The Billion Dollar Question: Are payer perspectives part of your strategy?

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Executive Summary

We've seen a number of disappointing drug launches over the years. No doubt at least a couple will come immediately to mind as you read this. While it is always difficult to see drugs underperform after launch, it always begs the question *"why?"* What could have been done differently to change the outcome? Was there poor differentiation in a competitive space? Was the market smaller than expected? Or, should more weight been given to payer sentiment and reimbursement dynamics throughout pipeline development. In this paper, we'll focus on this last option.

While we all know the importance of clinical endpoints when it comes to trials, should companies also be thinking of these in terms of payer reimbursement? And why are some companies failing to think about this until the 11th hour?

It may not be feasible to access and account for payer perspectives in every strategic decision. But it is critical to gain an understanding of the landscape – particularly how payers are looking at different therapeutic areas, what the standard implementation management tools are within an indication, and whether payers and other opinion leaders (e.g., clinicians) are paying close attention to a certain new mechanism of action or route of administration.

Having these insights will support proactive planning and resourcing decisions during drug development, and help manufacturers set realistic expectations with company leadership and other stakeholders. For smaller biotechs seeking investment, including an understanding of payer perspectives and reimbursement dynamics in the due diligence package and investor presentation points to a more robust strategy and planning process.

It's not only pharma companies that benefit from understanding the payer landscape. When a financial firm is conducting due diligence to make a go or no-go decision on investing in an asset, it's useful to have payer perspectives on what market access for that asset could potentially look like.

On the sell-side, a clear understanding of reimbursement dynamics can help investment banks to provide better guidance to clients who may be considering an acquisition based on an asset in development. Looking at how the drug will get to market is a key consideration in an investment plan.

In short, there is nothing to lose by considering payer perspectives – and potentially much to gain.

> Why are some companies failing to think about payer reimbursement until the 11th hour?

Approaches taken by big pharma companies vs. small biotechs

Every company will have different levels of resources and finances to invest in understanding and applying payer and reimbursement insights so it's important to take a pragmatic approach.

In big pharma companies, there are typically large commercial teams responsible for ensuring market access, who are thinking about coverage every step of the way throughout clinical development. Within biotechs, teams tend to be leaner and ensuring market access is not someone's full-time role. Sometimes, the conversation happens early in the process and is not revisited on a regular basis.

That is beginning to shift though, becoming a central area of focus in more smaller biotech companies. This is largely being driven by the VCs backing these companies, who want to make sure there is a market access plan in place to justify valuations.

It is becoming clearer that developers should be revisiting market access at every stage gate, from as early as indication selection, and again as they move their products through the clinic and work on portfolio optimisation.

What can developers do at each gate point to increase the likelihood of reimbursement?

There is no one-size-fits-all approach. It is an iterative process; manufacturers don't need the full depth of understanding from the beginning.

In the early phases of the drug development pipeline, companies are still formulating their target product profile (TPP), value proposition and potential clinical endpoints. During this phase, ensure you build a baseline understanding of the indication's dynamics and what coverage looks like across the market. Perhaps this is as simple as a quick pulse survey to check if the clinical endpoints that are being considered match up with what payers are looking for in a value story.

In Phase 2, you'll have a clearer TPP in place; and as you head towards phase 2b, you could start testing that TPP to see how payers react. As launch approaches, you can create a more detailed forecasting plan.

One early stage option is to ascertain how you can access the insights you'll need during each phase, and plan for incorporating those insights into development roadmaps. This way, you will make sure you are getting the right data from the beginning, so when it comes to the point where you need to make a larger scale investment around payer perspectives, you have the underlying data and are not going in blind.

