Principles of Trustworthy Communication

Among Patient Communities and Health Care Payers Regarding Novel Therapies

Findings from the NEHI Conference on Novel Therapies and Patient-Payer Communication

About This Report

This report is adapted from the lay conference summary submitted by NEHI to the Patient Centered Outcomes Research Institute (PCORI) in conformance with a PCORI Eugene Washington Patient Engagement Award, (Project EAIN 19981, "Developing Principles of Communication Between Patient Communities and Payers Regarding Novel Therapies."). It summarizes findings from a conference organized by NEHI-Network for Excellence in Health Innovation with the assistance of a nine-member project advisory committee consisting of patient community leaders, health care payment experts, and experts in health economics and outcomes and patient-centered outcomes research. The conference was held online through the Kaiser Permanente Center for Total Health virtual conference center platform on July 27-28, 2021. Over fifty patient advocates and health care payer leaders participated over the course of the two-day conference.

The views presented in this publication are solely the responsibility of NEHI and do not necessarily represent the views of the Patient-Centered Outcomes Research Institute® (PCORI®), its Board of Governors or Methodology Committee.

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About NEHI

NEHI is a national nonprofit, nonpartisan organization composed of stakeholders from across all key sectors of health and health care. Its mission is to advance innovations that improve health, enhance the quality of health care, and achieve greater value for the money spent. NEHI consults with its broad membership, and conducts independent, objective research and convenings, to accelerate these innovations and bring about changes within health care and in public policy. (nehi-us.org)

About PCORI

The Patient-Centered Outcomes Research Institute® (PCORI®) is an independent, nonprofit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers, and clinicians with this evidence-based information needed to make better-informed healthcare decisions. PCORI is committed to continually seeking input from a broad range of stakeholders to guide its work. (pcori.org)

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Executive Summary

The NEHI project on patient-payer communication was conceived to address an emerging need for communication among patient communities and health care payers regarding the introduction of newly approved novel therapies in our health care system. The primary goal is the development and dissemination of communication principles upon which patients and payers can build relationships for effective and trustworthy exchange of information.

A statement of principles was developed in consultation with a project advisory committee comprised equally of patient representatives and payer representatives. The statement was presented to a July (2021) virtual conference of over fifty key opinion leaders from patient communities and payer organizations, and subsequently revised based on discussions at the conference and comments submitted thereafter.
The statement centers on five over-arching principles:

1. Trust, empathy and transparency among patients and payers
2. Representativeness of patient voices and payer recognition of representativeness as demonstrated both by the authenticity of the patient experience communicated to payer, and the diversity of patients engaged in communication
3. A shared focus among patients and payers on evidence and a commitment to collaborate on the generation of real-world evidence
4. A shared focus on the impact of novel therapies on total costs of patient care, by patients and payers
5. Timeliness of communication, including communication that will occur ideally at points before payer decisions are made on coverage of new therapies and patients have the highest expectations for timely access

Conference attendees recognized major barriers to communication that must be overcome to sustain systematic, good faith communication, including:

1. A better understanding by patients of complex and evolving payer decision-making processes
2. Expanding opportunities for patients to address larger audiences of payers (i.e., find “common points of entry” into the payer sector)
3. Overcoming barriers to appropriate patient communication with payers before approval of novel therapies, such as at points when payers undertake horizon-scanning or drug pipeline reviews, processes that ultimately influence payer coverage of new therapies

Background

The project goal: address an emerging need for communication among patient communities and health care payers

This project addresses an emerging need for communication among patient communities and health care payers regarding the introduction of newly approved novel therapies in our health care system.[1] The primary goal is the development and dissemination of principles upon which patients and payers can build relationships for communication that is effective and trustworthy. A statement of principles was developed in consultation with a project advisory committee (four patient leaders and four payer thought leaders). The statement was presented to a July (2021) virtual conference of over fifty key opinion leaders from patient communities and payer organizations and revised based on discussions at the conference and comments from participants submitted in the weeks following.

The need for communication is driven by what is likely to be the continued approval of dozens of novel therapies by the U.S. Food and Drug Administration in coming years. If past is prologue, some novel therapies will prompt concerns among payers regarding their cost and the depth of evidence supporting their adoption. Patient communities have a vital stake in payers' evaluation and decision-making on these therapies, hence the emerging need for two-way communication among patients and payers.

