TuftsMedicine
Tufts Medical Center

NEWDIGS

in collaboration with

H2O HEALTH OUTCOMES OBSERVATORY

SPECIAL EVENT BRIEFING

How an RWE Infrastructure Could Enable Payment Innovation

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CONFIDENTIAL

Tufts Medical Center Institute for Clinical Research and Health Policy Studies
Center for Biomedical System Design

Background

Key Messages

- Improving our ability to track outcomes in ways that improve decision-making for all stakeholders is critical for improving healthcare quality and enabling value-based payment innovation for biomedical products.
- Emerging outcomes tracking platforms could offer a new infrastructure for supporting value-based payment innovation, but they currently fail to meet the needs of payers.
- Creating a "Payer-Ready Outcomes Tracking Framework" designed with input from all stakeholders could accelerate progress toward the RWE platforms needed to support value-based payment models.

Increasing concerns over the sustainability of health care systems are fueling efforts to better incentivize value received by patients in terms of improved health outcomes, rather than the volume of services or products delivered. Value-based payment innovation requires the ability to track clinical outcomes in ways that are efficient, timely and fit-for-purpose for decision-making by key stakeholders. While exciting advancements are emerging in outcomes tracking, so far they do not meet the needs of payers to design and implement scalable value-based payment models for biomedical products.

The Tufts Center for Biomedical System Design's NEWDIGS consortium and the Innovative Medicine Initiative's Health Outcomes Observatory (H2O) are co-hosting a special event on June 13 to elucidate the challenges and actionable opportunities for accelerating "Payer-Ready Outcomes Tracking Platforms" to enable value-based payment innovation.

The underlying challenge

Value-based payment innovations align payment to the value received rather than the volume of use. This shift from volume to value is gathering momentum globally, driven in large part by concerns over the sustainability of rising health care costs and the need to take full advantage of personalized and targeted therapies.[1] Innovative payment strategies can also mitigate risks associated with short-term uncertainties regarding the actual net benefits received by treated patients, the numbers of those patients, and the financial reimbursement made for that treatment within a particular payer's population.[2]

Value-based payment innovations require patient outcomes data, possibly down to the level of individual patients. Real-world evidence (RWE) outcomes tracking initiatives, examples of which will be discussed, are often designed to address a specific clinical or research need, but fall short in terms of being fit-for-purpose for innovative payment models. Multistakeholder design work within the NEWDIGS consortium has identified criteria necessary for credible and actionable evidence to support value-based contracts[3] and an approach to defining the associated outcome metrics.[4]

The criteria developed include:

- **Meaningful:** should matter to patients, clinicians, regulators, and other stakeholders and strongly correlate to treatment effectiveness for the treated patients in the payer's population

- **Measurable:** should be practical to collect (ideally as part of routine care), consistently available, and offer clear and unambiguous results
- **Timely:** outcomes should be likely to happen during a reasonable contract duration, and data should be available without undue delay (e.g., quarterly, monthly, or even real-time)
- **Robust:** resilient to uneven clinical execution, and not sensitive to potential sources of bias such as patient selection, interpretation and availability of test results, channeling, and gaming
- **Accessible:** available to both contractual parties at no or low cost, and able to be interpreted
- **Predictable:** should support estimates of expected success rates and expected variation in success rates so that parties are able to make informed decisions about risks and rewards.

Additional metrics criteria might encompass considerations such as inclusiveness – sensitivity to vulnerable populations, low digital literacy, and parity of impacts; patient participation and acceptance; and comprehensiveness, in terms of including all necessary information for decision-makers.

Barriers

The operational challenges to tracking payer-ready outcomes are substantial, including:

Feasibility

In many cases, it is not currently possible or practical to systematically track the desired metrics. For example, clinician time pressures preclude some data entry, and we lack suitable technology platforms to enhance efficiency; appropriate metrics may not exist in RWE sources based on administrative (claims) data; or there may be insufficient medical follow-up. Additionally, the importance of patient-reported outcomes (PROs) and patient generated (sensor) data in driving the "value" in value-based care is increasingly recognized; however, PROs are missing from most typical RWE sources. Co-creation is an essential step in metrics development but can be time-consuming.

