

Patient Value Is the Root of a Learning Health System

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For more than a decade, a plethora of policy and research activities have been undertaken to facilitate the transition of the US health care system away from a fee-for-service model to one rooted in value. Creating and sustaining a learning health system has been a consistent refrain from an assortment of entities including the Agency for Healthcare Research and Quality, Patient-Centered Outcomes Research Institute (PCORI), FDA, National Quality Forum, Institute for Healthcare Improvement, payer and provider task forces, and many academic centers.¹ The National Academy of Medicine defined a learning health care system as “one in which science, informatics, incentives, and culture are aligned for continuous improvement, innovation, and equity—with best practices and discovery seamlessly embedded in the delivery process, individuals and families as active participants in all elements, and new knowledge generated as an integral by-product of the delivery experience.”²

This is a lofty vision and, sadly, one that remains just beyond our reach. Although there are myriad examples of good intention, disruptive thinking, and even emerging promising practices, we can't seem to achieve systemic and sustained change. Why?

As both an observer and a participant, I posit 2 answers. First, patients are still often outside of the discussions critical to identifying the problems that a learning health system should be solving. This includes the ongoing debate about patient-defined end points for clinical development and research, the lack of data collection that reflects the real-life diversity of patients and their experiences, and patients' absence from discussions about the use of such data for decisions that directly affect access to care.

Second, the incentives for achieving a delivery and payment system that leverages data, technology, and policy for equitable outcomes are currently misaligned. An oft-noted reason

for this misalignment is complexity. Indeed, the labyrinthine issues of data access and ownership, legal and regulatory requirements, and infrastructure and resource needs prevent us from finding a way forward.

As the executive director of the Innovation and Value Initiative (IVI),³ I see how the goals of value-based decision-making and continuous, data-driven learning are central to both our principles⁴ and the promise of a learning health system. IVI is a learning lab within the value and health technology assessment community, grounded in the principles⁴ of patient-centricity, transparency, and open-source modeling to improve how we measure and talk about value in health care. We envision, and invest in, a value assessment environment in which the concerns of patients are paramount factors in evaluating benefits, risks, and costs.

To move toward a truly patient-centered learning health system, we all need to address the complexities head on. Data transparency and common-good use must be a greater priority. Solutions that facilitate exchange, standardization, and wider use of real-world data sources across decision contexts must be developed with patients and patient communities in leadership roles. Greater focus on the science and practice of authentic patient engagement by all actors must be upheld via policy and funding requirements for research and value-based reimbursement.

Fortunately, there is evolving dialogue and authentically patient-cocreated initiatives that I believe can transform mere words into community-level action and real change. For example, PCORI continues to invest considerable resources in research on patient-centered outcomes and their translation into practice.⁵ Its expanded authority to identify and compare financial and other impacts on patients, families, and caregivers will drive essential attention and investment into real-world data development and use of such information in clinical and policy decision-making.

The FDA continues to prioritize patient inputs to drug development and to be responsive to patient communities that are leading key initiatives that improve definition of biomarkers, clinical end points, and outcomes for use in both regulatory review and postmarket economic evaluation and coverage decision-making.⁶ Additionally, many patients and patient organizations across the spectrum of diseases continue their often-undervalued leadership in driving clinical research, investing in data development through registries and other research initiatives, and educating payers and policy makers about the impact of their decisions on equitable access, health, and quality of life.

The elements of a fully realized and effective learning health system in many ways parallel those that IVI envisions as essential building blocks of patient-centered value assessment:

1. **Patient leadership and inclusion.** Patients must be active and visible leaders in this learning process and meaningfully engaged at every step in the process. What do patients/families/caregivers prioritize in their health care? How are those concerns addressed in terms of research agendas and investments? How are relevant data collected and analyzed? How is learning from such data shared back with patients and patient communities for continuous learning?
2. **Grassroots engagement.** In order for the health systems engaged in research projects to be successful, their diverse local communities need to be involved and engaged. Improving patient representation in research at every stage in health care innovation, translation, and value assessment is key to meaningful change.
3. **Metrics and measurement must be redefined.** From my own experience in value assessment, it is not always clear that researchers are measuring what is meaningful to patients. And although data that are not strictly clinical in nature may be difficult to ascertain and to measure, we bear responsibility for sharpening our measurement tools because what can't be measured can't be improved.
4. **Transparency: open data, open models, open resources.** Despite the proprietary nature of health systems in the United States, a level of transparency must be maintained if the goal of a learning health system is to be realized. In order for new knowledge and methods to be disseminated and acted upon, mechanisms must be in place for this information to be shared.
5. **Collaboration.** The continuous learning concept is predicated on collaboration,⁷ so research projects must be developed in ways that mandate it. Funders of such research, both government and philanthropic,

must incentivize collaboration in the work that they support.

As if complexity isn't challenging enough, a critical issue in implementing these 5 principles is accountability: Who is responsible for establishing the standards and ensuring that all stakeholders—not just a few—take action to create sustainable change? Authentic progress toward a learning health system requires real investment and accountability to create incentives for change.

Here are a few ideas about strengthening accountability for a patient-driven learning health system:

- Research funders—including government, quasigovernment, and philanthropic organizations—can require that every research proposal, performance metric, outcome measure, or quality measure provide evidence that it answers questions⁸ that are important to actual patients, families, and/or caregivers.
- Similarly, such research funding can be made contingent on evidence of patient leadership in the creation, design, and/or coinvestigation of key questions. PCORI patient engagement research, FDA investment in patient-focused drug development guidance, and National Health Council resources on capacity building are but a few resources to support such a requirement.
- Scientific and professional journals can require that scientific publication submissions include evidence of patient involvement in the design and analysis of research. Doing so raises the bar for acceptable collaboration and study design and establishes expectations for transparency of such inclusion.
- Funders can require open access to published research so that patients and patient communities can review and benefit from such evidence.
- Employers⁹ can ask questions during their contracting for payer, pharmacy benefit manager, and other health advisory services that emphasize inclusion of data reflecting the patient lived experience, as well as processes that reflect patient/family/caregiver involvement in benefit design and access policy development.
- Health systems can incorporate the collection of patient goals as part of care delivery and as an essential element across any electronic health record/electronic medical record platforms. Likewise, vendors¹⁰ can innovate to improve capture of patient-generated health data to improve the real-world evidence ecology.

The 800-pound gorilla in any room in which these advances are to occur, of course, is the fact that our current financial ecosystem, from research through plan development and care delivery, generally lacks the mechanisms to incentivize these changes. Let's be honest: Systems change and navigation of

complexity are effort and resource intensive, and without meaningful incentives, people and organizations may be less likely to undertake these efforts based simply on the desire to “do the right thing.” Opportunities, however, do currently exist: Foundations and other funders of research and program development have flexibility to change requirements for funding, for example, and employers have market power in choosing their benefits consultants and vendors. Nonetheless, much broader shifts in funding and reimbursement are necessary, and they will take time. It is essential that, in addition to shifting awareness of and focus on the conceptual changes outlined above, we as a system continually look for opportunities to redirect the flow of funding to fuel change.

The path forward is challenging, but we are at a crossroads moment. A renewed commitment to patient-centered decision-making and evidence generation, combined with a focus on value in quality and payment reform circles, can accelerate our progress toward a true and sustained learning health system. Moving beyond academic concepts requires not only identifying emerging and promising practices in achieving improved care, but also establishing clear expectations for transparency, data sharing, and investment in research defined with equity and patient-defined priorities in mind.

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