The statement of principles (described below) should serve as one step, albeit an early step, towards channeling patient-payer communication towards constructive and evidence-based engagement regarding payer decisions that can be difficult for patients and payers alike. Dialogue at the July 27-28 conference and NEHI's background research affirmed that there is little systematic communication between patient communities and payers regarding adoption of novel therapies underway today. While patient groups and payer organizations can and do reach out to each other on an ad hoc basis, there are few organized processes in place that enable patient groups to systematically address large groups of payers when payer decisions are pending or enable payers to systematically reach out to patients who may have expertise that will be useful for payer decision-making. This absence of reliable and predictable channels of communication is a major barrier to patient-payer communication, but it also represents an opportunity for organizations that represent or serve large groups of patients or groups of health care payers, such as "umbrella" patient organizations (patient organizations that are not disease-specific, but represent or support multiple, disease-specific groups), payer trade associations, professional societies or affinity groups that serve payer organizations and payment professionals.

The emerging need for patient-payer communications also creates an opportunity and a challenge for the field of health and medical communications. Health communications research and practice are centered naturally around principles of good communication to patients, not communication from patients to others (payers, in this case).

PCORI's newly enunciated national priorities are a case in point. They center around research on methods to render medical and health-related evidence understandable and usable by patients. The launch of novel therapies is now driving some patient communities towards communicating with payers with information on their patient experience and, in some cases, with independent evidence. Payer decision-making on coverage of novel therapies also increases the demand on payers to communicate their decisions back to patient communities with transparency and clarity. Thus, the dissemination of guiding principles of patient-payer communication may be especially timely.

Defining the 'communications gap' in dialogue among patients and payers regarding novel therapies

Patients, patient groups and payers communicate often on many issues. Yet communication is more likely to be ad hoc at those points in time when a novel therapy is approved and payers face decisions on whether the therapy will be covered for patients, and the terms and conditions that will apply, such as terms of utilization management. Communication in the form of appeals from patients and clinicians over denials of coverage, or communication regarding prior authorization is routine, formally structured and may be regulated as a matter of state insurance law or (in the case of federal health programs) by federal regulation.

In the future payers and patients may have more access to patient-focused evidence at the time novel therapies are approved. The FDA's Patient-Focused Drug Development (PFDD) program, launched in 2012, engaged patient communities in early consultations to identify patient-centered outcomes for inclusion in, or further development as endpoints in clinical trials.[2] The current Patient-Focused Drug Development Program supports patient organizations in convening "externally-led patient-focused drug development" meetings to identify outcomes and endpoints of importance to patient communities.[3] Under terms of the 21st Century Cures Act (enacted in 2016), the FDA has created a program to qualify clinical outcome assessments (COAs) for utilization in clinical trials under specifically defined circumstances. Clinical outcome assessments may include patient-reported, parent-reported, and other caregiver-reported outcomes.[4] In 2019 the FDA launched a pilot grant program to support development of clinical outcome assessments in specific disease states.[5] The program goal is to develop sets of COAs that will serve as core outcome measure sets for determination of valid endpoints in clinical trials.

These developments remain works in progress, however. The evidence available for payers to consider when a novel therapy is approved may or not be fully informed by the experience of the full range of patients who might be treated by the therapy, or by the full range of outcomes seen by patients as critical to their well-being, such as quality-of-life outcomes. Participants in NEHI's July 2021 conference on patient-payer communication generally agreed that a "communications gap" exists relative to patient-payer communication when payer decisions on coverage of novel therapies are imminent.

What do patients and payers need to communicate to each other? The key issues

The emerging need for communication regarding novel therapies is driven by several high priority concerns among health care payers and patient communities. For payers, two concerns seem paramount.

Cost

Cost may be measured not only by a therapy's initial price, but by the total costs of administering the therapy, since many novel therapies are administered in hospital settings or otherwise involve complex processes of administration, (for example, hospital-based procedures involving complex preparations and monitoring, transfusions or even surgery).