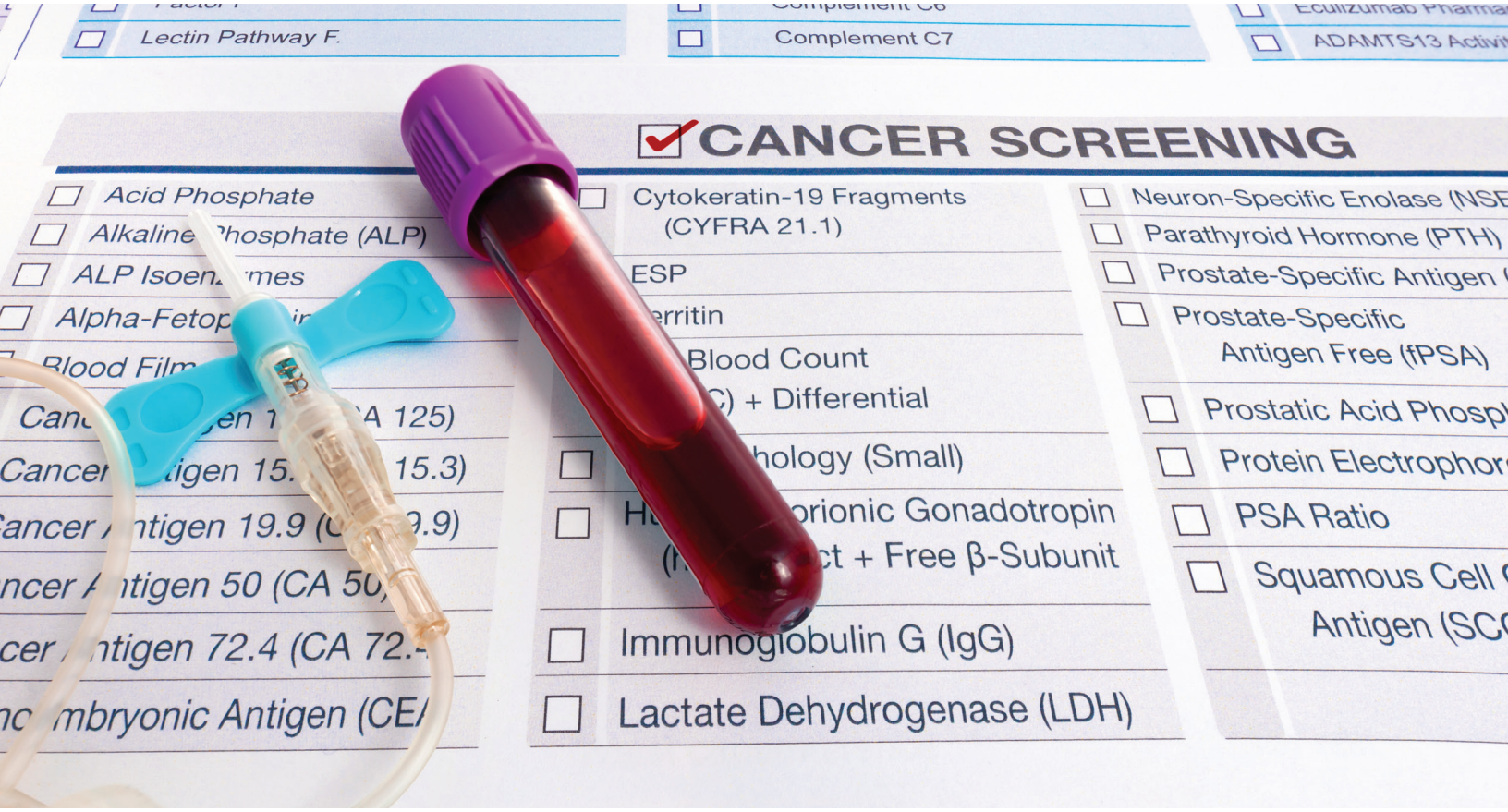
Let's consider oncology as an example. When companies think about phase 1 and phase 2 clinical endpoint selection, progression-free survival is often the go-to endpoint that manufacturers target. However, you'll be surprised at how often payers will fall back on overall survival when conducting the cost-to-value analysis, as that is more of a value proposition for them. Therefore, even if a company does not have good numbers for overall survival, but does have good numbers for progression free survival, having these payer perspectives earlier on will help to manage expectations and plan the value story effectively.

Ideally, it's about having a relationship with payers, understanding their perspectives from early on, and then maintaining that relationship – and understanding – throughout the cycle.

When has this been done well.... or not so well?

Some of the new biomarkers in oncology are showing great promise, including RET or MET targeting agents. During syndicated research conducted by MMIT based on conversations between payers and manufacturers, we found that some payers are concerned that physicians are not testing for these biomarkers, as they are so new. The concern here is that even if the payer does cover the drug, patients may not get access to it. Knowing this early on, however, enables manufacturers to formulate a strategy for educating physicians – and they can make this part of their value story. These are issues that manufacturers should be thinking about early on – and they have been doing a good job of it.

A well-publicised example from 2015/2016 is the billion dollar acquisition of Sprout Pharmaceuticals by Valiant, who acquired the company for access to Sprout's Addyi – a drug they were developing focused on female libido. After the launch, however, Addyi peak sales reached only $12-20 million – falling far short of the peak sales that justified the acquisition (in the hundreds of million dollar range). Valiant ended up selling the drug back to a group of Sprout shareholders for pennies on the dollar and a royalty later. Including market access as part of the due diligence strategy during the acquisition may have helped deliver a more realistic valuation or helped to identify a better path forward.



How are price strategies evolving as drug prices soar ever-higher?

It is difficult for payers to enforce coverage restrictions on rare disease – or orphan - drugs, especially when it is the first or second treatment within an indication and it is the only hope that a patient has. However, while most of these rare disease drugs do get coverage, quality of coverage is the issue that manufacturers need to get to grips with.

