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Prescription Digital Therapeutics: Measuring and Incentivizing Clinical impact

December 5th, 2024

1:00-4:30 p.m. ET/ 10:00-1:30 p.m. PT

Summary

Meeting Overview

On December 5th 2024, the Duke-Margolis Institute for Health Policy convened a group of expert stakeholders to discuss timely policy issues around the development and use of prescription digital therapeutics (PDTs). This group included PDT and pharmaceutical manufacturers, payers, PDT researchers, and legal, regulatory, and policy experts. This convening focused on challenges and solutions to robust evidence generation for PDTs, and potential reimbursement structures to support increased development and use of high-value products.

Challenge: Better Evidence Generation for PDTs

The first session focused on identifying gaps in the current evidence generation infrastructure for PDTs, and how stakeholders can collaborate to address these gaps. The session began with an overview of the key challenges to generating robust evidence for PDTs. To make coverage decisions, payers require evidence on how PDTs compare to the standard of care, their ability to promote patient engagement and adherence, and their cost-effectiveness. However, payers have stated that clinical study data on PDTs is often insufficient to answer these questions. Payers cite issues with: small study sizes; lack of a control group; lack of randomization or multi-site research; short follow-up periods; and failure to meet all clinical primary endpoints.

At the top of the session, participants heard about how nascent digital formularies assess products. While traditional formulary assessments tend to focus on clinical data and financial impact, digital health assessments also consider the users' digital experience in determining formulary placement.[1] Demonstrated user engagement and adherence to PDTs is critical to determining effectiveness. However, payers face multiple challenges to assessing PDTs for formulary placement. First, there is a wide spectrum of evidence available depending on the product — ranging from case studies to multiple randomized controlled trials. And even with

[1] This finding echoed an informational call with a state Medicaid office which discussed their strong focus on the user experience for their specific patient population.

robust evidence, payers have outstanding questions around how PDTs fit into the standard of care — are these tools replacements or additive?

In general, there was agreement that manufacturers, investors, and payers are not aligned on evidence expectations. Payers have stated that PDT studies lack clearly defined outcomes to address questions around reasonable and necessary coverage and to support payer assessments. Participants discussed the need for multi-arm studies that compare not only PDTs and placebo treatments, but also factor in how PDTs fit into clinical care pathways. There was a suggestion that one challenge to improving study design is that many manufacturers receive funding from investor organizations that focus on rapidly developing products and reaching the market. This can result in limited evidence generation cycles with outcomes focused on those important to FDA authorization, which don't necessarily produce the evidence needed to address payer concerns.

Participants highlighted the opportunity to integrate study outcomes that better capture digital experience and patient engagement and adherence. Generating data on these outcomes can address payer questions and support clearer reimbursement pathways. Even with improved evidence generation, ongoing uncertainty about how PDTs fit into the standard of care makes it difficult to design and assess studies that appropriately capture the role of PDTs in care and hinders coverage. One reason for this may be that the manufacturers themselves are unsure of where PDTs will fall in clinical care pathways when designing the studies. Pulling in more stakeholders early in the process could help align on the best clinical pathways to incorporate the PDT. Participants highlighted the opportunity to build on framing introduced in the 2025 Physician Fee Schedule (PFS), which describes digital mental health technologies (DMHTs) as adjunctive to stand alone mental health treatment.

Clarifying approaches to study design and the role of PDTs in clinical care pathways can contribute to larger efforts to standardize the assessment and reimbursement process for PDTs. This would also benefit manufacturers, who face varying evidence and coverage standards across payers. Aligning payers on the PDT-related study design and endpoint expectations as well as how they approach PDTs within their larger coverage policies can provide more certainty for manufacturers and incentivize further innovation in the space.

Challenge: Value-Based Approach to PDT Reimbursement

The last two sessions focused on how CMS and other payers could approach building reimbursement pathways for PDTs that incentivize value and clinical impact. Even with Medicare reimbursement of DMHT under the PFS, CMS has indicated that a statutory

amendment will be necessary for broader Medicare coverage of PDTs. This potentially provides an opportunity to establish a new reimbursement structure that moves away from the current fee-for-service (FFS) approach. There is a range of value-based payment (VBP) arrangements for medical products, as illustrated in a Duke-Margolis white paper which builds on the Health Care Payment Learning & Action Network (HCPLAN) alternative payment model (APM) framework.[2]

The second session opened with an overview of the German, French, and Belgian reimbursement pathways for PDTs.[3,4,5] These reimbursement pathways have integrated several value-based elements that CMS could build on given flexibility provided by a statutory amendment. These include establishing a coverage with evidence development pathway for PDTs with preliminary evidence, allowing negotiation between manufacturers and Medicare to set the PDT unit price, and tying part of reimbursement to the product success.

Participants discussed various considerations for developing and implementing VBP arrangements for PDTs in the US. Participants acknowledged that Medicare traditionally uses FFS payment, which is largely not a value-based program. Participants discussed potential data that is currently available or reasonably accessible which value-based payment arrangements for PDTs could utilize. These include standardized definitions of adherence and engagement, program enrollment, clinical outcomes as used in premarket clinical trials, and outcomes that capture clinical and quality of life benefits. Participants cautioned against relying on PDT opening rates-which do not capture actual engagement with the product.

