Advancing Data Capacity for Economic Outcomes in Patient-Centered Outcomes Research

Challenges and Opportunities

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The economic impacts of health care treatments and services on individuals and their families are central to many decisions people make about the use of health care. However, without the high-quality data needed to generate evidence on the clinical effectiveness and economic impacts of an intervention, decision-makers are generally limited in their ability to make informed health care decisions that reflect patient values and preferences. The importance of evidence on economic impacts, including nonmedical costs and work-related impacts, was recognized in the reauthorization of the Patient-centered Outcomes Research Trust Fund,[1] which added economic outcomes to the range of outcomes that should be considered as part of patient-centered outcomes research (PCOR).

The Office of the Assistant Secretary for Planning and Evaluation convened a symposium[2] that brought together patients, researchers, federal agency representatives, and other stakeholders to discuss research on the collection, linkage, and analysis of economic outcomes data for PCOR studies and provide a forum for discussing progress, challenges, and opportunities in this emerging area. In this paper, we synthesize cross-cutting considerations to guide efforts to build data capacity that emerged from discussions at the symposium. We then highlight initial opportunities to expand the availability and use of relevant, high-quality economic outcomes data as part of evidence generation in patient-centered outcomes research.

BUILDING DATA CAPACITY FOR ECONOMIC OUTCOMES IN PATIENT-CENTERED OUTCOMES RESEARCH STUDIES: GUIDING CONSIDERATIONS

Across panel discussions, symposium participants converged on 4 overarching considerations to guide efforts to build data capacity for PCOR studies with economic outcomes. Although not unique to economic outcomes data and distinct from principles that others have developed for the design and conduct of PCOR studies in this area,[3] these guiding considerations consolidate stakeholder views on how to improve our understanding of the economic factors facing individuals and their families.

1. Measurement and data collection for economic outcomes should enable a comprehensive, person-centered, and longitudinal understanding of health and other impacts. Measuring outcomes that are important to patients and families is critical for ensuring that PCOR studies generate

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the evidence needed to support informed decisions; incorporate patient values, preferences, and differences; and support patients' health and well-being. Longitudinal assessments are particularly important for understanding economic impacts across the course of treatment and life course trajectory or as an individual's or family's social or economic conditions change.
2. Research data on economic outcomes should be high-quality, accessible, timely, interoperable, and linkable. Economic data are often available in diverse data sources in different formats, requiring significant resources and specialized expertise to access, link, and analyze. Technical, cost, and administrative barriers should be lowered while preserving privacy, confidentiality, and data security safeguards to support participation in and advance research.
3. Collaborative partnerships across stakeholder groups are essential for aligning, advancing, and sustaining efforts to improve the measurement and use of economic outcomes in PCOR. Partnerships between patients and patient organizations, data stewards, and decision-makers can ensure that measurement priorities are aligned with the needs of patients, families, and communities; are sustainable and adaptable to stakeholders' needs; and help to disseminate and implement findings of PCOR studies. In addition, these partnerships may help to address patient and family concerns in providing or consenting to data use and establish a culture of trust among patients, caregivers, researchers, and other stakeholders.
4. Economic outcomes in PCOR studies should be defined, collected, and analyzed in ways that advance health equity. Participation of patients and patient organizations reflecting diverse socioeconomic and cultural perspectives and lived experiences in the development of economic outcome measures will facilitate the capture of high-quality economic data and support research, policy, and decision-making in ways that address health disparities and improve health equity.

The foundational principle underlying these considerations is the importance of collecting, linking, and analyzing data in ways that align with patients' values and priorities while recognizing and respecting heterogeneity in patient preferences. This also requires understanding the evidentiary needs of multiple stakeholder groups, including patients, providers, payers, and policymakers, and the best approaches for addressing those needs. Informed decision-making at the patient, system, and policy levels is hindered when the data needed to generate the relevant evidence are missing (including not reflecting economic impacts that are most important to patients), when studies are not designed to generate that evidence, and when the evidence is not disseminated in a manner that meets evidentiary needs.

