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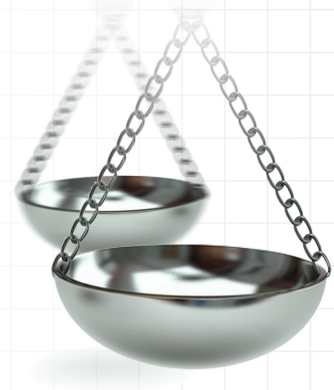

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Access in all areas? A round-up of developments in market access and health technology assessment: part 11

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In this update, we cover the evolving health technology assessment process in Australia and also examine how commercial health plans in the US influence patient access to treatments.

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Australia's mixed public–private healthcare system, underpinned by Medicare, provides universal access to essential medical services and subsidized medicines through the Medicare Benefits Schedule and the Pharmaceutical Benefits Scheme (PBS), respectively. The system's health technology assessment (HTA) infrastructure, led by the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC), ensures that funding decisions are evidence-based and cost–effective.

In September 2024, the government released the final report of the HTA Policy and Methods Review, the most comprehensive assessment of Australia's HTA system in more than a decade [1]. The review examined how medicines, vaccines, and medical technologies are evaluated for public subsidy across the PBS, Medicare Benefits Schedule, National Immunization Program and Life Saving Drugs Program. Following extensive consultation, the report delivered 50 recommendations focused on four core objectives: improving equity of access, particularly for First Nations peoples and pediatric populations; reducing time to access through more efficient pathways; enhancing transparency and stakeholder engagement and building capability to assess emerging technologies such as cell and gene therapies.

To operationalize these reforms, the government established the HTA Review Implementation Advisory Group (IAG) in February 2025, chaired by the PBAC chair and comprising representatives from consumers, clinicians, industry and academia [1]. Its mandate is to translate the review's 50 recommendations into a sequenced, funded roadmap for reform by January 2026. The IAG's interim report, released in August 2025, outlined its analytical framework and emerging priorities. Using a structured, evidence-based approach, each recommendation was assessed for impact, complexity, interdependencies and resource requirements. This process identified significant overlaps across recommendations, leading to consolidation into five interconnected focus areas guiding the next phase of reform: improved access and equity; greater transparency and engagement; modernized assessment pathways; better data and evidence; and building HTA workforce capacity and capability.

The access and equity focus area addresses long-standing disparities affecting underserved groups, notably First Nations peoples and pediatric patients. For First Nations health, the HTA review found major inequities in medicine access, with PBS/Repatriation PBS (RPBS) expenditure per person less than half that of non-Indigenous Australians. Key barriers include small patient populations, limited commercial incentives for submissions and misalignment between current HTA processes and community health priorities. The IAG proposes establishing a First Nations HTA Advisory Committee co-developed with Aboriginal Community Controlled Health Services and other stakeholders. This committee would advise PBAC and MSAC on priority health needs, participate in

horizon scanning for promising therapies and ensure submissions address impacts on First Nations outcomes. For pediatric access, the IAG notes that most PBS submissions focus on adult populations due to difficulties generating trial data in children. A targeted working group will explore age-agnostic listing approaches and identify priority pediatric conditions that warrant tailored assessment pathways. Finally, the IAG will define criteria for high unmet clinical need and highly advantageous therapeutic value, terms widely used but not formally defined. Establishing consistent definitions will enable modified or accelerated pathways for therapies that address urgent health needs or deliver transformative benefit.

The transparency and engagement focus area seeks to improve understanding, participation and trust in decision-making: stakeholders consistently raised concerns that HTA processes, while well-documented, remain complex, technical and inaccessible to non-experts. The IAG recommends developing a comprehensive stakeholder engagement framework outlining when and how engagement occurs across the HTA cycle, from horizon scanning and PICO development to appraisal, post-market review and disinvestment decisions. Complementary updates to PBAC guidelines will focus on two priority areas: first, managed entry agreements, which remain underutilized despite their potential to balance early access with uncertainty management. Second, comparator selection, where further clarification will improve predictability while maintaining flexibility. Together, these measures aim to improve consistency, transparency and the quality of dialogue between industry, government and patients.