Even with a statutory change that would provide leeway for innovation, participants highlighted barriers to utilizing VBP arrangements. First, PDT manufacturers have displayed hesitancy to rely on performance-based arrangements. This reluctance could be somewhat mitigated by establishing more robust evidence generation processes as outlined in the first session, but manufacturers could still be concerned with the time required to create VBP contracts and less predictability about revenue for their investors. VBP arrangements also require real-world evidence (RWE) collection infrastructure—potentially through claims and EHRs. Participants noted that creating a standardized collection process is difficult if each payer has different electronic data collection systems. Participants also discussed the need for stakeholders to share data collection responsibilities if VBP arrangements are implemented in order to mitigate burden on small PDT manufacturers.

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[2] Paying For Value From Costly Medical Technologies: A Framework For Applying Value-Based Payment Reforms
[3] PECAN DMD - Guide to DiGA in France
[4] Digital Health Applications in Belgium - Guide to Approval
[5] The three-year evolution of Germany's Digital Therapeutics reimbursement program and its path forward - PMC

Participants also considered the feasibility of incentivizing high-value PDT products within existing reimbursement systems. The 2025 PFS created codes specifically for a subset of mental health PDTs, but participants highlighted challenges to reimbursing PDTs like physician services—this pathway requires providers and health care organizations to procure PDTs directly, and thus puts the burden of negotiating prices and dispensing the product directly on health care organizations. Participants also pointed out that small tech manufacturers would need extensive guidance on compliance challenges regarding alternative payment arrangements, such as considering anti-kick-back statutes and the implications of any rebates. Reimbursing PDTs as DME removes provider burden to purchase the product, but without legislative action reimbursement in the Medicare program is limited to PDTs where the device is an integral component. In addition, both of these options would reimburse PDTs at a set amount unrelated to the clinical value of specific products within a category.

While reimbursing PDTs as self-administered drugs would theoretically allow individual products to be priced based on value, participants noted widespread provider hesitancy to engage in buy-and-bill for these types of medical products (even for practices which use buy and bill for other products) which could hinder uptake. This is partly due to the increased burden that providers would experience selecting appropriate products and negotiating with each company directly. One participant suggested that PDT reimbursement could potentially build on the payment structure for advanced diagnostic laboratory tests (ADLTs). During the first three quarters it is covered by Medicare, Medicare will pay for an ADLT code at the market price set by the manufacturer. After this initial period, the ADLT payment is determined by commercial pricing—so there may be a shift in payment if there is a discrepancy between the market and commercial price. If the commercial price is lower than the market price, Medicare will recoup the difference accordingly.[6] A similar approach could allow manufacturers to set the preliminary payment rate for PDTs and maintain this price with proof of equivalent market rate. This would allow Medicare to take advantage of private technology assessments but has the drawback of not directing paying for outcomes.

Participants expressed that integrating PDTs into pharmaceutical reimbursement may be easier because pharmacies have more experience navigating payment for "packaged units" and have processes set up for health technology assessments. This approach would require a statutory amendment to the definition of Part D coverage for Medicare. Participants also noted numerous difficult-but-surmountable logistical challenges around reimbursement as

[6] Medicare Part B Clinical Laboratory Fee Schedule Guidance for Laboratories on Advanced Diagnostic Laboratory Tests

prescription drugs, such as creating national drug codes (NDCs), databases, and standards for digital prescriptions. One participant discussed that the current ecosystem of "bespoke" specialty pharmacies that distribute PDT products are generally out-of-network in insurance plans and the number of different pharmacies makes contracting burdensome. Despite these difficulties, this direction of pharmaceutical reimbursement was generally preferred due to the benefit of private negotiation of prices, ideally reflecting clinical value, and existing health technology assessment infrastructure.

Participants also discussed ways to combine various reimbursement pathways to incentivize high-value use and innovation. One approach is to reimburse products as prescription drugs where reimbursement is based on product value, with additional payments for the physician services associated with time spent counseling patients on use and monitoring progress. Payers could also combine elements of pharmaceutical benefits and DME so that products go through value assessments but manufacturers are responsible for distribution. Within the Medicare program, participants highlighted Medicare Advantage Prescription Drug (MAPD) plans as the most realistic venue for coverage without a statutory amendment, since these plans have some coverage flexibilities and experience with assessing drugs in both the pharmaceutical and medical benefits. However, the viability of this option was questioned given limited interested from Medicare Advantage plans to engage in coverage reforms for PDTs, particularly within the budget-neutral supplemental benefits where there are more popular options.

Potential Opportunities

In the meeting's conclusion, participants noted that the new administration may be open to new ideas, and the first Trump administration was supportive of encouraging payment for novel devices. Allowing prescription software to be billed as a direct expense would incentivize this space, although not necessarily in a way the emphasizes high-quality products with the most clinical impact. Participants suggested the following opportunities to encourage movement towards value:

- Participants agreed that Medicaid is currently the biggest opportunity to innovate in this space. State Medicaid agencies can use state plan amendments and waivers to cover products and build the necessary assessment infrastructure. Value-based payments arrangements with Medicaid programs would also allow manufacturers to collect more data on clinical impact. Participants suggested that states and manufacturers would both benefit from standardization of these pilots to reduce uncertainty and contracting time and create higher quality evidence. "Blueprints" with best practices and value-based payment frameworks could help clarify opportunities for states.

- CMMI could create a pilot waiving the definition of a Part D drug, perhaps for a selected category of PDTs. However, participants acknowledged that evidence expectations for drug HTAs are significantly higher than the typical available evidence on PDTs, which could limit the coverage decisions even if the definition was waived or changed.
- It was noted that payers have more interest in DHTs when manufacturers are willing to take some financial risk for achieving clinical outcomes. Case examples and frameworks on effective methods for manufacturers to offer this could encourage more such arrangements.

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