White Paper

Journey Into the Whirlwind

Post-COVID pricing and evidence policy changes and their implications for development and commercialization

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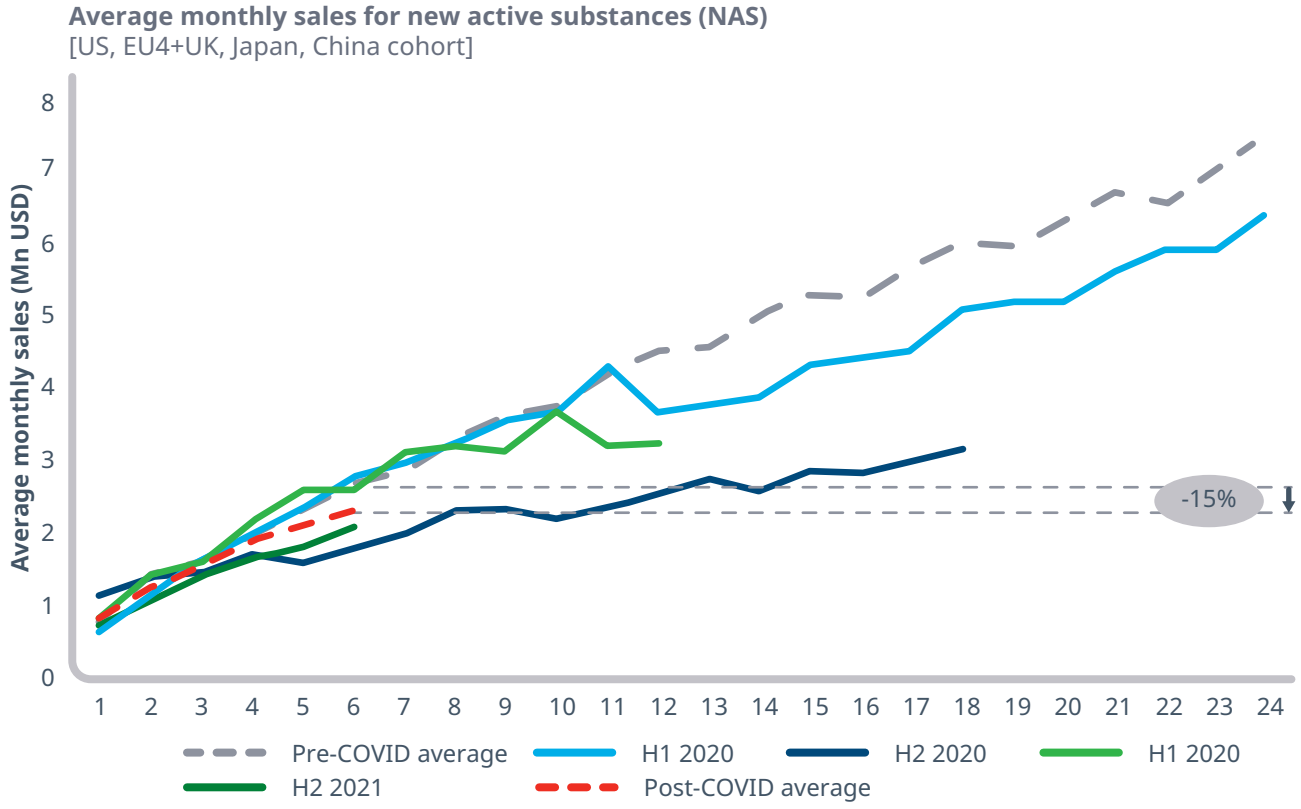
Introduction

The COVID-19 pandemic period (2020-22) has emerged as among the least commercially successful for new biopharmaceutical product launches [Figure 1]. While products with unambiguous evidence of substantial benefits to patients or health systems — including COVID vaccines and treatments — did well, most products have significantly underperformed pre-pandemic benchmarks. Reasons for the under performance include both COVID-specific factors and longer-term industry trends.

As it emerges from the worst of the pandemic, the life sciences industry must determine which disruptions to the market environment are transient and which are here to stay. Some, such as the decline in new prescription starts, will continue to recover as patients return to providers to seek care. Others, including the use of digital technology in both physician-patient communications and in marketing, sales, and medical communications, will likely continue, despite receding somewhat from pandemic peaks. Among the changes likely to endure, the accelerated evolution towards higher global evidence thresholds represents the greatest potential commercial challenge.

The pandemic increased financial pressure in health systems, translating to greater scrutiny of product value claims and expanded use of new tools and capabilities to manage access and utilization.