Fragmentation

Fragmentation in global healthcare systems inhibits the tracking of outcomes data that is "payer-ready". Interoperability standards have been slow to be universally adopted despite many standardization initiatives. In both the US and Europe, care fragmentation translates to many points of potential data collection: in primary care, hospitals, specialist physician offices, rehabilitation centers, nursing facilities, and more.

Fragmentation in the US is further compounded by:

- Multiple public and private payer systems in each state (Medicaid/Managed Medicaid, ACA, Commercial fully insured, Self-insured Employer, Traditional Medicare, Medicare Advantage).
- Providers generally treat patients covered by a multitude of insurance products and payers.
- Medications may be pharmacy dispensed, physician administered, or hospital administered, creating another layer of fragmentation for drug management and budget tracking.
- "Patient mobility" related to switching from one insurance plan to another every few years, a function of coverage being tied to jobs.

The scale of geographic fragmentation (states/nations) differs between the EU and US. In Europe, the highly federated governance and administration is a major driver of fragmentation.

Privacy and Security Concerns

Value-based agreements require the tracking of individual patient-level data which are generally not publicly available due to data privacy laws. Privacy protections differ between the US and EU, but both can present thorny issues for value-based contracts. At present, the Health Insurance Portability and Accountability Act (HIPAA) in US allows all business partners engaged in payment processes to have access to patient level data. That said, healthcare organizations must abide by a "minimum necessary" rule in order to avoid a violation of protected health information (PHI) in the data sharing process.

In the EU, compliance with the General Data Protection Regulation (GDPR) requires special risk management of data sharing and protection with safeguards in place to govern both access and formatting of the data shared. For instance, under the GDPR patients may withdraw consent for data sharing, necessitating a mechanism for withdrawing their data from holder files.[5] Also, secondary use of data requires appropriate patient consent and mechanisms to ensure patients are being kept informed of research queries into their data. A number of existing technologies, in particular around pseudonymization, or replacement of personal identifiers with placeholder values, increasingly allows analysts to address these issues.

Incentives

In theory, improving health outcomes should be the first and foremost aim of data collection efforts, and sufficient unto itself. Realistically, however, there are a number of challenges to payer-ready outcomes tracking related to incentives of the various parties – both financial and non-financial.

Commercial Value of Data: In the US, the data being tracked itself has financial value, and those tracking the outcomes (providers, payers, developers, and/or patients) may not want to share it free of charge. This is especially true if it will be used to assess their performance. In Europe, the situation is more complex for commercial entities. There are ethical concerns about monetization of health data whilst at the same time there is recognition of the need for appropriate investments in infrastructure to collect outcome data. As a result, there is insufficient legal security for viable commercial models.

Resistance to change: introducing outcomes-based payment models in healthcare requires mindset and management change and significant investments. The parties who need to make the investment (providers or payers) may not be the parties who benefit from this the most in the short run. The status quo is a powerful force when stakeholders are faced with uncertainties. Instead of investing more on tracking the right outcomes, payers could just increase premiums; likewise, providers could stay status quo to maintain the practice economics. The benefits of outcomes-based payment models in the long run are probably obvious to all, but the pressures of 'today' create powerful brakes to change.

Competitive Value of Data/Evidence: There are often concerns driven by a lack of trust among the parties that the sharing of the outcomes tracked threatens competitive advantages of providers, payers, and developers.