Evidence

A second key issue for payers is the strength of evidence of the effectiveness of the novel therapy, and the generalizability of evidence developed in FDA-approved clinical trials to results that will be generated in real world clinical settings. For example, twelve of the 53 novel drugs (23 percent) approved by the FDA's Center for Drug Evaluation and Research (CDER) in 2020 received an accelerated approval.[6] The FDA's accelerated approval pathway allows the agency to approve drugs based on clinical trials utilizing surrogate endpoints (proxies for endpoints typically observed over longer periods of time, such as measures of patient survival from cancer), subject to further confirmation in post-approval trials. Accelerated approvals have become increasingly common in recent years. The FDA's accelerated approval pathway is designed to expedite patient access to new therapies that meet serious, unmet medical needs. In some cases, payers see the effectiveness of a new and novel therapy as too uncertain, and potentially too limited, to justify the cost of the therapy. At the same time, both patient and payer participants in the July 2021 conference concurred that some novel therapies may result in offsetting current costs of care for patients and generate better outcomes and savings for patients and payers, which underscores a shared interest among patients and payers in generating reliable real-world evidence.

Concerns in patient communities fall into four categories.

Access

The first concern is access. Patients who may be eligible for a new and novel therapy are concerned that payers make prompt decisions on policy that will make the therapy available to any patient eligible for the therapy (such as decisions to include the therapy on the payer's drug formulary), and that the decisions minimize the amount of time an individual patient must wait (minimize time-to-therapy) before a clinician is approved to treat the individual with the novel therapy (such as minimizing time for prior authorizations).

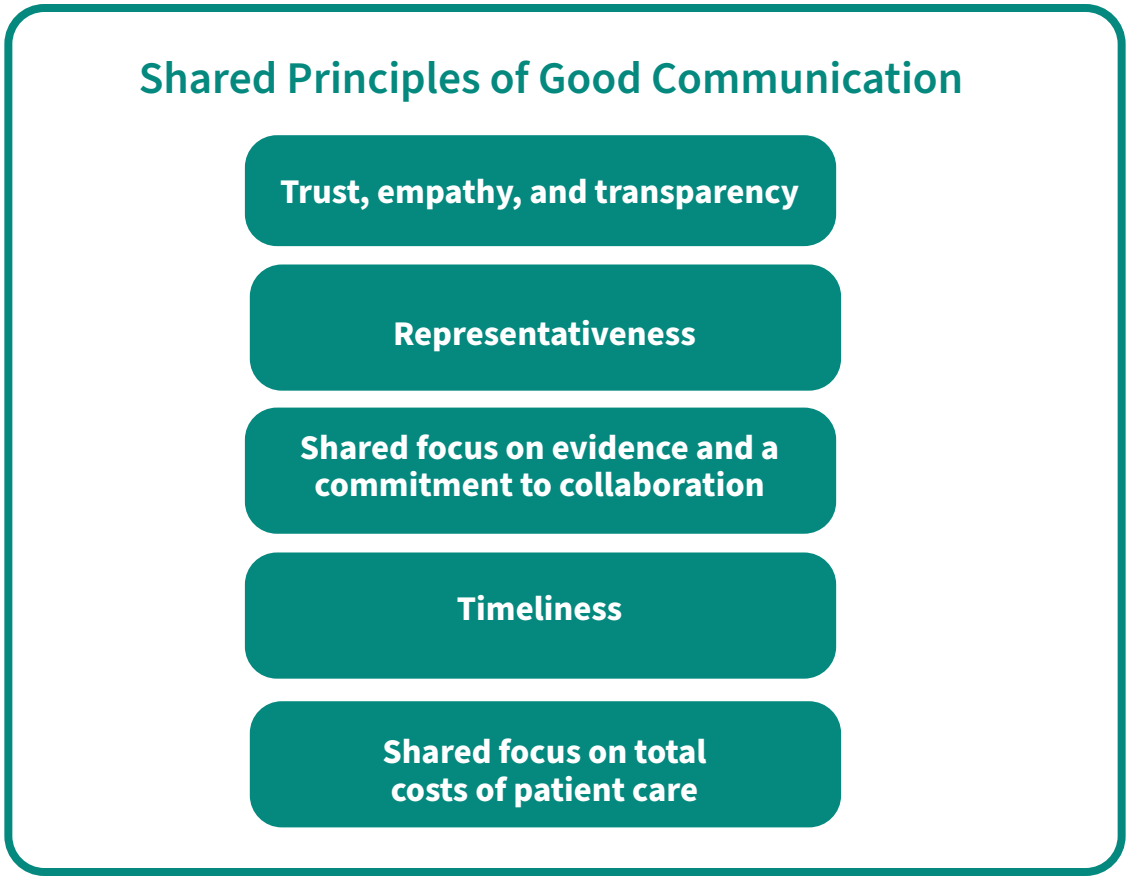
Affordability
The second concern is affordability. Patients are concerned that their share of costs for administration of a novel therapy will not be so high as to lead them to decline or discontinue therapy, or to create an unsustainable financial burden.

