

NEW DIGS

FoCUS

Financing and Reimbursement
of Cures in the US

RESEARCH BRIEF

By MIT NEWDIGS
FoCUS Project
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Payer perspectives on outcomes tracking for value-based payment arrangements (VBPs). With the new CMS rule changing Medicaid Best Price reporting to encourage VBPs, many options are available for which healthcare system players will collect, protect and adjudicate the outcomes data required by VBP participants.

DATA INTERMEDIATION NEEDED TO SUPPORT PRECISION FINANCING

A previous research brief that summarized the work by the FoCUS team on precision financing solutions has been published widely. Broadly, it outlined approaches including milestone-based contracts¹, performance-based annuities, installment financing², pooling methods such as stop loss insurance³, reinsurance, and multi-payer pools, subscription models, and carve-outs such as the Orphan Reinsurer and Benefit Model (ORBM)⁴.

Each of these has its own challenges that are routinely discussed, centered typically around regulatory and economic domains^{5,6}. Another challenge is ‘outcomes tracking’.

We define ‘outcomes tracking’ as all of the processes and rules, *specific to performance and payment data*, required for precision financing to work. These include:

- What data are needed?
- Who collects these data?
- Who can determine sharing of these data?
- Who analyzes these data with what rules?
- How are the data stored?

We believe the question is greater than collecting a single performance metric. Additionally, patient identification, treatment date(s), insurer, and other similar data is needed. Processes regarding data stewardship, governance, and analysis are also included and amplified because of the complex US healthcare system. A commonly understood

Key takeaways

Outcomes Tracking will involve all stakeholders – potentially including patients, but certainly payers, manufacturers, and providers.

There already exists tension over outcomes measurement across clinical trial endpoints, FDA label, and other data that manufacturers might hold.

Payers prefer clinical data – implicating providers in data intermediation.

Implications for data/IT systems are downstream from what we know today and need ongoing research

Outcomes Tracking issues need rapid resolution. Delay will only make them more difficult and complex.

challenge to multi-year financing, for example, is patient mobility: a patient can change their insurer. However, information cannot be easily shared among insurers. Hence, Insurer 1 will be hesitant to sign a performance-based, multi-year contract if out-year performance measurement (i) isn't enabled or legal, or (ii) is only feasible with outcome measures they believe are insufficient.

That is a longitudinal challenge; a cross-sectional one can be seen illustrated in Figure 1, a highly stylized outline of the US healthcare system with the flows of treatments and money, including our

ORBM concept. Along these flows with these stakeholders lies outcomes tracking that is going to be needed.

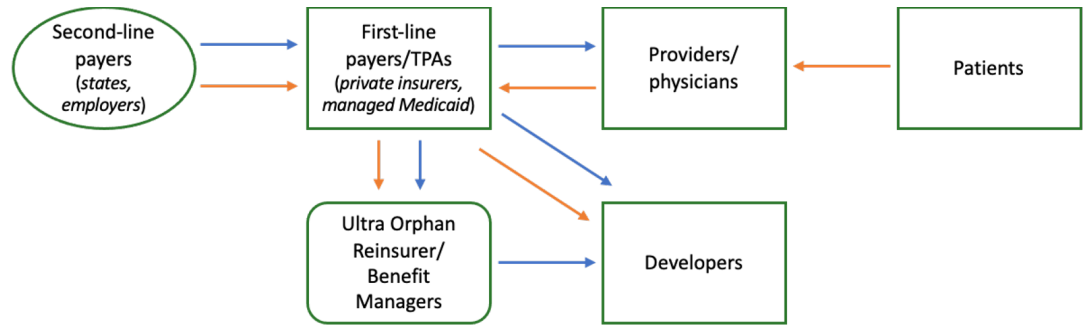


Figure 1. Flows of therapy and moneys in the US healthcare system

NECESSARY METRIC ATTRIBUTES

Choosing the right clinical performance measures is important and can be challenging. While the specific metrics will vary across disease states and products, a few general principles hold.