The modernized pathways focus area targets inefficiencies across Australia's complex ecosystem of HTA routes. Many current processes involve sequential or duplicative assessments that prolong timelines and increase administrative burden. The IAG is considering pilot programs that would allow PBAC to directly recommend to the Minister on certain categories currently requiring additional steps, including Life-Savings Drugs Program medicines (which now undergo separate expert panel review) and co-dependent technologies requiring both PBAC and MSAC assessment. For vaccines, the IAG sees scope to streamline the National Immunization Program pathway. Current processes involve sequential advice from the Australian Technical Advisory Group on Immunization (ATAGI) and PBAC, adding nearly 8 months to evaluation timelines. Proportionate appraisal approaches could remove mandatory PBAC review for vaccines consistent with already-approved classes. In addition, for proportionate medicine pathways, cost-minimization submissions, where products demonstrate equivalent safety and efficacy to existing comparators, could be assessed via simplified, lower-intensity pathways. These reforms would reduce duplication, improve timeliness, and align assessment effort with submission complexity and risk.

The data and evidence focus area reflects recognition that HTA methodologies must evolve to keep pace with emerging technologies and stakeholder expectations. Discount rate reform is a central element. Current 5% base-case rates significantly undervalue long-term benefits of treatments with high upfront costs, such as gene therapies. The review recommended reducing this to no lower than 3.5%, aligning with international norms (1.5–3.5%). Further guideline updates will address assessment of non-randomized evidence, surrogate end points, biomarker-driven and tumor-agnostic therapies, and patient-reported outcomes. These changes aim to modernize Australia's evaluation framework while maintaining scientific rigor. Real-world data and real-world evidence will also play a growing role in HTA. The IAG proposes mapping existing data sources and access arrangements to identify opportunities and gaps, leveraging digital health initiatives such as the Australian Digital Health Agency's Health Connect Australia. Increased use of real-world data can have multiple benefits, including strengthening cost-effectiveness models, utilization forecasts and post-market evaluations.

Regarding building HTA capacity and capability, sustained reform will require a skilled, adequately resourced workforce. The review highlighted increasing volume and complexity of submissions, driven by innovative modalities such as cell and gene therapies, advanced diagnostics and genomic technologies, which demand deeper scientific and methodological expertise. The IAG recommends a comprehensive assessment of HTA workforce capacity, identifying current gaps and future needs across both government and independent evaluators. Enhancing training, technical infrastructure and recruitment pipelines will be critical to maintaining Australia's leadership in evidence-based decision-making.

For companies operating in the Australian market, this reform agenda signals both opportunities and strategic considerations. The IAG's January 2026 final roadmap will provide detailed sequencing enabling companies to align internal processes, capability development and resource allocation with anticipated changes. The prominent focus on equity considerations spanning First Nations health and pediatric access, reflects government priorities that will likely influence assessment criteria. Companies developing therapies for these populations should proactively engage with emerging frameworks. For First Nations health specifically, companies with relevant pipeline products should establish relationships with Aboriginal Community Controlled Health Services and ensure product develop-

ment addresses outcomes meaningful to First Nations communities. Streamlined pathways for cost-minimization submissions and specific vaccine classes could substantially accelerate access for appropriate products while reducing evaluation burdens. The establishment of formal high unmet clinical need and highly advantageous therapeutic value definitions will create new thresholds requiring companies to substantiate claims through robust evidence. Companies developing therapies for rare diseases or conditions with limited treatment options should engage proactively with stakeholder consultations developing these definitions. Manufacturers who actively engage with the evolving framework rather than waiting for final implementation will be better positioned to influence outcomes and optimize their Australian market access strategies for the increasingly differentiated assessment landscape emerging from this reform process.

While Australia's reform agenda emphasizes systematic, evidence-based improvements to its centralized HTA infrastructure, the US presents a contrasting landscape where decentralized commercial health plan decision-making creates a fundamentally different set of market access challenges. The US healthcare system is often characterized by its relatively minimal market access restrictions compared with other developed nations, with those restrictions associated with commercial health plans policies. Recent evidence challenges the prevailing assumption that FDA approval translates relatively quickly and seamlessly into patient access and highlights not only that commercial plans are implementing significant coverage restrictions that can impact patient access, but also that the criteria used to establish those restrictions are not well understood.

