

# The Paediatric Medicines Gap

Why Australia needs a stronger framework  
for medicines developed & approved for children



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### **Acknowledgement of country**

This report was written on the lands of the Darug and the Eora Nations.  
The McKell Institute acknowledges Aboriginal and Torres Strait Islander peoples as the Traditional Owners of Country throughout Australia and their continuing connection to both their land and seas.

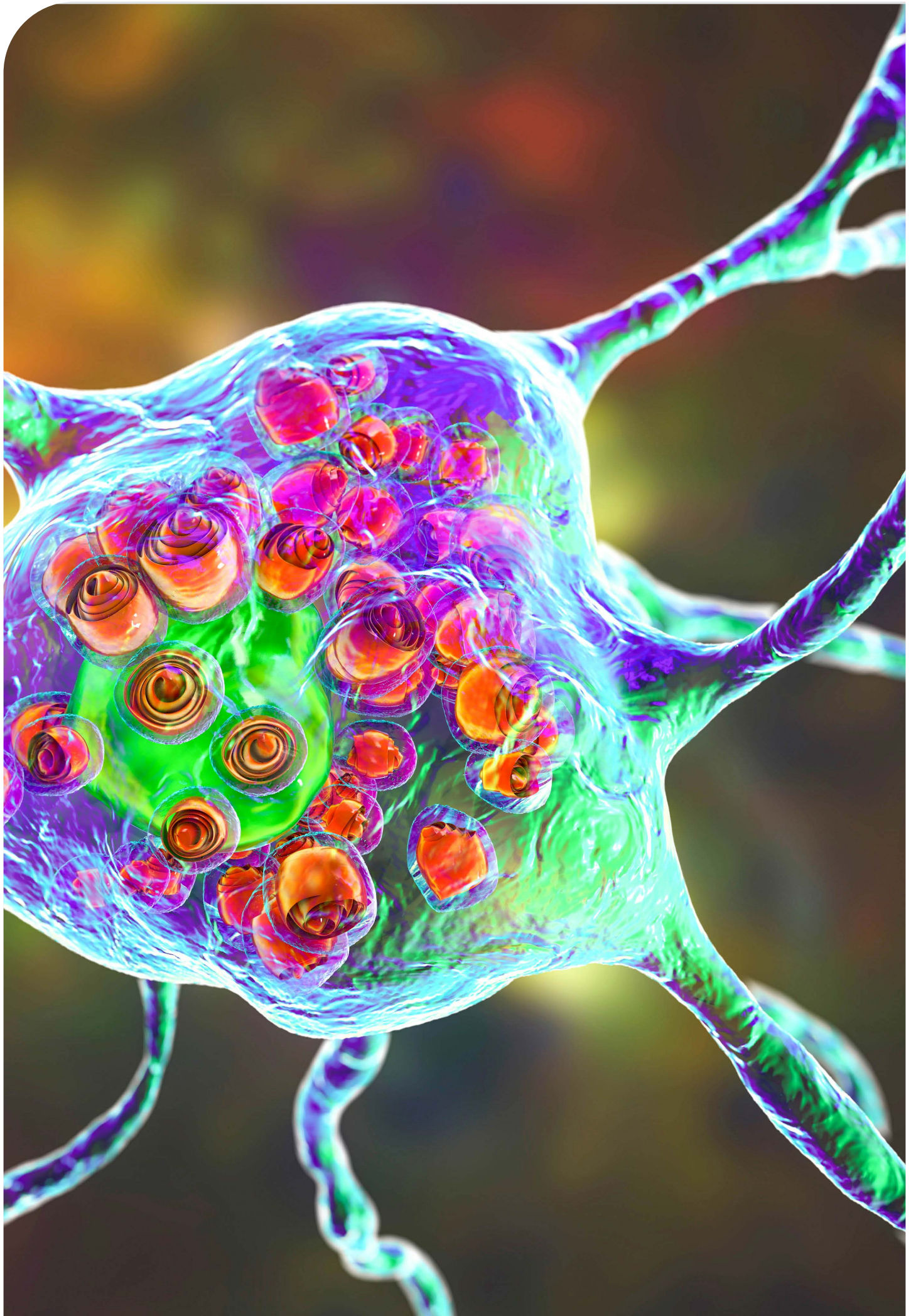
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*The opinions in this paper are those of the authors and do not necessarily represent the views of the Expert Advisory Group that informed this research. Any remaining errors are the sole responsibility of the authors and the McKell Institute.*

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# Executive Summary

Access to safe, effective and timely medicines is a cornerstone of modern healthcare. Yet for too many Australian children, particularly those living with rare diseases and profound or severe disability, that promise isn't fully realised. This report examines why children in Australia continue to face barriers to accessing the medicines and therapies they need, assesses the human and economic costs of those barriers, and sets out a practical reform agenda to improve the health outcomes of Australian children.

