients rarely interact directly with pharmaceutical companies or medical product manufacturers, with the exception of participation in a clinical trial. But current approaches to clinical trial design may not include a stage where there is a review by patients of the techniques that will be employed to measure impact. Even with recent trends promoting patient-focused drug development, patient communities are not consistently included in that process. As a result, clinical trial endpoints may be chosen without patient input and so may not resonate with patients. Moreover, narrowly defined inclusion/exclusion criteria can lead to the exclusion of a wide range of individuals from clinical trials, which, if not clearly explained, can destroy hope and erode trust among patients.

The pursuit of ever-higher precision in outcome measurements has led to the present, lengthy validation processes for reliable patient-centered endpoints. Also, when patient-centric measures are integrated into trial designs they frequently fail to meet the standards required for clinical evidence and cannot be included in the eventual label. To include patient-focused outcomes in the label they must be integrated from the early stages of the planning process.

Overall, the disconnect between the pharmaceutical industry and patients often leads to a situation where the industry develops its products for patients instead of with patients.

2. Technology provides new opportunities but may also create new barriers

At present, health care is fundamentally changing through the introduction of Digital Health Technology (DHT) for a wide variety of applications. DHT is defined by the US Food and Drug Administration as "Systems that use computing platforms, connectivity, software, and/or sensors for health care and related uses. They include technologies intended for use as a medical product, in a medical product, or as an adjunct to other medical products (devices, drugs, and biologics). DHTs may also be used to develop or study medical products."[2] Here, we will discuss DHTs in the context of pharmaceutical product development.

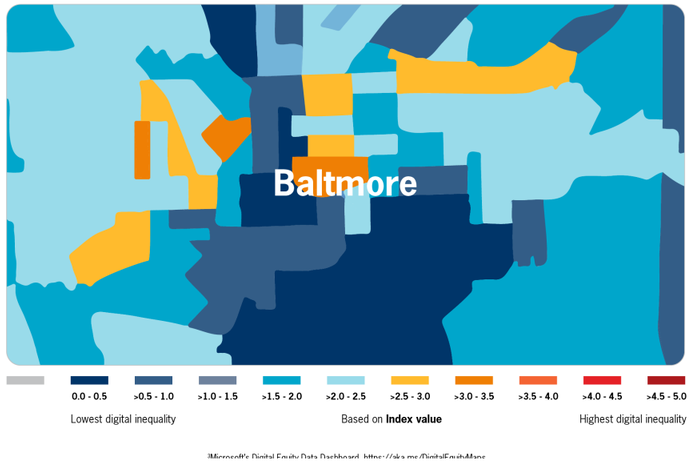
Telehealth

The expansion of telehealth during the COVID-19 pandemic has created increased access to specialists for some patients since telehealth can be delivered to a patient's home at short notice and without long travel and waiting times.

Despite the obvious opportunity for increased patient access, telemedicine can create privacy concerns for some patients. For instance, patients may not have privacy at home and may prefer to take telehealth appointments from their car where they cannot be overheard by family members when discussing sensitive health issues. Beyond the privacy concerns related to telehealth, access may be limited by digital inequity in the US,[3] as well as limited financial means and knowledge required to afford and operate the equipment necessary for a telehealth appointment.