For example, within Duchenne Muscular Dystrophy, policies often have an initial authorisation duration of six months. However, in order to get a reauthorisation, the physician has to submit proof of improvement on the therapy that the patient is on. That could even mean the number of steps that the patient was able to walk in comparison to their ability before starting treatment. That is a significant restriction. If the physician is not able to prove the required outcome, how does the patient ensure continued access to the therapy?

As drugs like gene therapies hit the million dollar mark and over, we are starting to see more of these type of restrictions within rare disease therapies – and manufacturers need to understand while they are likely to get coverage, they to build an understanding around quality of coverage.
As one-time (or one-shot and cure) treatments come to market, we are likely to see a lot more alternative reimbursement approaches playing into contracting strategies. Outcome-based and value-based payment vehicles have already been proposed in a few scenarios, with a diversity of outcomes.

Bluebird bio is one developer that has struggled with pushback on pricing for its gene therapies in Europe, even after taking a novel approach. After its one-time gene therapy for severe beta thalassemia, Zynteglo, was approved in Europe, Bluebird tried to make Zynteglo's $1.8 million price tag more acceptable to payers by proposing a one-time €315,000 for the product, with additional annual instalments of €315,000 linked to patient outcomes. Despite effectively putting 80% of the list price on the line if patients did not benefit, Bluebird failed to come to an agreement with payers in Germany – and later announced a winder withdrawal from Europe. Compared to the US, many European countries have single-payer healthcare systems, giving them the weight to negotiate for lower prices.

For companies entering new markets, researching the perspectives and approaches of key opinion leaders (both payers or physicians) in those regions helps manufacturers understand how different healthcare systems operate, so they can develop their value stories and pricing strategies and set reasonable expectations.

Managing market access in the rare and orphan diseases space

A significant areas of growth in the sector is from companies finding rare disease indications for larger products, as often happens within cancer indications. Another area is when companies expand an existing agent's indications – a prime example being AstraZeneca's Ultomiris. When identifying and prioritising new indications, how can developers get payers on their side?

It's essential to understand an asset's position in the market so the manufacturer can begin developing a sound value story. Within rare indications (and non-rare indications for that matter), there are several factors to take into consideration such as unmet therapeutic need, the target population, potential ROI within that indication and more.

If you're looking to prioritise a particular indication, competitive intelligence is key. As part of this process, biopharma companies often use Evaluate to understand who else is approaching that indication and what that pipeline looks like. It's important to gauge competitive intensity and know what other competing mechanisms or modalities are in development, and where they are in the clinic. When it comes to securing funding, with the public markets and even the private markets getting tighter, developers need to be able to show investors that they have something that is differentiated.

It's also important to understand what the payer and reimbursement landscape may look like for competing agents. Developers need insights on competitors' market access, current payer perspectives on competitors' drugs, and the different utilisation management tools they are implementing for certain mechanism of action or routes of administration within the market basket. It would also be valuable to conduct high-level pharmacy vs. medical benefit analysis.

When tapping into payer perspectives, it's useful to understand how they would be likely to adjust their views should a new mechanism of action or route of administration enter that indication in the future. It's often interesting to see how payers hold different perspectives in that regard across different disease subtypes (i.e., rare diseases vs. immunology vs. oncology and so forth) and even disease states.

What's your asset's market position?

Many companies work with Evaluate and MMIT on these types of projects, helping them to gain a baseline understanding of: indication management (i.e. is it a rare indication or a non-rare indication), the unmet therapeutic need, the target population, and the potential return on investment within that particular indication. We also look at how that company's project compares against the current standard of care, and get a view on the overall cost of care within the indication. Triangulating data from all these factors helps to justify decisions around which indication to prioritise.

How do companies focus on innovation, but also stay on the side of payers?

The entire industry welcomes innovation. All stakeholders want patients to have access to the best therapies. But everyone also wants to secure their position in the value chain. From analysis of conversations between payers and manufacturers, MMIT often hears of payers embracing new mechanisms of action, especially in the US market. Payers need a clear value proposition to ensure patients do get access to and benefit from these therapies. This helps payers to highlight the cost-to-value benefit in front of their decision-making committees – making sure their organisation is secure while providing coverage for that new mechanism of action.

Often, manufacturers are excited about a new mechanism of action or route of administration that they are bringing to market – and so they should be. But they do need to understand how the market works, so they can formulate a sound value story to share with stakeholders through the pipeline.

> Payers need a clear value proposition to ensure patients do get access to and benefit from these therapies.

How can you engage with payers when there are no historic reference points?

When deciding whether to focus on a new mechanism of action or route of administration, there may be concerns that an innovative approach could put the company at a disadvantage because there's a greater risk of not being able to anticipate potential difficulties around reimbursement.