1. Metrics² should be meaningful, measurable, timely, robust, accessible, and predictable
2. Data must be credible, feasible, acceptable, and operable
3. Finally, an attribute that has arisen in previous MIT FoCUS Design Labs has been ‘universality’. I.e., to what extent does data need to be universal? Do all payers need to participate to achieve this? And can universality be achieved with the current systems for data intermediation, or do these to be built *de novo*?

These were synthesized for the primary research in Figure 2. It shows a perfect world in which all of the important attributes that meet the criteria above – FDA label information, trial endpoints, metrics for contracting, and claims data – were captured. It also shows how many of each attribute can be found if, e.g., only claims data were available.

These categorization approaches were used in the discussion with payers.

KEY QUESTIONS FOR PAYERS

Figure 1 suggests that many stakeholders might collect, handle and use data: payers (first- and second-line), pharmacy benefit

managers (PBMS), providers (clinicians, large treatment centers, small community hospitals, outpatient centers, and more), and patients.

We conducted confidential, semi-structured primary research with payer organizations representing ~123 million lives and many individual plans. The research followed a two-stage process between March and September of 2020. First, we interviewed and educated a contracting executive on a 33-question framework with sub-parts organized into three themes:

1. Current state of financing and payments models for new therapies
2. Data that is needed
3. Systems that are needed

The contracting executive then sought internal experts to provide responses. Upon completion, a second read-out interview was conducted with the executive and other participating colleagues as possible.

WHAT WE LEARNED

Our textual analysis of the written responses and transcribed interviews produced five themes.

1. Use of value- or performance-based contracting

We found wide variation in the knowledge of precision financing, and in preferences between outcomes-based rebates or payments as the main feature. This appears to depend almost entirely on experience: whether a payer has any such contracts in place – both total and division between CAR-Ts and gene therapies.

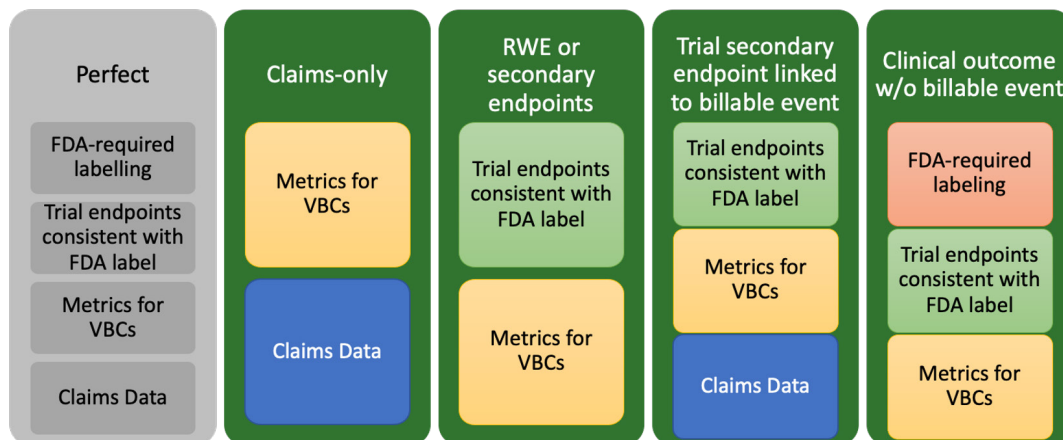


Figure 2. Stylized schematic of data attributes and sources

The existing approaches being used by our sample, to manage precision financing solutions, also came across as highly variable in terms of sophistication and maturity.

2. Expectations for the growth in performance-based contracting

Respondents clearly stated that they expect growth – and complexity. In part, the complexity will grow simply as the number of contracts increases (meaning more payers would contract for existing products and more products will launch and be contracted.) In addition, precision financing contracts will be used as more products launch for more diseases. The connection between the clinical trial data, the FDA label, and medical policy also will vary by disease. This variation in the needs of the diseases, from a performance-based contracting and measurement perspective, will primarily drive future complexity.