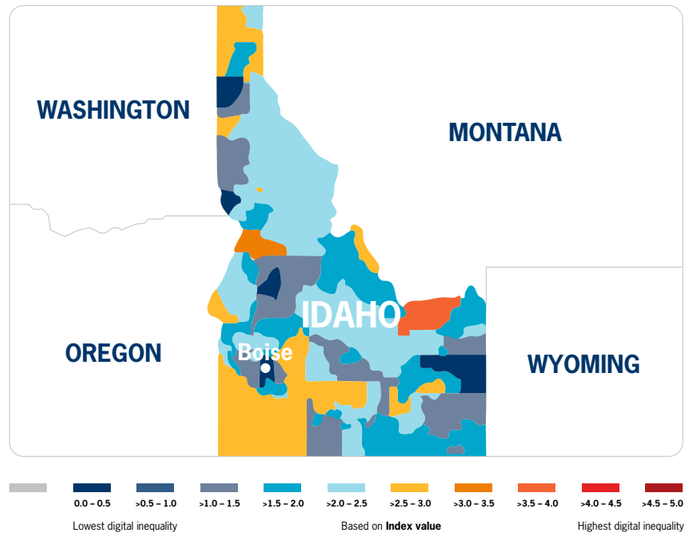
Digital inequity can exist in many rural parts of the US, such as in rural southern Idaho, or in urban areas, such as certain neighborhoods in Baltimore (Fig 2). Together with economic disparities, this digital inequity can contribute to a form of "technologic apartheid" that excludes some populations from the use of telemedicine.

Digital equity by census tract



[4]Microsoft's Digital Equity Data Dashboard. https://aka.ms/DigitalEquityMaps

Digital equity by census tract Ref 3



Microsoft Digital Equity Data Dashboard: identifies regions with digital equity gaps indicated by factors including: low rates of broadband availability, low rates of broadband adoption, low rates of broadband usage, gaps in broadband affordability, low rates of computer ownership, and other indicators.

3. Strategies: for greater equity, communicating value, and building trust

1 Promoting health equity

The Centers for Disease Control and Prevention (CDC) defines health equity as “the state in which everyone has a fair and just opportunity to attain their highest level of health. Achieving this requires focused and ongoing societal efforts to address historical and contemporary injustices; overcome economic, social and other obstacles to health and healthcare; and eliminate preventable health disparities.”[4] As such, achieving health equity is fundamental to providing value and to establishing or regaining patient trust, and represents a significant opportunity to extend the provision of healthcare value.

In April 2022, the US Food and Drug Administration (FDA) issued a draft guidance to increase racial and ethnic diversity in clinical trials.[5] Achieving health equity in drug development and clinical trials requires the recognition that underserved groups exist in the US and that underlying problems are systemic. While the pharmaceutical industry is aware of the guidance and the underlying problems of diversity, equity, and inclusion, companies have been slow to date to act. An essential step toward accelerating the integration of health equity within the pharmaceutical industry is to establish far more collaborations with external partners throughout drug development.

Executing such initiatives requires the recognition that large and sustained financial investment is required in recognition of the responsibility of multinational pharmaceutical companies to develop therapies that work not just for some patient groups, but instead to develop products that deliver value to all such patients.

One example program that is aimed at establishing health equity and regaining patient trust is the “A Million Conversations initiative” by Sanofi.[6] The goal of this initiative is to acknowledge and recognize the health disparities existing around the world and to learn how these issues can be addressed—beginning by starting a dialog with patients.

2 Communication with patients and across stakeholder groups

Communication is an essential tool for addressing problems of patient equity and regaining patient trust. The communication required to reach these goals must not only involve all healthcare stakeholder groups, but also overcome barriers to communication created by regulatory requirements, mistrust, and political divisions in the wider society. The desired outcome of this communication is to identify unmet needs, characterize treatments that are useful to patients, and determine clinical trial endpoints that are relevant to patients. The experience gathered by organizations like Braver Angels[7] indicates that while divisions may look insurmountable initially, a surprising amount of agreement can be achieved once the conversation is started across stakeholder groups and common values are identified.

3 Communication with patients via trusted messengers

Trusted messengers are generally patients who build a community of people who share the same disease and are important intermediaries for balanced and appropriate communication between pharmaceutical companies and patients. Having access to individual patient experiences may enable a more holistic, person-centric approach to drug development and clinical trial conduct.

With education by trusted messengers, patients can be empowered to self-advocate for participation in drug development. The education provided must be accessible to patient communities, and hence social media and other digital media are preferable to brick-and-mortar venues as settings for education. Using digital platforms also increases the reach of information to patients from historically excluded communities who may not want educators to have access to their communities or homes for fear of bias and stigmatization.