Patient experience
A third concern for patients is the ability to communicate their perspective on the patient experience to payers before final payer decisions are made. When patients perceive that clinical trial evidence (or payers' assessment of clinical trial evidence) is not fully reflective of patients' experience with their disease and the potential impact of a new and novel therapy on their quality of life, they will seek opportunities to communicate their patient experience to payers. This may be especially true if clinical trial evidence does not include results that are based on patient-focused, but less traditional outcome measures such as patient-reported outcomes, or caregiver-reported outcomes.

Patient expertise
A fourth concern can be defined as communication of patient expertise, as distinct from patient experience. Patients and caregivers are experts in the daily management of patient care. This is especially true of patients with rare disease or low prevalence conditions. Their expertise may extend to important factors or nuances of care that may not be apparent to clinical trial designers or payers: issues in daily medication management, for example. In addition, patients with rare conditions, relatively low-prevalence, or complex conditions are often treated most successfully by a relatively small number of frontline clinicians who may or may not be active in clinical trials or in generating real world evidence. The ability of patients to recruit these frontline clinicians and offer their expertise to payers can be an important factor in assessing a novel therapy, including opportunities that may arise to generate savings in the total costs of patient care.

Principles of Patient-Payer Communication Regarding Novel Therapies

Participants in the project advisory committee and in the July conference expressed general agreement around five over-arching principles.



1. Trust, empathy and transparency

Patient-payer communication regarding novel therapies should start from a presumption of trust and empathy from each side to the other. The presumption is that patients and payers both seek the best outcomes for patients. Ultimately patients and payers should strive to build a concrete foundation for sustained trust. At least three factors are important:

- **Standards of transparency**
  Patient groups that are capable should adopt and maintain recognized standards of transparency in their governance and funding sources. Patient groups that are less experienced or less well-resourced can still seek to emulate standards of transparency in their communication with payers.[7]

Payers should enunciate and maintain clear standards of transparency in communication to patients regarding their decisions on formulary inclusion, benefit design, utilization management and contracting.[8]

- **Best practices in patient engagement**
  Patient engagement methods are evolving in fields such as patient-focused drug development, health economics and outcomes research (HEOR), and in clinical practice. Adaptation of best practices from these fields to patient engagement with payer decision-making processes (such as pharmacy and therapeutics – "P&T" – deliberations or utilization management decision-making) could help lay a foundation for trustworthy communication among patients and payers.
- **Data**
  Empirical evidence is most persuasive to payers, (see "Shared focus on data and commitment to collaboration," below). Patients who can communicate with data to payers are in the best position to sustain a relationship of trust and empathy. On their part, payers will be best positioned to sustain trust when they convey a genuine openness to assess data on historically under-studied or under-reported patient outcomes that patients identify as important to their well-being (for example: patient-reported outcomes that may not be represented in clinical trial evidence).

2. Representativeness

Representativeness is a foundational factor in trustworthy and effective communication, in at least two respects:

- **Authenticity**
  While many persons or organizations may have valuable perspectives on patient experience to offer to payers, patient-payer communication should be grounded in the perspective of real patients, family members and caregivers who represent authentic experience with the patient journey.
- **Diversity**
  Patient communities and payers alike have a shared responsibility to consider diversity in the patient population when decisions are imminent on formulary inclusion, benefit design and utilization management of novel therapies.
  Patient groups have a responsibility to be representative of diversity in the larger patient population, or to recognize and communicate the limitations on their representativeness. Alliances and collaborations among patient groups that represent a broad spectrum of patients in the patient community will enhance trustworthy communication.
  Payers have a responsibility to consider the impact of formulary inclusion, benefit design and utilization management decisions on patient subpopulations and on patients who face diverse, real-world barriers to equitable health care. Barriers may include social risks faced by patients and systemic biases in the health care system that impede care.