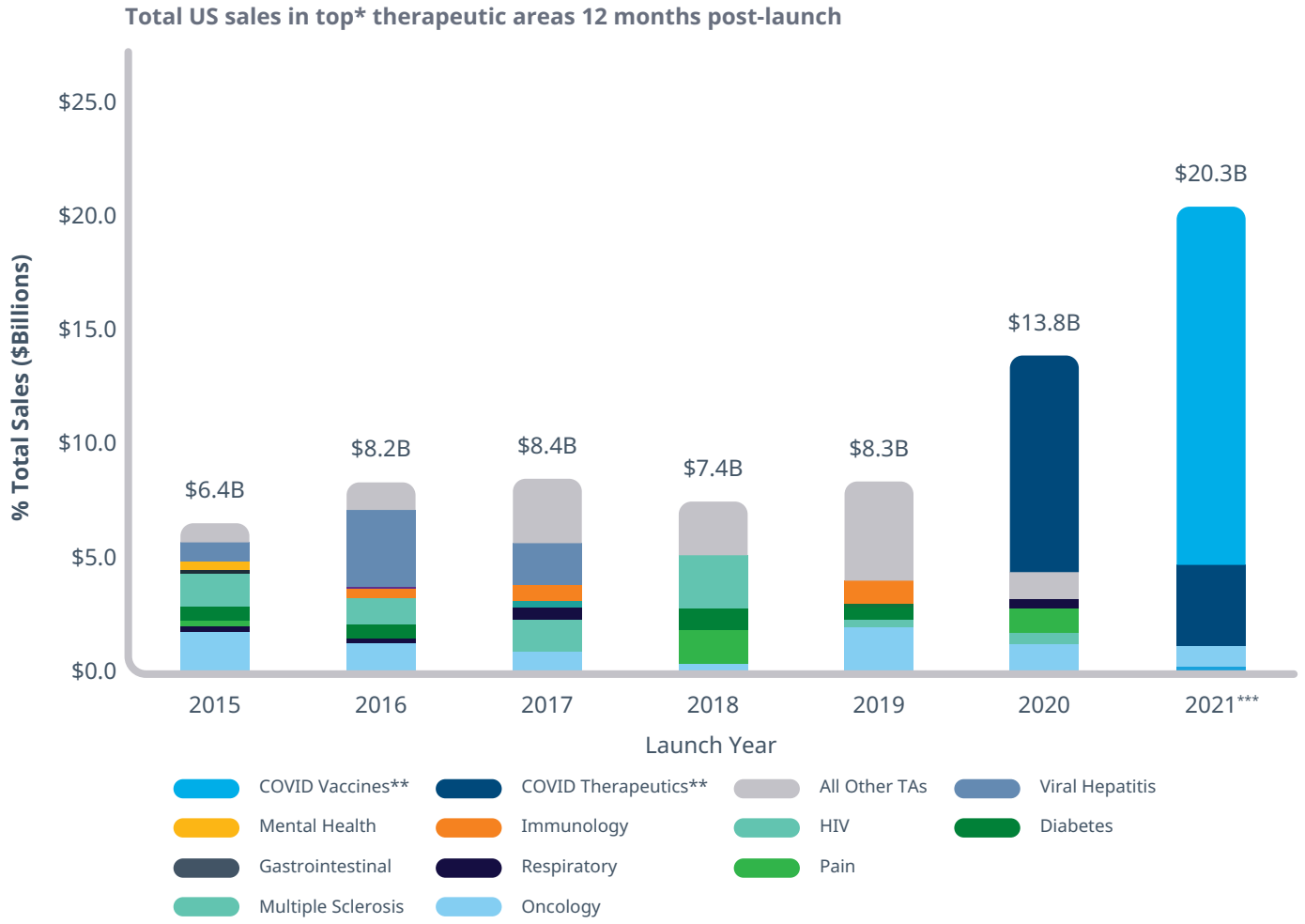
Figure 1 — The pandemic period has badly disrupted the launch environment



Rickwood S, Smith, A, and Gores M. "Launch Excellence VII: The Three Pillars of Post-Pandemic Launch Excellence," IQVIA White Paper, September 2021.