4 Medical Affairs role in building patient trust

Medical Affairs departments and Patient Advocacy departments are uniquely positioned to bring the patient voice into development decisions, clinical assessment, and education, and thereby to deliver broadly based, ongoing, and meaningful engagement with patients and patient communities. Similarly, they are best positioned to raise internal awareness that the effective application of rapidly advancing medical science requires a deep understanding of the patient populations and individual needs. Most importantly, in the context of this discussion, Medical Affairs departments can play a critical role in maintaining longer-term relationships with patients and maintaining their trust as the interaction with other departments changes through product development and launch. To gain the trust of underserved patient populations, the industry must be seen to act on the consultations made. Medical Affairs should therefore be an active partner in cross-industry and across healthcare initiatives that further increase the value delivered to all patient groups.

Education of historically excluded populations can make the difference between acceptance and rejection of available treatments. For example, a Native American tribe in the Pacific Northwest was approached for participation in a vaccine trial for COVID-19. Before agreeing to participate, the tribe had many questions about the trial. These questions were not adequately answered by the trial sponsor and the tribe declined to participate. This failure of communication, therefore, resulted in the failure to include an underserved population in the trial. However, once the vaccine was released, the tribe could access all the information they needed and were consequently vaccinated faster than the general public.

5 Designing clinical trials that integrate patient input and address patient needs

Including the patient perspective in clinical trial design

Patients should be included in identifying unmet medical needs and in developing clinical trial endpoints which are designed to meet real-world patient needs.The process should clarify how patients understand medical terms like “anemia” or “fatigue,” what terminology patients might find offensive, or what imagery is appropriate for a patient population. Gathering and integrating this information can be a lengthy process often requiring several months. But effectively understanding these nuances about patient populations can make the difference between the success and failure of a clinical trial.

This is especially true for patients with ultra-rare diseases where clinical trial populations are necessarily small. A final consideration for the design of patient-centric clinical trials is the question of what information gathered in the trial can be shared with the patient or the patient’s other healthcare providers.

6 Changing mindsets to deliver value and build trust

Implementing a patient-centered approach to health care that delivers value to and earns the trust of patients requires a change of mindset among all stakeholders. This approach to health care should enable the transition from disease management to promoting health and well-being. It should be based on a transparent determination of value that is understood by patients, providers, and payers. The biggest challenge to implementing this approach is changing the mindset of the pharmaceutical industry. This change would require recognition by the industry that:

- It cannot hope to understand the mindset of communities that industry decision-makers do not belong to or participate in.
- Help does not go one way from the pharmaceutical industry to the patients but rather that the pharmaceutical industry and patients help each other.
- Patients put their health (and sometimes their lives) on the line in clinical trials and therefore deserve adequate and timely compensation.

A change of mindset is also required from healthcare providers. While the training of physicians is heavily focused on science, education on sensitivity and cultural competence will be required to bring high-value health care to underserved communities and earn their trust. Implementing changes in the healthcare system that enable high-value care and gain or regain the trust of patients requires long-term investments and actions. However, these long-term developments can begin with measures that can be implemented in the short term. Here we list several calls to action for members of the pharmaceutical industry.

Innovating clinical trials

In conventional trials, patients make visits to physical treatment centers, whereas in decentralized trials, the interaction with patients is enabled by technology. Such technologies used in decentralized clinical trials include electronic administration of clinical outcome assessment data collection, telehealth or remote clinical trial visits, and wearables or sensors for passive data collection.

Patients tend to feel more supported in decentralized trials because the protocol includes more interactions with the care team as opposed to centralized trials where patients must wait for an in-person visit. Using telehealth appointments can avoid barriers to participation such as hours of operation and burdensome travel to the trial site. Overcoming these obstacles can make clinical trials much more accessible and inclusive.