A comprehensive study by Rucker *et al.* examined coverage policies for 389 drug-indication pairs and how it aligns with clinical guidelines across 18 of the largest US commercial health plans, representing approximately 70% of the commercially insured market [2]. Utilization management (UM) criteria serve as gatekeeping mechanisms that influence patients' access to specialty pharmaceuticals through prerequisites such as step therapy requirements and patient subgroup restrictions. When implemented appropriately, these tools can enhance patient outcomes, minimize prescribing errors, curtail unnecessary drug utilization and reduce healthcare expenditures. Clinical practice guidelines issued by national professional organizations provide evidence-based recommendations for managing specific conditions and theoretically should inform health plans' coverage policies to ensure safe, effective and efficient care aligned with current medical evidence and expert clinical consensus. The analysis of 5699 coverage policies revealed that most plans (61%) imposed UM criteria. When clinical guidelines recommended UM, 67% of plans' coverage decisions aligned with the recommendation; when guidelines did not recommend UM, only 37% of decisions were consistent. The weak alignment between guideline recommendations and actual coverage decisions suggests that clinical guidelines alone provide insufficient influence over payer decision-making processes. To further explore this, Chambers *et al.* examined coverage policies for 25 US FDA-approved cell and gene therapies in the same US commercial health plans [3]. The analysis of 532 coverage policies revealed that 276 policies, or 51.9%, imposed additional coverage restrictions beyond FDA-approved product labeling. These restrictions manifested primarily as patient subgroup requirements, such as specific clinical or genetic eligibility criteria, or as step therapy protocols requiring prior treatment failure. Notably, among the 251 patient subgroup restrictions identified, only 59% aligned consistently with pivotal trial eligibility criteria, while 12.4% were entirely inconsistent with trial criteria. The consistency of step therapy requirements with pivotal trial evidence proved even more problematic, with 55.4% of such requirements deemed inconsistent with pivotal trial criteria. The study further documented substantial variation in restrictiveness across therapeutic areas, with coverage restrictions proving significantly more common for noncancer indications than cancer indications, at 62.7 versus 44.0%, respectively. Perhaps most concerning, nine coverage policies denied coverage entirely for certain therapies, and no coverage policies could be identified for two approved therapies. Complementary findings also come from a study by Liu *et al.* examining Medicare Part D coverage for three well known GLP-1-based therapies – injectable semaglutide, oral semaglutide and injectable tirzepatide – from 2020 through 2024 [4]. While the study documented substantial expansion in formulary coverage, with coverage rates exceeding 90% for all three therapies by the third quarter of 2024, it simultaneously revealed a dramatic surge in prior authorization requirements. Prior authorization requirements remained below 25% until the third quarter of 2023, then increased sharply to approximately 83% for all three therapies by the third quarter of 2024. This dramatic shift occurred despite the substantial clinical need for these therapies among Medicare beneficiaries, with approximately 19% of Medicare beneficiaries, representing around 13 million individuals, potentially benefiting from these therapies for approved indications including Type 2 diabetes and cardiovascular disease prevention.

These studies fundamentally challenge the conventional characterization of the US market as offering relatively unrestricted access following regulatory approval. Commercial health plans are actively managing specialty drug

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access through coverage restrictions and UM approaches such as prior authorization requirements, creating an access environment more complex and restrictive, and less evidence-based than traditionally understood. For pharmaceutical manufacturers, success in this evolving landscape requires not merely regulatory approval but rather strategic evidence generation aligned with payer evidence requirements, proactive engagement with multiple stakeholders across the coverage determination process, active engagement in guideline development processes and comprehensive patient support infrastructure to navigate increasingly restrictive coverage policies. The need for thoughtful clinical trial design, with particular attention required for head-to-head comparisons where feasible, validated surrogate end points and eligibility criteria that reflect real-world patient populations rather than narrow trial-specific restrictions aligns with general requirements of European HTA agencies. The fundamental prerequisite for achieving meaningful market access across countries is the need to demonstrate genuine value supported by robust evidence [5,6].

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