More recently, major policy changes — the US Inflation Reduction Act (IRA), the GKV Stabilization Act in Germany, and the EU HTA Regulation (HTAR) — are accelerating the emergence of a new and increasingly global evidence threshold necessary for commercial success. This new threshold will be both higher — stronger evidence of meaningful effect sizes — and broader — randomized trials augmented with RWE and patient- or caregiver-reported data. Collectively, this translates into demands for greater robustness, focus on comparative evidence, and new types of endpoints providing clear evidence of relevant, patient-relevant outcomes.[δ]

Figure 2: COVID has crowded out other spending



*Markets with the highest overall proportion of launch sales shown;
**COVID treatments include Veklury (2020), Regen-Cov(2020), Bamlanivimab plus etesvimab (2021), Xevudy (2021), molnupiravir (2021), Paxlovid (2021), Vaccines include Pfizer, Moderna, and J&J vaccines
***2021 data through Dec 2021 and thus incomplete for products launched after January 2021
Source: IQVIA Institute; IQVIA NSP Dataset; Market Access Strategy Consulting analysis

δAs with the period prior to the pandemic, value capture opportunities associated with providing greater health system benefit will remain. Products like Roche's Phesgo, which replace an IV therapy with a subcutaneous alternative, thus reducing demands on scarce infusion capacity, continue to be successful. While earlier evidence planning for such products is valuable, they fall outside the scope of this paper.

The impact of the pandemic on health care budgets

The pandemic represented an unprecedented, one-time exogenous shock to health systems, driving up hospitalization costs and introducing unforeseen COVID treatment and vaccine costs[†], while at the same time reducing demand in many chronic and non-emergent therapeutic areas. In some countries the pandemic exposed significant underinvestment in everything from hospital capacity to sectoral wages that must now be addressed. Additionally, across many therapeutic areas, a substantial backlog of patients awaiting diagnosis or treatment has accumulated, and many of these patients are now presenting with more advanced or complicated illnesses. The costs of clearing these backlogs and of treating the many millions of patients will be substantial and may take several years to work through the system.

Of more direct consequence for access and evidence are the dynamics of biopharmaceutical spend. During the pandemic, expenditure on new products (those launched within the previous 12 months) reached historic highs, but this was mostly on vaccines and treatments for COVID itself. Consequently, spending on most other new products has largely been crowded out. [Figure 2] Indeed, only those products that could credibly claim to transform or disrupt the standard of care — either by significantly improving clinical outcomes or by stripping time or costs out of health systems — achieved significant commercial trajectories. Given that the COVID-specific products themselves had unusually strong value claims — the prevention of millions of unnecessary deaths and hospitalizations — payers and other stakeholders have a clear benchmark of what good looks like.[‡]

This phenomenon closely parallels the analogous shock presented by the innovative antivirals for Hepatitis C in 2014-16. The launch first of Gilead's Sovaldi and then several competitors and follow-ons created a substantial budget shock reflecting much higher demand than had been anticipated in many key markets, including the U.S. Additionally, the HCV case did not feature concurrent or offsetting declines in care volumes elsewhere, so aggregate system costs rose unexpectedly.

As the impact receded and payers and health systems adjusted to the HCV therapies, however, market access conditions remained challenging, most notably in the case of the PCSK9s. Launched starting in 2015 by, respectively, Amgen and Sanofi/Regeneron, Repatha and Praluent offered dramatic reductions in LDL-c, far beyond what was possible with statins or other available therapies. Armed with trial data demonstrating these substantial and sustained LDL-c reductions, both products entered with high hopes and analyst expectations of a market exceeding $4 billion in value.

Unfortunately, in the post-Sovaldi market, payers around the world pushed back, fearing the potential budget impact of a population as large as that with elevated cholesterol and at risk of a cardiovascular event. Noting that LDL-c was only a surrogate marker and that pivotal trials had not shown evidence of improved cardiovascular outcomes, payers limited access to these products, particularly at their list prices — roughly $14,000 annually in the US, up to €7,000 annually in Europe. Whereas the HCV products could offer a powerful and clear value proposition — curing a disease in as little as eight weeks, with minimal side effects — the PCSK9s could not at launch offer similar clarity. Access was constrained nearly everywhere, and commercial performance fell far short of expectations.

[†]For the period 2020 through 2027 the phased rollout of vaccines and booster shots is expected to result in $380 billion in incremental spending globally. Expected use of novel therapeutics for COVID-19 is estimated to result in a total of $120 billion over seven years, resulting in a total impact of vaccines and therapeutics of $497 billion, or about 3% of cumulative global spend during that period. IQVIA Institute, "Global Use of Medicines 2023 – Outlook Through 2027," January 2023.

[‡]It should be acknowledged that during the early days of the pandemic the unmet need was exceptionally high, which led health care systems temporarily to accept lower standards, especially before the vaccines became available. For example, Gilead's Veklury was initially approved on relatively limited evidence.