OPPORTUNITIES TO BUILD DATA CAPACITY FOR ECONOMIC OUTCOMES IN PATIENT-CENTERED OUTCOMES RESEARCH

With these guiding considerations in mind, we explore opportunities to build data capacity for economic outcomes in patient-centered outcomes research in 4 domains: measurement, data collection, data linkage, and data access and use. Within each domain, we highlight opportunities for improvements that are expected to be particularly impactful at this early stage. This initial exploration provides the groundwork for further discussion and development (and implementation, where feasible) of efforts to advance data capacity in this area.

Measurement

Existing economic outcome measures and measure development priorities may not be aligned with the evidentiary needs of all decision-makers, especially patients, families, and caregivers. As a result, data collected and available for research and analysis may not provide the evidence needed to inform decisions and improve health outcomes. Robust and equitable stakeholder engagement is needed to identify and prioritize the types of decisions that could be supported by PCOR studies and the types of data on economic outcomes needed to support this research.

Identify Existing Economic Outcome Measures and Approaches That Could Be Refined to Better Capture Patient Priorities

Refinements to existing measures might include adding or revising items included in financial hardship scales,[4] expanding efforts to validate measures with diverse populations, and, if appropriate, developing new measures. Examples of populations that might be prioritized for these efforts include people with disabilities and special needs (eg, individuals with intellectual and developmental disorders and their families), those with high health care involvement (eg, patients with multiple chronic illnesses), low-income and uninsured individuals, residents of rural areas, and other populations with known health disparities and history of exclusion from decision processes (eg, people of color, including Black and African American patients, American Indian/Indigenous American patients).

Advance Methods for Measuring Household and Family-level Economic Outcomes

The economic burden of health care can reach beyond the patient and impact the finances, health, and overall well-being of an entire household or family. For example, billing data might show that a patient has been consistently refilling a medication on-time, suggesting that the medication is affordable for the patient, but might not reveal that the patient has been unable to buy food for the family or heat the family's home during the winter. Although approaches to defining households have been developed for use in other fields, they may not capture the relevant relationships to address the questions of interest in PCOR studies, limiting the understanding of, and ability to address, adverse impacts on caregivers and families. Researchers and patient organizations could explore opportunities to develop replicable approaches for defining households and families in different use cases and explore their measurement needs. Exploring methods to add household-level measures to current data sources or use address information to add household identifiers to these sources could help generate economic measures that may be more predictive of health and financial impacts than individual-level measures in some contexts.

Develop Standardized Economic Outcome Measure Sets

When robust measures exist, a standardized set of "core" economic outcomes and contextual measures could be captured in ongoing surveys, patient registries, medical billing claims, and electronic health records to facilitate comparisons across studies, populations, and settings.[5] Examples of core measures might include days missed from work, changes in job status, productivity changes, delayed or avoided health care visits or medications due to cost, difficulty paying bills, and enrollment in financial assistance programs.

Data Collection

Missing data, small sample sizes, differences in variable definitions, and other limitations of current economic data can make it difficult to assess important economic outcomes in PCOR studies or to assess impacts for specific populations, such as uninsured individuals. Improving the collection of relevant, timely, and high-quality data on economic outcomes will require the commitment of several key stakeholder groups to address data gaps by adding and standardizing data elements, combining data, or devising innovative and low-burden data collection approaches. Opportunities to align the collection of these economic data within research networks that bring together electronic health records, claims, and registry data covering large populations could support efficient and impactful PCOR studies on large populations.

Expand Capture of Out-of-pocket Spending

Given the importance of direct medical costs to patients, such as out-of-pocket spending, organizations that aggregate claims data could take steps to add information that is needed to estimate these costs. In particular, adding information, such as plan benefit design and cost-sharing features or the actuarial value of plans, which has been recommended previously,[6] would facilitate research on a number of topics, such as the impact of different benefit designs and the development of algorithms to predict difficulty adhering to treatment due to a patient's out-of-pocket cost burden. In addition, systematic capture of a wide range of nonmedical costs, including travel, lodging, and child care or other costs that families routinely face when patients are referred to regional centers for procedures or when they need additional care at home after hospital stays—would provide a more comprehensive measure of out-of-pocket spending.