Nevertheless, decentralized clinical trials can have disadvantages. Some patients may prefer the option to have an office visit because they value in-person interaction. Other concerns surrounding clinical trials include the development and validation of tools such as redesigned visual analog scales included in an app, and the interpretation of data collected through DHTs.

Also, data collection through DHTs may not be appropriate for some disease states. For instance, a wearable device in a schizophrenia trial would mean putting a tracker device on members of an already paranoid population. The cultural diversity of a trial population may also pose challenges to data interpretation. Data interpretation should include standards for cultural debt, cultural adaptation, and linguistic validation to determine whether measurements and scores collected via DHTs are equivalent across cultural groups.

Data collected via DHTs can be skewed by unforeseen events such as failure of a device used for the trial, but more complex misinterpretations are also possible. For example, in a clinical trial for a rheumatoid arthritis treatment, the hypothesis was that as pain decreased, the patients would move more and this increase in movement would be recorded by a wearable device. In this study, one patient reported decreasing pain while the wearable device registered an absence of movement. It was initially suspected that the patient was not wearing his device or not using it correctly. However, when asked, the patient reported that the device was working properly, and he was now feeling well enough to resume his sedentary work as an author. Such examples illustrate the importance of understanding the full patient experience to correctly interpret data recorded with DHTs.

4. Calls to action!

1 Communicate openly and consistently with patients

Communication with patients should occur frequently and use patient-owned forums and advocacy groups to provide a neutral ground. As already identified, there is a need for Patient Liaison personnel to mirror the current and well-recognized activities of Medical Science Liaisons.

Patients should be involved in:

- Identifying meaningful unmet clinical need(s), target conditions, and target product profiles.
- Generating insights into the patient journey including the behavioral journey, clinical journey, and long-term care management.
- Design, recruitment, and execution of clinical trials.
- Identifying patient-centered outcomes and patient preferences, especially in rare disease trials.
- Decision-making processes, health technology assessment decisions, and appeals processes, as well as treatment and prescribing decisions.
- The collection of real-world evidence on efficacy and safety, as well as patient and caregiver support.

The relationship with patients should be ongoing and long-term to maximize treatment outcomes.

2 Provide patient and community education

Community education enables patients to ask for the treatments they need. It should be transparent about the drug development process and empower patients to understand the results of clinical trials. Community education should also be responsive to patient concerns and give patients a mechanism by which they can share their concerns.

Again, Patient Liaison personnel could be especially effective in delivering consistent community education.

3 Include a Patient Value Proposition within each target product profile

The drug development process should reflect the specific needs of the targeted conditions and patient populations, and patient value must underpin the development process. Achieving this flexibility requires a commitment to integrating a Patient Value Proposition into the target product profile.

Reflecting the patient's perspective in drug development processes and procedures requires a thorough approach with advanced analytic techniques to better capture the whole of the patient's experience.

4 Recruit diverse and inclusive trial populations

Clinical trials are designed to recruit from a narrow pool of people who can qualify for participation but this can lead to problems in treating real-world patients. For instance, a new treatment may not have been studied in people with a history of cancer. This problem becomes even more pronounced if diseases that are prevalent in certain populations are excluded. For instance, if clinical trial protocols exclude patients with comorbid cardiometabolic conditions, many people with those conditions may not have access to the new treatment. To ensure equitable access to treatment, it is essential to recruit from a diverse patient pool.

5 Bring everyone into the conversation

The dialog around changes in the healthcare system must include all stakeholder groups, including the pharmaceutical industry, healthcare providers, payers, regulators, and patients. At present, the communication between these groups is so limited that each group may well have "unknown unknowns" about the other groups. These knowledge gaps must be identified and addressed. It will also require challenges to compliance barriers to communication.

Concerns about data privacy can limit access to patients, and in turn, may limit the development of more patient-centric products. It is therefore imperative to find legally and ethically acceptable avenues for communication with patients. Identifying these avenues requires collaboration between departments inside pharmaceutical companies as well as collaboration between other stakeholder groups.

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