3. Shared focus on evidence and a commitment to collaboration

Patients and payers may disagree, at least initially, on the terms and conditions of covering a new and novel therapy. Ultimately both have an interest in proof of effectiveness (how well diverse patients within the patient community will respond to the new therapy). A shared patient-payer commitment to evidence is another foundational factor for sustained, trustworthy and effective patient-payer communication.

Depending on the circumstances, patients may have several distinct types of expertise to communicate to payers:

- **Expertise from patient-focused drug development**
  A small but growing number of new therapies are now developed as a result of early engagement by patient communities. Patient engagement can be seen in sponsorship of early-stage research, participation in (or creation of) patient registries, participation in clinical trials, development of patient-centered outcome measures, and other aspects of patient-focused drug development. Insights from this experience should inform patient-payer communication.
  Patients have a special motivation to communicate evidence and perspective on the impact novel therapies may have on patients' quality of life and functional status, and the impact of treatment on family members and caregivers. Metrics of these outcomes may be in the form of clinical outcome assessments ("COAs," such as patient-reported outcome measures, or PROMs) that are historically under-evaluated, or for which metrics have been validated and reported only recently. Reports of patient perspectives on such overlooked measures can be a significant contribution to closing the patient-payer communication gap.
- **Expertise from the patient journey**
  Patients, family members and caregivers are expert sources of knowledge on their conditions and in the standards of health care available to them, including costs of care. Patients and caregivers may be expert sources for payers on opportunities to achieving savings in costs of care (see "Shared focus on total costs of care," below).
- **Expertise in identifying and recruiting expert frontline practicing clinicians**
  Novel therapies are approved to treat unmet medical needs. While research scientists and clinician-researchers may be experts in the biological mechanisms of a novel therapy, frontline clinicians are experts in the management of patients with unmet needs. Frequently a relatively small number of frontline clinicians are the leading experts on treatment of patients with rare, low-prevalence, or hard-to-treat chronic conditions. Patients are frequently the best source for identifying the ranks of expert frontline clinicians.

- **Partners in development of real-world evidence (RWE)**
  Patient communities can build confidence in their communication with payers by signaling their openness to collaborate or participate in the generation of real-world evidence of the effectiveness of the new therapies.
- **Payer initiative**
  Payers should be transparent in communicating specific concerns about existing evidence regarding use of a novel therapy and their goals for generating additional evidence. This includes communicating as clearly as possible their concerns about gaps or weaknesses in the evidence available to them when a novel therapy is launched.

4. Shared focus on total costs of patient care

In the best-case scenario, the introduction of a new and novel therapy will lead to transformative, even curative outcomes for patients. Novel therapy may eliminate the need for at least some recurring health care services previously needed to sustain the patient's health. Patients and payers should have a shared interest in finding opportunities for improving the efficiency of care and reducing total costs of care, provided new therapies generate better outcomes for patients. Patients and payers can act on this in at least four ways:

- **Sharing insights on the cost burden of disease**
  Patients are positioned to share with payers their experience with the cost burden of their disease, and opportunities to reduce or eliminate unnecessary costs to patients and payers through more efficient care, and insurance coverage that supports efficient, coordinated care.
- **Real-world evidence (RWE)**
  Patients and payers' shared interest in generating real-world evidence on the effectiveness of a novel therapy should extend to their mutual interest in generating RWE on the total costs of patient care, measured over sufficient time to demonstrate a novel therapy's durability.
- **Utilization management**
  Utilization management decisions (such as decisions on prior authorization of therapy) may determine the time-to-therapy for a patient expecting treatment and the progression or severity of a patient's condition. This will influence patient outcomes and total costs of care, measured over time. Thus, some appropriate patient engagement with utilization management decisions may be essential for realizing patients and payers' joint interest in optimizing total costs of patient care.
- **Value-based payments**
  Many payers are committed to alternative, value-based payment arrangements (such as outcomes-based contracting) for health care providers and for purchasing novel therapies from pharmaceutical manufacturers. Patient-payer communication should align around opportunities for patient engagement with payers and manufacturers to allow patients to inform the design of value-based arrangements that aim to optimize outcomes and total costs of patient care.