Address Population Gaps in All-payer Claims Databases

All-Payer Claims Databases (APCDs) are potentially useful resources for measuring the direct medical cost of treatments and out-of-pocket spending. However, APCDs have well-known gaps, such as excluding data on uninsured individuals and, in most cases, employees of large firms that self-insure (which is the majority of workers covered by employer-sponsored insurance), as well as individuals who move across state lines. APCDs could become more powerful through efforts to create multi-state APCDs[6] and new partnerships with safety net providers who could contribute data on their uninsured populations to advance research that improves health equity.

Expand Data Collection on Work-related Outcomes

Given the importance of employment as a source of health insurance for many Americans and the importance of work in maintaining access to health care and household income, expanding the capture of work outcomes can significantly enhance the ability of PCOR studies to assess the economic impacts of treatments, services, and policies. Routinely capturing work and work-related disability status during health care encounters can help to provide a snapshot of work outcomes, alongside the development of other approaches for data capture that do not rely on visits to health care providers. In addition, capturing the impact on work for informal caregivers and household members will contribute to the understanding of the economic impacts of health care on families more broadly.

Develop Innovative Patient-driven Data Collection Approaches

Patients and organizations representing their interests could close data gaps by developing or expanding existing data collection platforms to capture high-quality data on economic outcomes that could be linked, aggregated, and shared in ways that maintain privacy and security. Mobile health platforms can efficiently collect patient-generated data on clinical outcomes and care experiences[7] and could be leveraged to capture economic outcomes, such as employment status, days missed from work, caregiving, and financial hardships.

Data Linkage

Privacy, security, and governance issues often limit the linkage of some data sources commonly needed to assess economic outcomes in PCOR studies. Improving existing methods and developing new linkage approaches to ensure privacy, security, and data governance would facilitate PCOR studies on economic outcomes. Expanding linkages among traditionally siloed data sources, such as data related to housing and nutritional assistance and health care claims data, would facilitate the exploration of a wider range of research questions while efficiently leveraging data that have already been collected. Both technical and nontechnical resources could be expanded to support linkages and increase the utility of economic data available for PCOR studies.

Develop Governance Frameworks That Balance Access and Privacy

Collection and linkage of detailed patient-level data require appropriate privacy and data security safeguards. Data stewards could develop governance frameworks, standardized data use agreements, and consensus methods (eg, privacy-preserving record linkage) to build trust between parties involved in data linkages and expedite administrative and technical processes. Collaborations between patient groups, data stewards, and researchers could help to identify use cases in which data linkages would generate unique benefits for patient communities, such as using clinical, claims, patient-reported outcomes, and social services data to better understand the relationship between the receipt of food assistance, decision-making related to treatment, and outcomes. Developing such use cases may also facilitate engagement from patients as partners and/or advocates who see the utility of linked data to answer questions of most interest to stakeholders.

Create Synthetic Datasets to Expand Access to Individual-level Data

Synthetic datasets,[8] which allow researchers to use simulated data to maintain the properties of the underlying data while masking sensitive data elements, offer a promising approach to minimize threats to patient privacy while allowing analyses that produce informative findings. Examples include the Agency for Healthcare Research and Quality's Synthetic Healthcare Database for Research,[9] which has been used previously to measure health care utilization but includes limited measures of economic outcomes.

Develop Best Practices for Data Linkage

Selection and implementation of methods for data linkage, which could include deterministic or probabilistic methods, should be informed by the relevant strengths and weaknesses of the approaches with respect to the datasets and goals of the linkage (eg, individuals lacking social security numbers, limited numbers of matching variables, or variables measured with error). In addition to potentially introducing (new) errors, data linkages can propagate issues from the source datasets, both of which can affect the utility of the resulting linked datasets. Both the quality of the original datasets and the resulting linked datasets should be carefully evaluated for their suitability for use in PCOR studies.

Data Access and Use

In addition to the privacy concerns noted above, resource barriers often limit the ability of researchers to access and use data on economic outcomes in PCOR studies. These barriers may especially limit access by researchers from systematically excluded groups—for whom disparities in federal grant funding have been widely reported[10]—as well as their community partners. Leveraging resources and reducing other cost-related and administrative barriers to facilitate access to data on economic outcomes is critical to building a shared research infrastructure as a public good.