Willingness to Collect Data: If providers and patients are not motivated to collect data, the quality of the outcomes tracked will be compromised. For example, patient engagement in data collection will be needed for tracking PROs. Initial experience from the Innovative

Medicine Initiative's H2O observatories suggest that patients are often willing to contribute their time to reap the benefits of better information and improved care. The Center for International Blood & Marrow Transplant Research (CIBMTR) similarly offers an example of effective non-financial incentives that motivate providers to collect data for outcomes tracking when, in addition to its value for research, it also meets administrative reporting needs for quality and credentialing of their institution. Further, tradeoffs of time and effort for improved certainty, when the data quality is sufficient to be of value, may be valid for clinicians as well as patients through demonstration of improved outcomes and potentially lower costs.

Lack of a Mandate/Leadership: The barriers to accelerating payer-ready outcomes tracking are substantial. A targeted and systematic effort will be required to address them in a way in ensures success and sustainability for all stakeholders. At present, there is no mandate or leadership in place to drive this within the US or EU. Frameworks for real-world data use, monitoring, and analysis have been defined by regulators for marketing authorization (FDA/EMA), and by HTA organizations for analyzing or projecting product value (EUnetHTA, NICE, ICER) mostly at the product launch. However, these do not address the needs of payers to track the performance (value actually delivered) of individual drugs for individual patients.

Examples of emerging capabilities

While the challenges to "payer-ready" outcomes tracking are significant, a diverse range of emerging capabilities in the marketplace offer hope. Trends reflect important advancements in:

Scalability: Platforms that advance longitudinal tracking from single to multiple products, diseases, and geographies (e.g., H2O, World Federation for Hemophilia/Gene Therapy Registry, and CIBMTR)

Privacy and security: Powerful new pseudonymization technologies emerge that create sufficient safeguards to satisfy regulators' needs. Often they offer access to diverse federated data sources where data is both encrypted and PHI compliant, and support transparency and trust in transactions, while at the same time protecting the intellectual property of any proprietary emerging RWE is protected (e.g., BeeKeeperAI)

Efficiency: Governance models of federated data networks that streamline RWE generation at scale (e.g., FDA's Sentinel Network and National Evaluation System for Health Technology, or NEST)

These capabilities offer exciting potential opportunities for accelerating our progress toward payer-ready outcomes tracking. Several specific examples that bring these capabilities to life are highlighted below and will be discussed on June 13.

Health Outcomes Observatory (H2O)[1,6]
H2O is a strategic partnership between the public and private sectors to create a multinational ecosystem to collect and incorporate patient outcomes into healthcare decision making at an individual and population level.

Region: EU (Tier 1 observatories: Netherlands, Austria, Germany, Spain)

Type: Multiple diseases (pilot areas cancer, inflammatory bowel disease, and diabetes, with plan to expand into other areas)

Approach: Standardized data governance and infrastructure system for patient-reported outcomes and preferences. Empowerment of patients with data.

Payment innovation considerations: sets up infrastructure for health outcomes data collection; governance model for multistakeholder access after multistakeholder approval; hybrid approach with federated elements, but allowing analytic code running on micro-data.

PCORnet®[7,8]
PCORnet® is a national resource, funded by PCORI, where high quality health data, patient partnership, and research expertise deliver fast, trustworthy answers that advance health outcomes.

Region: US

Type: Multiple diseases

Approach: PCORnet is a distributed research network which enables the use of real-world clinical data (primarily from electronic health records) from across 8 Clinical Research Networks for efficient patient-centered research. Non-clinical data sources currently associated with the network include claims data and patient reported outcomes collected by health systems. Previous efforts to integrate complimentary non-clinical data included the Patient Powered Research Networks (PPRNs).

Payment innovation considerations: PCORI's re-authorization mandate in 2019 was extended to include economic outcomes more broadly, but cost-effectiveness and QoL-type metrics were deemed to still be out of scope.

Real Endpoints[9,10]
Real Endpoints Marketplace works collaboratively with industry clients to design, negotiate and implement value-based agreements across multiple payers (regional health plans), aggregating payer lives to make contracts accessible to regional plans.