3. Reliance on clinical data

Respondents agreed with the performance measurement criteria listed above. They also saw tension among available clinical trial results, the FDA label, and parameters of performance-based contracting that manufacturers could bring. Payers expressed a preference for metrics based on clinical data rather than claims data or other real-world data.

4. Implications for data/IT systems

There were insufficient responses for reliable results under this theme, but it was apparent, as it has been from MIT FoCUS design labs, that there remains a lot of work to do to understand even whether centralized or decentralized systems are architectures at best, before the specific systems themselves can be assessed.

Respondents did convey a sense that providers almost certainly would be the providers of the data, and this made them a key part of any outcomes tracking solutions.

5. Payer-manufacturer roles in reducing uncertainty

Some payers expressed skepticism whether performance-based contracting addressed the non-financial core issue of whether manufacturers had produced sufficient evidence for coverage and reimbursed patient access. Rather than using performance-based contracting to fund *de facto* Phase 4 studies these payers thought manufacturers should fund further studies to justify coverage and reimbursement.

By contrast, other payers expressed an expectation that where manufacturers sought performance-based contracting innovation, the onus was upon them to bring to payers that innovation, complete with clear plans for outcomes tracking and payment mechanics. In our sample, there was no discussion about collaboration; however, this could merely reflect knowledge and awareness, rather than policy.

WHAT DO OUR RESULTS MEAN?

Many of the issues surrounding outcomes tracking are not actually about data collection and analysis. Rather, there are issues regarding whether to pursue precision financing, especially performance-based contracts, at all. This may be due to the varied, but generally low, knowledge of and experience with performance-based contracting. As a result, all subsequent questions have high variability in their answers.

Taken together, our research suggests that payers are still evaluating basic questions of precision financing goals, design and mechanics (e.g., rebates vs milestones, how many years in a contract, clinical vs other data, whether contracts might be universal, and the data centralized, etc.).

Collaboratively constructing outcomes tracking systems to build scale and standards to reduce costs cannot occur as long as these questions can remain open. Collaboratively addressing these questions, however, could occur. FoCUS provides one example of where this work is moving forward.

In the meantime, the marketplace is generating at least partial outcomes tracking offerings. They emphasize:

- Data consolidation and use for payments and contracting (payer-led)
- Data consolidation and use for treatment quality (payer-led, provider-led, or collaborative)
- Data consolidation and use for patient quality of care and access equity (payer-led, patient-organization-led, or collaborative)

LIMITATIONS AND AREAS FOR FUTURE RESEARCH

The small sample size of large commercial payers limits the generalizability of the findings. Further research is needed to understand the outcomes tracking perspectives of smaller commercial payers, self-insured employers, Medicare, Medicaid. Extending the research into both centers of excellence, and possibly community providers for future therapies, given their confirmed critical role is needed.

This research did not study the legal and regulatory implications for outcomes tracking data stewardship and governance. This gap also needs filling by future work.

CONCLUSIONS

These large commercial payer perspectives uncovered here suggest that the recognition is growing that outcomes tracking capabilities are needed to efficiently implement performance-based precision financing solutions for the whole US. In the absence of collaboration among payers, developers, providers and key third parties, disjointed approaches will arise to meet urgent needs but will not easily connect to create an efficient, likely federated, outcomes tracking solution.

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ABOUT FOCUS

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to collaboratively address the need for new, innovative financing and reimbursement models for durable and curable therapies that ensure patient access and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by these therapies leading to system-wide, implementable precision financing models. This multi-stakeholder effort gathers developers, providers, regulators, patient advocacy groups, payers from all segments of the US healthcare system, and academics working in healthcare policy, financing, and reimbursement in this endeavor.

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