Support the Development and Accessibility of Datasets That Include Economic Outcomes as Direct Research Products

Traditional research funding mechanisms often do not support large-scale economic data collection, and they may limit the time horizon for data collection. Funding organizations could develop targeted funding opportunities that have the primary goal of enhancing existing databases or producing new data resources that address important data gaps and that can be shared securely within the PCOR community. Given the particular value of linked datasets for a wide range of stakeholders, ensuring that linked data—especially longitudinal data—are strategically constructed and maintained to support broad research agendas rather than individual projects will reduce the burden on individual research teams, expedite evidence generation, and support both public and private sector needs. In particular, longitudinal, linked federal data could sustain a wide range of research on economic outcomes for high-need populations.

Expand Access to Analytical Resources

To facilitate research and promote standardization, PCOR stakeholders might establish electronic repositories or curate existing repositories that support the sharing of data and code, such as code to conduct linkages or generate specific economic outcome measures, such as out-of-pocket costs. Lowering barriers to accessing technology solutions for data collection or data sharing could also facilitate new research by leveraging patient-generated economic data to improve the quality of linkages as well as the rigor and reproducibility of patient-centered outcomes research over time.

CONCLUSIONS

Symposium participants affirmed the importance of assessing patient-centered economic outcomes, along with clinical and other health-related outcomes, to generate evidence needed for decision-making by patients, providers, payers, and policymakers. An overarching theme of the discussions was the need for a paradigm shift that better aligns the measurement of economic impacts with the priorities identified by patients, families, and caregivers. Data gaps, analytical limitations, ethical considerations, and logistical issues must also be addressed to improve data capacity and ensure that PCOR studies consider the full range of outcomes, as stated in the reauthorizing legislation for the PCOR Trust Fund. These efforts may be initially guided by the considerations that emerged from the symposium and include the key opportunities identified. Continued engagement among stakeholders involved in the production and use of data on patient-centered economic outcomes can help to identify additional priorities for building data capacity and expanding the use of these outcomes in PCOR studies over the next decade.

REFERENCES

1. Further Consolidated Appropriations Act, 2020, Pub. L. No. 116-94, (2019). https://www.congress.gov/116/plaws/publ94/PLAW-116publ94.pdf
2. RAND Corporation. Symposium on Building Data Capacity to Study Economic Outcomes for Patient-Centered Outcomes Research. Accessed March 30, 2023. https://www.rand.org/health-care/projects/pcor/hhs-symposium.html
3. Patient-Centered Outcomes Research Institute. Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research. Accessed May 1, 2023, https://www.pcori.org/research/about-our-research/patient-centered-economic-outcomes/principles-consideration-full-range-outcomes-data-pcori-funded-research
4. Smith GL, Banegas MP, Acquati C, et al. Navigating financial toxicity in patients with cancer: a multidisciplinary management approach. *CA Cancer J Clin*. 2022;72:437–453.
5. The Gravity Project. Initial Social Risk Data Set Specification: Medical Cost Burden. Accessed May 1, 2023. https://confluence.hl7.org/display/GRAV/Medical+Cost+Burden
6. State All Payer Claims Databases Advisory Committee. *State All Payer Claims Databases Advisory Committee Report With Recommendations Under Section 735 of the Employee Retirement Income Security Act of*

1974. Undated. Accessed July 31, 2023. https://www.dol.gov/sites/dolgov/files/ebsa/about-ebsa/about-us/state-all-payer-claims-databases-advisory-committee/final-report-and-recommendations-2021.pdf

7. Wyner Z, Dublin S, Reynolds J, et al *Collection of Patient-Provided Information Through a Mobile Device Application for Use in Comparative Effectiveness and Drug Safety Research*. 2018. Accessed March 30, 2023. https://aspe.hhs.gov/sites/default/files/private/pdf/259016/FDAMyStudiesAppAPatientCenteredOutcomes.pdf
8. Gonzales A, Guruswamy G, Smith SR. Synthetic data in health care: a narrative review. *PLOS Digit Health*. 2023;2:e0000082.
9. Agency for Healthcare Research and Quality. Synthetic Healthcare Database for Research (SyH-DR). Accessed March, 2023. https://www.ahrq.gov/data/innovations/syh-dr.html
10. Hoppe TA, Litovitz A, Willis KA, et al. Topic choice contributes to the lower rate of NIH awards to African-American/black scientists. *Sci Adv*. 2019;5:eaaw7238.