Region: US

Type: Tracking of individual product contracts across multiple payers

Approach: Claims data

Payment innovation considerations: Real Endpoints Marketplace actively supports value-based agreements through collection of payer data to adjudicate and report value-based agreements. Challenges include limitations of the data (claims data, drug specific, limited to participating payers) and access to data (payers limit use of data to contract-specific purposes)

BeeKeeperAI[11]
BeeKeeperAI's zero-trust platform, EscrowAI, accelerates the validation, training, and deployment of artificial intelligence (AI)/machine learning and analytic models on real-world, privacy protected information (PPI).

Region: US, Canada, Europe, Taiwan, Thailand

Type: EscrowAI enables real-world value-based demonstrations, decision making, and monitoring of PPI impact

Approach: Multi-party, SaaS-based solution maintaining data sovereignty, privacy, security, and the protection of model intellectual property through an automated zero-trust collaboration workflow

Payment innovation considerations: EscrowAI enables and accelerates computing on PPI data, thereby enhancing the precision of value-based decision making

World Federation for Hemophilia Gene Therapy Registry (WFH GTR)[12,13]
The WFH Gene Therapy Registry is a global initiative aimed at collecting long-term data on people with hemophilia who receive gene therapy.

Region: Global

Type: Registry; single disease/multi-product

Approach: Builds off existing research and quality assessment data platform for hemophilia: online web-based system with standardized data (core data set developed by multi-stakeholder steering committee). Hemophilia treatment centers enter data; linkages to existing registries are provided to prevent duplication of data entry on same patient.

Payment innovation considerations: Data collection is expected to commence upon licensure of the first gene therapy for hemophilia. Long-term outcomes will be needed to assess durability of therapy and occurrence of adverse effects; small number of patients dispersed over many countries and regions. Hemophilia treatment centers who do not administer gene therapies may need to be relied on for follow-up.

Center for International Blood & Marrow Transplant Research (CIBMTR)[14]
CIBMTR is a collaborative resource of data and experts supporting research in cellular therapies to improve patient outcomes.

Region: US (primarily). Data-sharing agreement with EBMT in Europe, also supports centers in Japan and Canada

Type: Registry; multi-disease

Approach: Builds off existing research and quality assessment data platform for bone marrow/stem cell transplantation.

Payment innovation considerations: Strategic priorities include automating CIBMTR data collection to reduce reliance on manual data entry and facilitate rapid data sharing.

Table 1. Attributes of case examples

	Region	Type	Data source	Data access	Output	Structure
H2O	EU (Netherlands, Austria, Germany, Spain)	Multiple diseases: Piloting on Cancer, IBD, diabetes	PRO, Clinical	As legally and ethically appropriate, after approval by a multi-stakeholder panel, federated approach	Individual for patients; population for other stakeholders	Federated – National observatories manage data; core outcomes set allows aggregation at EU level
PCORnet	US	Multiple diseases	Clinical	Data are maintained locally at participating sites (DataMarts) to ensure privacy and security	Population	Distributed, with common data model
Real Endpoints	US	Multi-product	Claims or other systematic-ally available data	Secure data file transfer	Individual or Population	Centralized
Bee-KeeperAI	Global	Multi-party, zero-trust AI/ machine learning and analytic collaboration platform	Real-world, third-party PPI; Data and model type agnostic	Curated data sets remain in data steward's secure environment	Mutually agreed AI/ machine learning or analytic model results	Multi-party, privacy preserving SaaS solution protecting data sovereignty, privacy, security, and model intellectual property
WFH GTR	Global	Single disease/ multi-product	PRO, Clinical	Data entry required	General dashboard presenting aggregate-level data will be created for the public; Sponsors get dashboard for their product only	Centralized (US: ATHN, Europe: Hub and spoke model) Core data set; Data quality program includes checks to source
CIBMTR	US (primarily) Data-sharing agreement with EBMT in Europe	Multiple diseases in products, in context of procedure	Clinical	Currently manual; working to automate data sharing	Population	Centralized?