Analogue analysis is an approach that Evaluate and MMIT often use in this scenario. The manufacturer could look at analogues that had a new route of administration that entered into a similar indication with a similar market basket (i.e. a basket with a similar number of agents in it). One example would be launching an IV agent into a generous-sized oral market basket. It may also be useful to analyse analogues that were launched by smaller companies vs. larger companies at a different price point. These types of approaches help to identify how payers reacted in similar situations, as well as how the market reacted to access restrictions across different indications.

In addition to access, analogues are also valuable in understanding uptake – as these two factors go hand-in-hand. Overlaying access and sales datasets – and a host of other types of datasets on the clinical side – gives manufacturers the full picture during their planning process. With insight into what happened in historic scenarios, companies are better equipped to have the right conversations with payers and secure the right coverage.

Understanding payer perspectives can help with the development of a strong target product profile (TPP)

Developers need access to best-in-class data and analytics to flesh out their TPPs. They need to define their identifiable patient population, pricing strategy, clinical endpoints and other elements that form the building blocks of their clinical development plans and go to market strategies.

Syndicated data from Evaluate and MMIT provides the foundation for that. We can also assist with custom research and analysis, speaking with payers to understand what the current landscape looks like and how the asset in development would need to be differentiated. This could also involve speaking to clinicians, as well as regulators to understand what endpoints they would need to see to grant regulatory approval.

It's helpful to compare the TPP of an agent with that of potential competitors. Tapping into MMIT's panel of P&T decision-makers can help provide the insights needed. This could be done through a five-day turnaround rapid response survey or a more in-depth interview, depending on needs and resources. The manufacturer could ask, within a particular price range, how respondents would be able to manage their TPP vs. that of a competitor. Exploring different scenarios allows the manufacturer to understand how payer perspectives shift through different utilisation management scenarios and pricing hypotheses, in order to narrow down their understanding of the type of TPP that would resonate with the market.

> Think about payer sentiment early and think about it often so you are collecting the data you need as you move through the pipeline.

Concluding thoughts

Understanding payer sentiment and the dynamics of the reimbursement landscape is not a one-off exercise, but an intelligence gathering project that is revisited throughout the clinical development process, and iterated on as the drug moves forward through each stage gate. Think about it early and think about it often so you are collecting the data you need as you move through the pipeline.

When it is clear that you have done your due diligence you are in a stronger position to present a clear value proposition to payers, investors and other stakeholders. Understanding the type of access or reimbursement hurdles they may face helps developers to plan better, set realistic expectations, communicate better and positively impact the likelihood of a successful launch.

About MMIT

For nearly two decades MMIT has been solely focused on solving the "what and why" of market access, and has been a trusted, go-to-market partner. We believe that patients who need lifesaving treatments shouldn't face delays because accessing drugs can be confusing. As the leading provider of market access data, analytics and insights, our expert teams of clinicians, data specialists and market researchers provide clarity and confidence so that our clients can make better decisions.

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Evaluate provides trusted commercial intelligence for the pharmaceutical industry. We help our clients to refine and transform their understanding of the past, present and future of the global pharmaceutical market to drive better decisions. When you partner with Evaluate, our constantly expanding solutions and our transparent methodologies and datasets are instantly at your disposal, along with personalised, expert support.

Evaluate gives you the time and confidence to turn understanding into insight, and insight into action.

Evaluate Pharma offers a global view of the pharmaceutical market's past, present and future performance with best-in-class consensus forecasts to 2028, unique broker forecasts, and the application of proprietary methodologies to support highly robust, detailed and accurate analysis.

Evaluate Omnium provides a complete, dynamic view of development risk and commercial return across all phases of the clinical lifecycle – including early-phase and privately-developed drugs not covered by analysts' forecasts. With product-specific data including Predicted Peak Sales, Probability of Technical and Regulatory Success (PTRS), R&D Costs, Net Present Value, Time-to-Peak and more, Evaluate Omnium makes it easier than ever to quantify and compare risk and return across the full pipeline landscape.

Evaluate Epi is curated by epidemiology experts and delivers comprehensive, global epidemiological data in granular detail, on a highly interrogatable platform. Customers have access to impartial data for 15 therapeutic areas, and over 230 indications and 9,500 sub-populations across 27 core markets (up to 49 for some countries).

Evaluate Medtech provides a transparent and trusted source of market intelligence and consensus forecasting for the global medical device and diagnostic landscape, using the same proprietary methodologies as Evaluate Pharma. Customers can quickly understand how the market views products and portfolios – and where their opportunities, risks and priorities lie.

Evaluate Consulting & Analytics are specialists in solving unique and complex biopharma pipeline, portfolio and commercialisation challenges with best-in-class datasets, powerful analytical capabilities, and deep therapy and commercialisation expertise.

Evaluate Vantage provides award-winning, thought-provoking news and insights on current and future developments in the pharma, biotech and medtech industries, and is the only news service underpinned by Evaluate's commercial intelligence and data.

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