Policy changes after COVID

Notwithstanding the HCV example, the subsequent period nevertheless saw a number of innovative products launch successfully, often at very high prices, particularly in oncology and rare or orphan disease areas in which the populations were small and the overall impact within the bounds of past precedent. While evidence thresholds shifted upwards in some areas, the effect was not universal. As we emerge from COVID, however, payers and governments are thinking more broadly, particularly in the context of geopolitical instability and competing budgetary demands. Across many of the most commercially important markets recent and planned policy changes will significantly alter the dynamics for access, evidence, and price.

Among the large European markets, Germany exerts the greatest overall influence, reflecting the combination of its rigorous comparative effectiveness review process and its central role in other countries' price referencing systems. By granting manufacturers pricing freedom for the first twelve months from launch, Germany has ensured early access to medicines and granted manufacturers a relatively high starting point for subsequent price negotiations. Further, Germany has shielded orphan drugs with annual turnover of less than €50 million from the full HTA process, strengthening incentives to invest in development of products for rare diseases.

The GKV Stabilization Act, which went into effect in early 2023, alters the current rules. The twelve-month post-launch free pricing window has been reduced to only six months. Additionally, the orphan threshold would decline from €50 million to only €30, meaning that high-priced products for rare conditions would be subjected to the same rigorous evaluations as other products, and much earlier in the lifecycle. German data indicates that orphan products take up to nine years to reach €50 million in annual turnover. That would fall to about three years with the lower threshold.[§] Further, because this change will now necessitate direct comparisons with standard treatments and the use of clear, patient-relevant endpoints, Germany is effectively narrowing one of the few paths to market for products approved on single-arm trials or with surrogate endpoints. The reduced free-pricing window will similarly challenge manufacturers' strategic decision-making by reducing

[§]If, with the introduction of the Joint Clinical Assessment (JCA) under the EU's Health Technology Assessment Regulation (HTAR), a JCA report is available for orphans at the time of launch, this timeline could be further reduced. If IQWiG has reviewed the JCA dossier at time of launch and provided its input on the PICO at that time, the more rigorous requirements associated with the full AMNOG process could apply in de facto terms for all orphan products at launch.

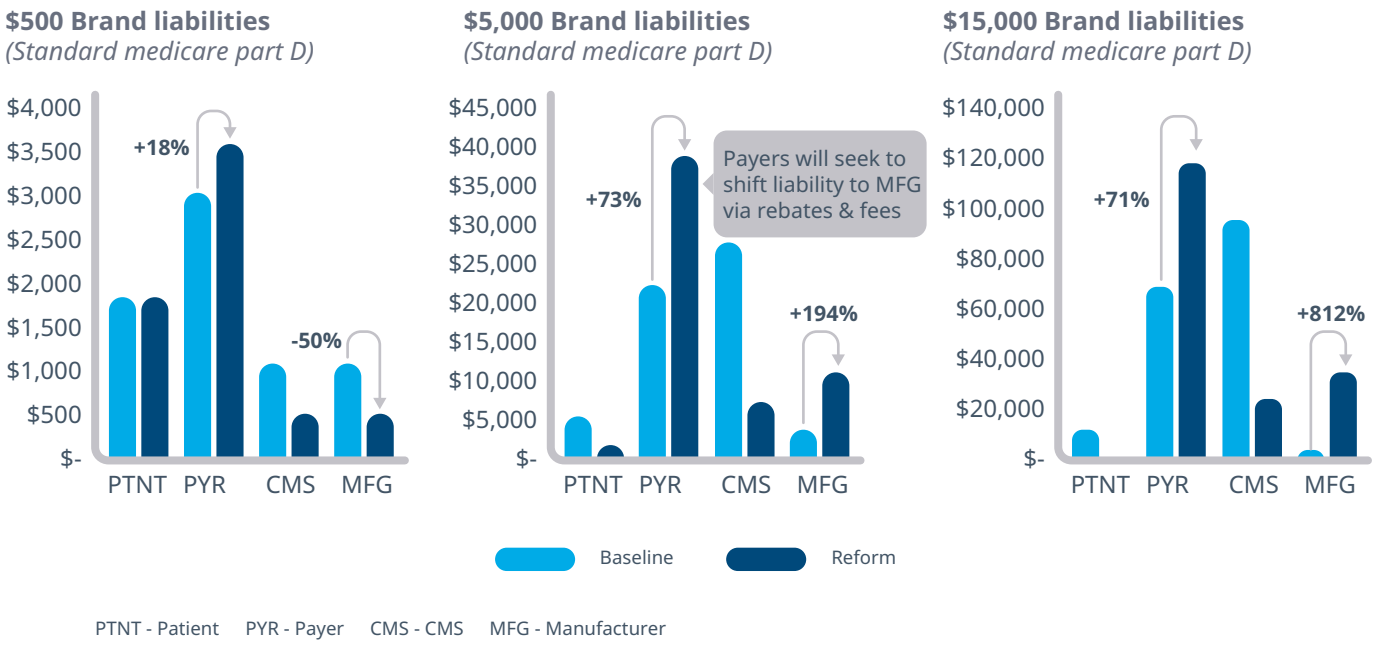
asset valuations, especially when evidence packages are unlikely to sustain high price levels after AMNOG—*Arzneimittelmarkt-Neuordnungsgesetz* or, in English, "Pharmaceuticals Market Reorganisation Act".