Up to 600,000 Australian children are living with a rare disease or a profound or severe disability. For roughly half of these children, no approved medications or therapies exist in Australia. Even where treatments do exist, around 20 per cent of children with treatable conditions are unable to access them due to a range of equity and access issues, and the economy-wide costs of this lack of access are likely to exceed AUD\$2 billion.

The report begins by examining the gaps in Australia's paediatric-specific approach to medicines access. Unlike the United States and the European Union, Australia imposes no legislative requirement on sponsors to study new medicines in children, offers no meaningful commercial incentives — such as market exclusivity extensions or paediatric data protection — to encourage paediatric development.

Without specific mechanisms to encourage paediatric medicine development, evidence generation in children can lag behind adult programs. This may mean that some medicines reach the Australian market before paediatric indications are established, creating uncertainty for families and clinicians and reinforcing the need for pathways that support earlier paediatric research and evidence generation.

The report then turns to the inequities experienced by children with rare diseases and by vulnerable populations. Families in rural and remote areas incur diagnostic out-of-pocket costs more than three times higher than families in major cities. Aboriginal and Torres Strait Islander children and children from culturally and linguistically diverse communities face compounding geographic, financial and



cultural barriers to both treatment and clinical trial participation. These inequities carry a measurable economic burden: delayed or absent treatment increases care intensity, drives caregivers out of the workforce, and imposes productivity losses plausibly in the order of several billion dollars annually.

The report also examines the regulatory and structural constraints on Australia's clinical trial network, including uncertainty in the Clinical Trial Approval pathway, inconsistent ethics and site-governance timelines, chronic recruitment difficulties inherent to small paediatric cohorts, and a stop-start funding model that undermines research infrastructure and workforce retention. Case studies of metachromatic leukodystrophy (MLD) illustrate how these systemic failures play out for real families: transformative therapies approved overseas remain financially and practically out of reach for Australian children, often until the narrow window for disease-modifying benefit has closed.

Fortunately, while these challenges are significant, pathways to reform are already mapped. The Australian Government's Health Technology Assessment (HTA) Policy and Methods Review, completed in 2024, made two recommendations directed squarely at paediatric access — the adoption of age-agnostic PBS listings and the establishment of a joint TGA-PBAC working party on paediatric

evidentiary requirements — supported by a broader suite of reforms including a High Unmet Clinical Need pathway, a bridging funding program, full parallel TGA and PBAC processing, expanded use of real-world evidence, and national horizon scanning. The implementation of these well-informed recommendations remains critical, and is advocated for in this report.

This report is intended to make the case for that implementation and to identify the complementary reforms to development incentives, clinical trial settings and equity of access needed to ensure the HTA Review's promise is realised.

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**Australia has the regulatory maturity, clinical excellence and research infrastructure to be a world leader in paediatric medicine. The recommendations that follow chart the path from an identified problem to a solved one.**

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# Key Findings

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- 1 **There are up to 600,000 children** living with rare disease or profound or severe disability in Australia

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  - 2 **For roughly half of these children**, no approved medications or therapies exist in Australia

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  - 3 **20% of children** with treatable conditions are unable to access treatment due to a range of equity and access issues

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  - 4 **Economy wide costs** of this lack of treatment access are likely to total in excess of AUD\$2 billion

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  - 5 **Australia lags behind** comparable jurisdictions in time between medicines' regulatory approval and their listing on the Pharmaceutical Benefits Scheme (PBS).  
Australia – average 466 days, Japan – average 102 days, Germany – average 136 days, UK – average 156 days

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  - 6 **Australia doesn't provide** meaningful commercial or regulatory incentives to promote therapy development and testing for orphan drugs and paediatric specific drug formulations