AI=artificial intelligence, **ATHN**=American Thrombosis and Hemostasis Network, **EBMT** = European Society for Blood and Marrow Transplantation, **IBD**=inflammatory bowel disease, **PPI**=privacy protected information, PRO=patient-reported outcomes, **SaaS**=software as a service
Centralized, federated, and distributed refer to data architecture and governance

Finding a path forward

While the barriers to payer-ready outcomes tracking are formidable, addressing them effectively is a critical next step in the evolution of value-based payment models. Importantly, there are a number of macro trends that can shift the dynamics and accelerate developments towards value based payment models:

a) Policy developments such as the Inflation Reduction Act in the US or the new Joint Clinical Assessments in Europe will require more thinking around innovative endpoints in both clinical trials and real world to meet not only regulator/HTA requirements at launch, but also demonstrate value in the long run.
b) Technology vendors may have innovative patient wearable/devices to help manufacturers enhance patient outcomes through targeted therapies, dose adjustments or personalized patient solutions. However, these will also require longitudinal patient outcomes in order to become viable commercial propositions.

Existing and emerging outcomes tracking platform could serve as powerful infrastructures for supporting value-based payments innovations, but currently do not meet the needs of payers at scale. On June 13 we will have an opportunity to explore potential ways to leverage and extend current platform capabilities to meet these needs. Concepts for discussion will focus initially on the US, but could be tailored to the EU and other global jurisdictions.

Priority areas for consideration include:

Define Clear, Measurable, and Meaningful Endpoints that are agreed to by all parties and are feasible targets for tracking.

Align incentives across payers, providers, and patients: all 3 have shared interest: achieving better patient outcomes, which is facilitated through the timely and appropriate use of innovative medicines.

Advance IT solutions that enhance the user friendliness, efficiency, quality, scale (including federated analysis capabilities), and timeliness of outcome tracking to support value-based agreements. Patients' willingness to collaborate in outcomes tracking can be a catalyst. However, this requires IT solutions that are user friendly and keep up with technological developments.

Ensure Appropriate Data Security and Privacy, in compliance with local, national, and standards.

Leverage Emerging Capabilities in coordinated ways wherever feasible in order to accelerate progress and avoid duplication, waste, and inefficiency.

Build Trust Among Parties to address universal concerns about privacy, confidentiality, and dispute resolution processes.

Establish Leadership and a Call-to-Action for a pre-competitive, multi-stakeholder collaboration focused on this goal.

Conclusion

Accelerating the evolution to value-based payment models is critical for improving patient care and ensuring the sustainability of healthcare systems. Enhancing our ability to track clinical outcomes in ways that are 'payer-ready' to ensure the timely and appropriate patient access to biomedical products is an essential component of this transition. On June 13, we will have an opportunity to explore potential opportunities for collective action toward this important goal.

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Abbreviations

ACA	Affordable Care Act
AI	Artificial intelligence
ATHN	American Thrombosis & Hemostasis Network
CIBMTR	Center for International Blood & Marrow Transplant Research
EBMT	European Society for Blood and Marrow Transplantation
EMA	European Medicines Authority
EUnetHTA	European Network for Health Technology Assessment
FDA	Food and Drug Administration
FoCUS	Financing and Reimbursement of Cures in the US
GDPR	General Data Protection
H2O	Health Outcomes Observatory
HIPAA	Health Insurance Portability and Accountability Act
HTA	Health technology assessment
ICER	Institute for Clinical and Economic Review
LEAPS	Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation
NEWDIGS	NEW Drug Development ParadIGmS
NICE	National Institute for Health and Care Excellence (UK)
PCORI	Patient-Centered Outcomes Research Institute
PPI	Privacy protected information
PPRN	Patient Powered Research Network
PROs	Patient-reported outcomes
RWE	Real-world evidence
SaaS	Software as a service
WFH GTR	World Federation of Hemophilia Gene Therapy Registry