The relative decline in the attractiveness of the US market starts in the commercial setting, where insurers and health systems feel increasingly empowered to restrict access to products and to extract substantial net price concessions, even in previously off-limits TAs like oncology. This has created widening gaps between gross and net revenues, even for products that had comparatively successful launches. For example, recently launched oral migraine drugs, which exhibited impressive uptake despite the pandemic, appear to have suffered significant margin compression. Generally, the expansion of bridging and affordability programs, combined with more traditional access rebates, has steadily increased margin pressure, without consistently expanding access.

As in Germany, the US has remained an attractive market for orphan and rare disease therapies, as well as for oncology treatments more generally. For the latter, the fact that most patients were covered by the Federal government's Medicare program for Americans over the age of 65 has helped to perpetuate attractive market conditions, since Medicare has famously been prevented from negotiating drug prices. Indeed, in contrast to the Medicare market, gaining accessible formulary placement in the US commercial market has grown steadily more difficult and expensive, with payers increasingly willing to exclude approved products from their formularies.[1]

With the passage of the Inflation Reduction Act — designed to save $300 billion in drug costs over a decade — the US market will grow more complicated. In particular, the introduction of price negotiations in Medicare — beginning with Part D drugs in 2026 and extending to Part B in 2028 — and the increase in payer liability in the so-called "catastrophic" phase from 15% to 60% will dramatically amplify scrutiny of price and value claims [Figure 3]. For plan sponsors facing a dramatic increase of their cost liability for some high-cost drugs, initiatives to manage utilization and access much more tightly seem an inevitable consequence. As in Europe and Japan, what constitutes meaningful innovation will reflect measurable, incremental clinical value, rather than scientific novelty.

Figure 3 — The part D price shock will increase scrutiny of value claims



Assumptions: (1) Only product patient is on, (2) full 12 month compliance, (3) Manufacturer Paid PRD discounts as proposed, (4) Many scenarios possible
Source: IQVIA Institute net sales adjustment data, IQVIA WAC library, US Market access strategy consulting analysis

The shifting evidence environment

Globally, the accommodations of regulatory and access decision-makers for accelerated approvals face reassessment. In the US, where the FDA introduced the expedited pathway in the 1990s to facilitate faster access to HIV therapies, the explosion of accelerated approvals in oncology and rare disease has cut times to market while raising questions about the clinical value delivered. One recent review concluded that only one in five oncology products approved on an accelerated basis ultimately generated confirmatory evidence of an overall survival benefit [Figure 4]. This means that a large majority of patients may have derived no meaningful clinical benefit from these therapies, raising critical questions about the value of both single-arm trials and a range of surrogate endpoints.[2] US payers facing difficult budget choices — particularly Part D plan sponsors — will think twice before adding novel therapies with limited evidence. As FDA Commissioner Robert Califf told an audience at the BIO international conference in June, "welcome to scrutiny."[3]

In fact, as US payers exert additional pressure on manufacturers' value claims, the US will begin to resemble other countries, which routinely challenge products with immature or incomplete data or surrogate endpoints. The independent Institute for Clinical and Economic Review (ICER), while not mandated by any responsible entity in the US, has already begun to exert influence over assessment and coverage decisions. And as noted, the accommodations made for orphan products in Germany are being rolled back, in large part because half the products granted access after a limited benefit were subsequently found to have no proven benefit. The implications of a US market characterized by European-type evidence expectations would be significant, with far reaching consequences for clinical operations, portfolio strategy, and geographic balance.