5. Timeliness

Virtually all health care payers operate on annual budgets, and most offer health plans that require yearly enrollments. Payers plan for the introduction of new therapies up to 18 months in advance of a health plan year. Payers' planning assumptions regarding a novel therapy may naturally change when the therapy is finally approved and its launch price and associated costs of administering the therapy are more apparent. Patients' expectations for prompt access to a new therapy are usually highest precisely when a novel therapy is approved and launched, which is also when payers face the greatest time pressure to render decisions on formulary inclusion, cost sharing with patients, utilization management, and contracting.

Patients and payers would be best served by communication in advance of the launch of a novel therapy. In practice there are substantial barriers and constraints to effective communication, such as the uncertainties over the final state of clinical evidence supporting the therapy, and its ultimate price and associated costs. Patient-payer communication before FDA approval may also be constrained indirectly by FDA regulation. Pre-approval communication of health economics and outcomes information from pharmaceutical manufacturers is regulated by the FDA and generally restricted to communication with payers and health care providers.

Changes in policy may be necessary to facilitate reasonable, pre-approval communication between patients and payers regarding a novel therapy. Valuable communication can still be exchanged regarding the patient experience with the disease targeted by a novel therapy awaiting approval, and other information that sets a context for decisions that payers will make after full FDA approval.

Challenges and Opportunities for Adoption

The statement of communication principles outlined above will be disseminated widely to patient groups and health care payers, in keeping with a major goal of this PCORI-funded project. However, research, discussions in the project's advisory committee meetings, and discussion at the July 2021 project conference affirmed that there are several challenges that confront well-intentioned patients and payers in building trustworthy communications relationships. The major challenges include:

Barriers to patients' understanding of payer decision-making processes

Patient thought leaders perceive payer decision-making processes as a "black box." Payer leaders suggested that payers (or organizations acting on behalf of the broad health care payer community) reach out to patients with programs to provide education and understanding of payer decision-making processes and the goals, mandates, and constraints payers face. Novel drugs are invariably classified by payers as specialty pharmaceuticals and trigger a higher level of scrutiny by payers compared to more conventional drugs.

Thus, patient education in payer decision-making processes should be updated frequently to keep up with the increasing complexity payers face in evaluating novel therapies and determining payer policy on coverage. The current stream of novel therapies emerging with FDA approval often employ complex and (by definition) novel biological approaches to treating disease. This has triggered more complex review processes on the part of payers and introduced more steps and more intermediaries (such as organizations performing specialized analyses) into the chain of decisions made by payers on formulary inclusion, benefit design, and utilization management of novel therapies.

Limited opportunities for patients to engage with multiple payers

The health care payment system in the U.S. is decentralized, with thousands of private sector organizations and multiple public programs each covering health care benefits for their segments of the population. While there are vehicles for pharmaceutical manufacturers to place information on novel drugs in a central location accessible to multiple payers,[9] similar vehicles do not exist that enable systematic communication between patient groups and health care payers. Patient organizations must make a choice as to which payers they will target for engagement, and when. This is a serious challenge for smaller or less well-resourced patient organizations, but leaders from larger and more established patient organizations reported at the July 2021 conference that they are also forced to make strategic decisions on which payer organizations they will approach for engagement and communication. There are few programs or initiatives in place that enable patient organizations to find "points of entry" to the payer sector and allow them to address large aggregations of payers all at once.

Countries outside the United States that perform health technology assessment (HTA) of novel therapies have created programs for engagement and communication with patient communities that provide a useful comparison to the more informal and ad hoc channels of communication in the U.S. Unlike the U.S. health care system, these systems look to a central HTA agency to assess the cost effectiveness of a new therapy, and results of the assessment inform price negotiations or price-setting by the payment authorities in these countries. The HTA agencies thus become a single focal point for patient communities to engage with each country's health care payment system. Prominent examples include Canada (Canadian Agency for Drugs and Technologies in Health, CADTH), England (National Institute for Health and Care Excellence, NICE), and Germany (Institute for Quality and Efficiency in Health Care, IQWIG).

