NEHI Network for Excellence in Health Innovation

Patients, Payers and Novel Therapies:
Envisioning Good Communication Principles and Practices

(*The views expressed below, while solely those of NEHI staff, are based on insights from the NEHI project on "Developing Principles of Communication Between Patient Communities and Payers Regarding Novel Therapies." A more detailed statement is in development for discussion at the NEHI Conference on Novel Therapies and Patient-Payer Communication, July 27-28, 2021)*

FDA approval of Aduhelm, the new treatment for Alzheimer's Disease, provoked swift reactions, among them the fear that desperate families facing the heavy burden of Alzheimer's will now be pitted against health care payers under constant pressure to keep health insurance affordable. Whatever one thinks of the merits of the FDA's decision, it sheds new light on communication regarding new and novel therapies between patient communities (individual patients, their families, and the groups that represent them) and the nation's vast labyrinth of health care payers and the organizations that support them (insurers, prescription benefit managers, self-insured employers, benefit consultants, government health programs and others).

Patient advocates communicate their views on policy to policymakers and payers frequently. The arrival of highly novel new therapies (such as gene therapies, immunotherapies, and RNA-based therapies) has, if anything, raised the policy stakes even higher on outstanding issues such as the limits of utilization management by payers, and the use of health technology assessment (HTA) in drug pricing. This is communication that cuts across many disease states and new therapies.

Meanwhile, patients and clinicians communicate with payers every day about specific therapies and the needs of individual patients; for example, communication between patients and their clinicians with payers regarding requests for prior authorizations or appealing denials of coverage. This is communication that takes place well after the payer has set the terms and conditions of coverage for a new therapy. It is formal and structured, and some aspects of this case-by-case communication may be regulated by state and federal government as a form of consumer protection.

A third type of communication now falls between policy advocacy and the appeals of individual patients. This is communication that occurs when a new and novel therapy is at or nearing FDA approval, but health care payers have not yet made decisions on whether to cover the therapy or set terms for coverage such as prior authorization or step therapy requirements.

The need and the demand for this kind of communication will likely increase. These days dozens of novel therapies are approved every year to treat serious unmet and life-threatening conditions. Some new and novel therapies arrive with high initial costs that trigger close scrutiny by the payers just when a patient community feels the most urgency to gain access to the therapy and minimize the time to initiation of therapy, particularly therapy for a degenerative or rapidly progressing condition. In addition, payers see an uncertain base of evidence supporting use of some new therapies, often attributed to accelerated FDA reviews. In some cases patients and patient groups are among the most knowledgeable and sophisticated experts, not only on account of their experience with the disease in question, but on

new therapies as well, having served as active partners in basic research, clinical trial recruitment and even venture funding of drug development. The question becomes: how do patient communities and payers exchange evidence and expert information in an orderly and constructive way, and how can communication triggered by dozens of new drug approvals every year be managed across the labyrinth of health care payers? Can this lead to stronger, trustworthy communication among patient communities and payers, and avert painful disputes between the two?

In research and interviews conducted by NEHI to date we can see barriers to improved communication among patient communities and payers in this period when novel therapies are at or nearing approval, but before payer coverage decisions are final. We see two sets of needs: a need for principles of good, two-way communication to which both patient communities and payers can aspire, and a need for new or expanded channels of communication between patient communities and payers.

Four potential pillars of good, two-way communication stand out:

Transparency: Patient groups that gain accreditation and maintain well-regarded standards for regulating conflicts of interest and disclosure of funding sources enhance their credibility with payers. (The National Health Council standards are an example.) Patient groups lacking resources for accreditation can still emulate high standards of practice. Payers can enhance trust with patient communities by transparency in decision-making, including transparency as regards the criteria for coverage decisions and the evidence on which decisions are made. (The recent ICER "Cornerstones of Fair Patient Access" framework summarizes standards offered by several sources.)

Representativeness: Patient communities and payers should be able to discover a strong, mutual interest in understanding the different responses that patients may experience from a new and novel therapy (the so-called heterogeneity of treatment effect). Good faith communication will center on the need to understand evidence regarding heterogeneity and collaborate on filling gaps in evidence. A patient group not representative of the range of diversity within a patient community can seek out alliances with other groups that focus on under-represented patients. Gaps may be particularly evident relative to patients traditionally under-represented in pre-approval clinical trials (such as women, Black and Latino Americans), and in patients whose health outcomes are heavily influenced by social determinants of health.

Shared focus on evidence: As noted, payers question the strength of evidence supporting use of some new and novel therapies. Meanwhile some patient groups may be essential experts on their disease state and on the effectiveness of the therapy in question. The expertise of patients and caregivers may be especially important regarding the impact of a therapy on patient outcomes that may be under-reported or unstudied in clinical trials, such as quality of life or functional status outcomes. Patient communities and payers should be willing to collaborate on their mutual interest in ascertaining the long-term effectiveness and durability of a novel therapy, including collaboration to monitor results from real world use of the therapy.

Timeliness: Health care payers adhere to strict decision-making cycles that support yearly health insurance plan enrollments for most U.S. patients. A cycle of decisions on a novel therapy may begin as early as 18 months or more before a plan year starts. Few formal processes are in place for early, pre-approval communication among patient groups and payers, but even early, informal information

exchange may save time in the payer decision-making process and create a trusting relationship for further communication.

New or expanded channels of communication are still needed

Aspirational goals will likely have a limited impact without innovative communication practices and reliable channels of communication among patient groups and health care payers. We see at least three major challenges that must be overcome:

Simplifying patient community engagement with the highly fragmented health care payment system: In the U.S. health care system thousands of organizations make decisions on novel therapy coverage on behalf of an even larger number of health care purchasers and individual health insurance subscribers. Even the most sophisticated patient groups may need to knock on hundreds of payer doors. Efforts to create reliable, predictable ways (or "common points of entry" to the payment system) through which patient groups can communicate with the largest possible payer audience will enhance communication. This is a role that umbrella patient organizations, payer trade associations and some professional societies may be well positioned to play.

Operationalizing patient engagement with payer decision-making: Pragmatic approaches are needed for patient engagement with payer decision-making processes. For example, how can payers best engage with patient groups when decisions are made in the pharmacy and therapeutic (P&T) review process, the utilization management process, or in the design and execution of payer-manufacturer contracting (such as value-based contracts) that generate evidence on the effectiveness of therapy in real world practice?

Assuring timely patient engagement with payer decision-making: Patient-payer communication will be enhanced if patient groups can address the largest potential payer audiences, and if communication occurs early or at points in time that mesh with payers' decision-making schedules. Innovative approaches are needed in which payers can solicit earlier patient engagement, perhaps including patient community engagement with payers' pharmaceutical horizon scanning and drug pipeline review processes. Some foreign health systems solicit patient engagement through national health technology assessment (HTA) programs. Lessons learned from these programs will need to be adapted to the U.S. health care system where there is no national HTA process at this time.

Engagement between patient communities and payers, while not exactly a new relationship, is still an undeveloped relationship in the U.S. health care system. It is a complicated relationship, given the great complexity in the health care payment system and the great diversity of patient organizations seeking representation. Recognition by all parties of basic ground rules for communication, even if aspirational, can help guide conversations among patient payers, and spark steps to create reliable channels of two-way communication among patient communities and health care payers.