Even without the formal adoption of European-type HTA models, the shift in the evidence environment has already begun. During the pandemic, launch trajectories were well below historical expectations, even in areas of comparatively high-unmet need. For example, we saw the launch of two innovative molecules for relapsed refractory diffuse large B-cell lymphoma (DLBCL), an orphan condition characterized by very poor outcomes. Both products — Monjuvi (tafasitumab) and Zynlonta (loncastuximab) — were approved on an accelerated basis based on limited data from single-arm Phase 2 trials. The primary endpoint in each trial was overall response rate (ORR), and each study included a series of secondary endpoints, including duration of response (DoR), and progression-free survival (PFS). Despite the accelerated approvals and high unmet need, however, adoption has been very limited, suggesting hesitation among clinicians to use products without comparative evidence of incremental clinical benefit.

Formal HTA systems want data against the relevant standard of care, presenting an additional challenge. Embedded in this requirement is the desire to understand whether a new therapy is providing better value or benefit than what is already available, ideally the therapy most commonly used in the treatment setting. Evidence from recent US launches suggests that, at least informally, a similar expectation is emerging. For example, Zeposia (ozanimod), a product approved based on a Phase 3 study against interferon-β-1a, has struggled to gain a foothold in the US market for multiple sclerosis therapies. While interferon-β-1a was for many years a common front-line treatment in relapsing MS, treatment patterns evolved sharply after the launch in 2016 of Ocrevus (ocrelizumab), the first B-cell modulating therapy, and B-cell modulating therapies have since become the dominant class in the category. Comparative data against an active comparator may be insufficient if it is not the standard of care.

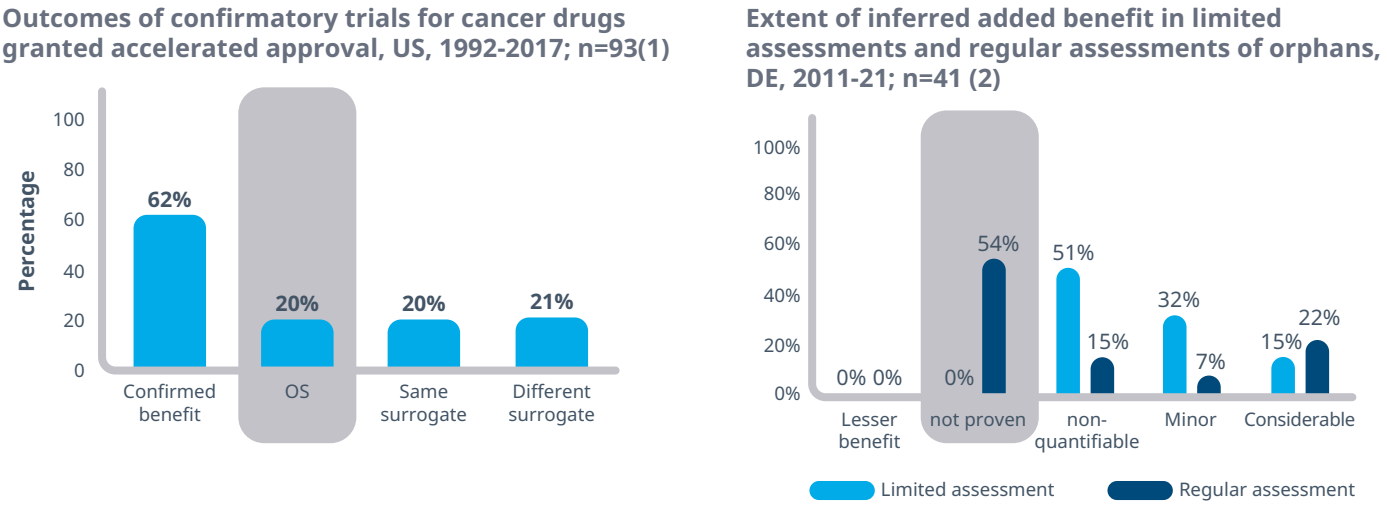
Additionally, payers increasingly challenge endpoints, especially surrogate markers. Germany has famously challenged endpoints like sustained viral response (SVR) in hepatitis and even progression-free survival (PFS), the latter on the grounds that, based on a purely radiographic interpretation, it is itself neither a patient-relevant outcome nor strongly correlated with a patient-relevant outcome, particularly survival. Most commonly these critiques point at the potential

disconnect between intermediate effects, such as the reduction in LDL-C, and the downstream outcome of interest, since the validity of surrogate outcomes has rarely been fully established in a rigorous manner.[4,5]