The patient engagement programs of foreign HTA agencies vary in the scope and depth of their outreach to patient communities, but several functions are common in these programs, including –

- **Patient recruitment**
  HTA agencies use several processes to identify individual patient representatives for active involvement in HTA reviews of new therapies. Patients are recruited by various methods: by nomination from patient groups, by proactive searches by the HTA agency, and by targeted outreach to patient representatives with special expertise.
- **Direct support of patients and patient groups**
  Methods employed by HTA agencies include programs to train patient representatives in HTA methods, and assignment of facilitators to assist patient representatives in marshalling data and devising presentations to the HTA agency. Some programs provide for direct reimbursement of patients for their time.
- **Rules of transparency for patient representatives and for the HTA process**
  HTA agencies impose various requirements on patients and patient organizations to make formal disclosures of conflicts of interest. The HTA agencies also adopt rules for their own procedures that guarantee rights of public comment and public participation in HTA proceedings, and in review of HTA reports.

There is no central HTA authority in the U.S. health care system, but the non-governmental, non-profit Institute for Clinical and Economic Review (ICER) now conducts cost-effectiveness assessments (defined as 'value assessments') of several new and novel therapies every year. Thus, ICER increasingly serves as a focal point for patient community engagement with the U.S. payment system as regards new and novel therapies, albeit at one step removed from payers and payer-supporting organizations such as health insurers, prescription benefit managers, and self-insured employers.

ICER has established its own processes for outreach to and engagement with patient communities who are stakeholders in the clinician and payer adoption of novel therapies.[10] Several U.S. organizations have responded to the increasing role of HTA in both the U.S. and foreign health systems by developing new frameworks for engaging patients in meaningful roles with HTA reviews.[11] A European Union-funded initiative, the Innovative Medicines Initiative (IMI) has also funded a "Code of Conduct" for patient engagement over the life cycle of new medicines, including patient engagement with HTA and payer agencies.[12] These initiatives and proposals offer examples that bear further examination and research, by PCORI and others, for potential adaptation to the decentralized landscape of U.S. health care payment.

Limited opportunities for communication before FDA approval of a novel therapy

Health care payers generally plan up to 18 months in advance of a health insurance plan year. As part of the planning process they estimate the potential financial impact of new therapies that may become available during the plan year. It is standard practice among health insurers (public and private), prescription benefit managers, and health benefit consultants to conduct 'horizon scan' or 'drug pipeline reviews' exercises to identify new pharmaceuticals that could receive FDA approval before or during a health insurance plan year. Here again, pharmaceutical manufacturers have the prerogative to deposit information on unapproved drugs in depositories available to multiple manufacturers, subject to FDA regulation that limits on claims made by the manufacturers. As of now, patient groups do not enjoy a similar capability to deposit information in a dossier-like format for the benefit of multiple payers. Patient groups with sufficient resources can and do publish reports and studies that are accessible to payers and may inform payers' pre-approval assessment of drugs awaiting approval. However, there are few formal processes in place that bring patient representation directly into the horizon scanning or pipeline review processes that payers rely on for the advance planning that influences decisions made later on coverage of newly approved novel therapies.

Conclusion

Despite an increasing focus on patient engagement and patient-focused outcomes in novel drug development, there is still little in the way of systematic communication between patient communities and health care payers when payers are faced with imminent decisions on formulary inclusion, utilization management, patient cost-sharing, and related issues such as design and execution of value-based arrangements. Trends in drug development and in the health care marketplace may bring more systematic communication among patients and payers to the fore in years to come, but for now communication remains somewhat haphazard. Significant barriers to effective and trustworthy communication remain to be overcome. These include barriers, real or imagined, that are created by a lack of trust and transparency among a patient community and health care payers when a novel therapy is launched. The principles of communication outlined in this paper are designed as a guide for better, more effective, and trustworthy communication among patients and payers as our health care system evolves towards what we hope will be more systematic communication and collaboration among patients and payers in the future.

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Endnotes

1 In this summary and in all materials prepared for this project, and unless otherwise specified, the term "patients" is used to refer to individual patients, patients' family members and caregivers, organized patient groups and other groups directly representative of patients with a condition that is now or is likely to be treated with a novel therapy. The term "payers" is used to denote health insurers, prescription benefit managers (PBMs,) end-purchasers such as self-insured employers, and other professionals or organizations that support payer decision-making on coverage of novel therapies, such as benefit consultants, specialty pharmacies, and others.

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