More recently, however, questions have emerged regarding whether some endpoints — including those approved by regulators — can effectively capture an effect relevant to the patient. In Alzheimer's Disease (AD), the Clinical Dementia Rating Scale Sum of Boxes (CDR-SB) is increasingly common as a primary end point, yet the evidence linking it to patient-relevant outcomes is thin. Key pivotal AD trials, including those for both Aduhelm and Leqembi, have been powered to show significant changes in CDR-SB, but how this translates into something meaningful to a patient or caregiver remains unknown. Increasingly payers and HTAs want to understand what patients define as a relevant change — the so-called minimal important difference (MID).[6,7] Would, for example, a 25% decline in the rate of change on CDR-SB mean a patient would remember a loved one's face for another six months or could live independently for longer?** In a period of rising budget pressure, access for expensive therapies based on only intermediate, surrogate, or scale-based measures, even in areas of high unmet need, may no longer be assured. Clinicians may hesitate to prescribe them, particularly if benefits are uncertain, and in the US Part D market, the higher cost exposure facing plan sponsors will intensify scrutiny of product value claims.

In cases like AD, where the natural history of the disease remains poorly understood and treatment options limited, or gene therapies, for which time horizons reduce the utility of traditional payment models, additional real world evidence (RWE) could be employed to help contextualize regulatory endpoints, or to provide additional detail about patient experience with a condition or a treatment. In many cases, however, manufacturers have instead used traditional RWE as an alternative to more robust evidence generation strategies, hoping in effect to substitute a lower-cost or lower-risk real world analysis for a head-to-head study. Payers, unsurprisingly, have challenged these approaches, asking why, if an alternative treatment was available, a manufacturer sought instead to use an indirect treatment comparison based on published data, external control arm, or similar design. Significantly, of the orphan products subsequently shown upon full assessment to offer a meaningful benefit in Germany, all used randomized controlled trials to generate the confirmatory evidence[8] [Figure 4], and the emerging guidance from the EU HTA Regulation, as well as individual countries, reasserts the primacy of randomized controlled trials as the gold standard.[9,10]

Figure 4 — False hopes – the disappointing reality of accelerated approval pathways



**In its recent assessment of Leqembi, ICER notes that "The absolute difference in CDR-SB of 0.45 points between groups, while statistically significant, may or may not result in a change in status that is meaningful to individual patients and caregivers." Lin GA, Whittington MD, Wright A, Agboola F, Herron-Smith S, Pearson SD, Rind DM. Beta-Amyloid Antibodies for Early Alzheimer's Disease: Effectiveness and Value; Evidence Report. Institute for Clinical and Economic Review, March 1, 2023. https://icer.org/assessment/alzheimers-disease-2022/#timeline

Implications for evidence

As governments and payers around the world seek to manage healthcare costs in the wake of the pandemic, it is difficult to envision a reversal of the trend towards higher evidence thresholds. Indeed, as healthcare data continues to proliferate and technology enables payers and health systems to harness it, their ability to draw their own conclusions about the value of a novel therapy will only rise, diluting manufacturer value claims.[11] In this new, post-pandemic environment it will be increasingly critical to demonstrate meaningful, patient-relevant benefit over standard of care (SOC). The key components of this can be broken down further:

- **Comparator choice** — if the comparator is not part of the current standard of care (SOC), the company needs to have a clear explanation, i.e., the standard changed while running the trial or an absence of an SOC, or that standards vary across countries. Many companies still seek to de-risk clinical development plans (CDPs) by running trials against the weakest possible competitor, but payers and HTAs have caught on. Products with the wrong comparator — or none — when an alternative is available will struggle
- **Population definition** — The HTAR, in particular, has reinforced the importance of much closer alignment between regulatory and access strategy. Where there is a mismatch between trial population, label, and the population for which access is sought, payers will restrict or even deny coverage. Advance planning is essential to allow for generation of robust comparative evidence that can meet the requirements for Joint clinical assessment (JCA) in Europe, and for other stakeholders elsewhere. Understanding the payer-relevant populations and comparators in each market and anticipating the PICO[††] based on the HTA and payer landscape will become a pivotal first step in designing evidence generation plans
- **Patient-relevance** — Not all outcomes are considered equally important to patients, and regulatory endpoints may no longer be sufficient, particularly in areas like neuroscience and oncology, or anywhere the use of scale-based measures is common. Further, since the pandemic has enabled more remote or hybrid care models, there is increasing interest in whether outcomes vary across settings and models. Payers, HTAs, and, increasingly, the FDA and other regulators want to see measures that can easily translate into improvements that matter to patients — outcomes that directly measure mortality, morbidity and outcomes related to patients' feelings, beliefs, preferences, needs and functions (such as the ability to perform activities in daily life).[7,12] A rigorous or "scientific" assessment of patient experience is increasingly important, both for trial recruitment and evidence generation purposes
- **Meaningful** — in addition to patient-relevance, outcomes must exhibit an effect size meaningful to the patient. As in the AD example, does the demonstrated change on the primary outcome measure translate into a change in the patient's health status sufficient in size that the patient experiences a meaningful difference? A 20% reduction in the rate of change on a scale might not have a meaningful impact on time to event. Products unable to demonstrate this are likely to face higher hurdles

Strategic imperatives

To navigate this environment, we see several important success factors. First, companies must start earlier and develop **comprehensive, integrated, multi-stakeholder evidence plans**. Solving for regulatory approval is no longer sufficient, and without carefully planning for payers, physicians, and patients, companies risk commercial failure. Unless there are very specific ethical or operational reasons not to use an active comparator, strategies must seek to

[††]PICO (Population, Intervention, Comparator(s), Outcomes) framework provides a standard format for the definition of a research question and helps to specify the data requirements for the assessment.

deliver evidence of a meaningful, incremental benefit relative to what is already available. Further, where potential trial designs are likely to lead to ambiguity or uncertainties for payers, companies should begin quite early on to engage with regulators and HTA bodies and devise strategies to enrich the evidence in ways that will help their customers minimize potential associated risks.

Similarly, **endpoint selection** takes on greater importance. If an accepted regulatory endpoint cannot translate easily into meaningful patient-relevant benefit, payers and health systems will de-risk themselves by reducing access and driving down price. In some cases, it may be possible to augment or even validate the endpoint with additional RWE characterizing natural history or patient experience, especially in rare conditions unfamiliar to most stakeholders. Further, novel patient-centered endpoints unique to an individual condition and reflective of milestones and effect sizes important to the patient are also effective but do require careful advance planning and — significantly — may introduce uncertainty into a trial design. The payoff, however, may be better access and uptake.

The emerging evidence environment presents significant commercial risk for pipeline assets, especially in oncology, rare disease, and other specialty TAs, and in the US for high-cost oral solids and self-injectables more generally. Every company in these spaces urgently needs to **interrogate asset NPVs and underlying assumptions**, to clean up their pipelines, and update portfolio strategies. Companies hunting for assets need to be extremely rigorous in their diligence and valuation exercises and consider how higher evidence expectations will affect potential commercial returns.

In addition, the evolving access environment will amplify **concerns about industry R&D productivity and ROI**.[13] Costs remain high, but a more challenging access and evidence environment risks eroding returns still further. Companies need to accelerate adoption of decentralized trials (DCTs), adjust their risk appetites in Ph1/Ph2, validate and integrate relevant Patient centered endpoints (PCEs) / electronic clinical outcome assessments (eCOAs) into programs, and kill programs much earlier. Above all, companies must tighten alignment between development, medical, and commercial in order to shape the integrated

evidence strategy, advise on adequate and meaningful comparators, and ensure the patient-relevance of planned endpoints.

And in some rare cases, particularly if the path to profitable US access appears poor, **delaying launch or altering planned indication sequencing** until additional data are available may be an appropriate consideration. For multi-indication products, for which optimizing the price/volume tradeoff is critical, insufficient evidence planning will have a particularly erosive effect on asset values.[14] Given the challenges of changing course — our research on launches consistently finds that fewer than one launch in five is able successfully to correct its trajectory after the first six months[15] — at minimum companies should carefully model the consequences of trading time to market for better access and higher peak sales.

Lastly, with growing scrutiny and complexity, traditional strategies for **evidence dissemination and communication** need to evolve, as well. A well-designed, thoughtful evidence generation strategy is central to commercial success, but if the evidence is not communicated to the right stakeholders, through the right channels, its impact is reduced. Here again the role of Medical Affairs is critical, alongside Market Access, and has implications for the GTM/customer engagement model.[16]

As the market recovers from the shock and disruption of the pandemic, a combination of heightened budget pressure, regulatory and policy changes, and rising health system capability to harness healthcare data is establishing a new, higher evidence threshold for new products. While the mechanisms vary from market to market, the direction of travel is consistent, and a growing number of cases reinforce the significant commercial consequences of failing to meet this rising threshold. Unless companies take urgent action, they risk poor launches, continued margin erosion, and declining returns on investment.

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