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  - 7 **Structural constraints are limiting** the scale, equity and efficiency of Australia's clinical trial network
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# Recommendations

## RECOMMENDATION 1

### **Establish the joint TGA-PBAC working party on paediatric evidentiary requirements**

The joint TGA-PBAC working party recommended by the HTA Review should be established as a priority, with participation from industry, paediatric clinicians and researchers, and patient and family representatives. Its guidance on evidentiary requirements for extending TGA-registered therapies to paediatric populations should explicitly address small-cohort trial evidence, extrapolation from adult data, surrogate endpoints and real-world evidence, to ensure a seamless pathway from TGA approval through health technology assessment (HTA) to PBS reimbursement for children.

## RECOMMENDATION 2

### **Establish the High Unmet Clinical Need pathway with paediatric and rare disease populations expressly recognised**

The High Unmet Clinical Need (HUCN) pathway recommended by the Review should be established with children with rare and complex conditions expressly identified as a priority population. The pathway should include proactive identification of unmet paediatric need, invitations to sponsors to submit relevant therapies, prioritised HTA review and dedicated case management for paediatric submissions.

## RECOMMENDATION 3

### **Implement the HTA Review recommendations in full, with paediatric access reforms prioritised in the first tranche of implementation**

The Australian Government should commit to full implementation of the HTA Policy and Methods Review, with the reforms bearing most directly on children's access to medicines — age-agnostic listings, quality values framework, the joint TGA-PBAC working party, the High Unmet Clinical Need pathway and the bridging funding program — sequenced in the first tranche of implementation, accompanied by a published implementation timeline and public reporting against it.

## RECOMMENDATION 4

### **Establish a national horizon-scanning function with a dedicated paediatric lens**

The national horizon-scanning function recommended by the HTA Policy and Methods Review (Recommendation 47) should be established with an explicit mandate to identify emerging paediatric therapies and areas of unmet paediatric need early, enabling proactive HTA planning and engagement with sponsors before, rather than after, Australian children fall behind their international peers.





## RECOMMENDATION 5

### **Introduce meaningful commercial incentives tied to completed paediatric development**

Australia should introduce commercial incentives — such as market exclusivity extensions, data protection for paediatric-specific development and expedited review pathways — tied to the completion of agreed paediatric studies, recognising that fee waivers alone do not materially offset development costs that can run into the billions of dollars. Incentives should be calibrated with the joint TGA-PBAC working party and designed to encourage development that culminates in both TGA registration and PBS listing of paediatric indications.

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## RECOMMENDATION 6

### **Reform clinical trial regulatory settings and provide sustained funding for a national paediatric trials network**

The Government should act on the findings of its review of the Clinical Trial Approval (CTA) scheme by clarifying responsibility for pathway selection and TGA evaluation criteria, eliminating duplication between HREC and TGA review, and strengthening TGA-HREC collaboration. It should also improve the consistency and timeliness of National Mutual Acceptance and site-specific approvals, and replace the current stop-start funding model with sustained core investment in a coordinated national paediatric clinical trials network — embedding research capacity, protected research time and specialist trial staff within tertiary paediatric centres.

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## RECOMMENDATION 7

### **Embed equity of access for First Nations, culturally and linguistically diverse (CALD), and regional and remote families**

Equity must be designed into every element of the reform agenda. This should include culturally safe recruitment, consent and care models developed with Aboriginal and Torres Strait Islander communities; improved data collection on First Nations and CALD participation in trials and access programs; decentralised and tele-trial models that bring participation to regional and remote families; and targeted support for the travel, accommodation and out-of-pocket costs that currently see remote families paying more than three times as much as metropolitan families to obtain a diagnosis.

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## RECOMMENDATION 8

### **Establish a national coordination mechanism for paediatric medicines**

Responsibility for paediatric medicines development is currently dispersed across Commonwealth regulators, State health services and research funders, with no single mechanism to identify and prioritise unmet paediatric need. The Government should establish a national coordination mechanism — connecting the TGA, PBAC, research funders, clinical trial networks, industry and patient organisations — to align regulatory requirements, research investment, trial activity and reimbursement pathways behind a shared national paediatric medicines strategy.

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# Introduction

Access to safe, effective, and timely medicines is a cornerstone of modern healthcare. Yet for Australian children, particularly vulnerable cohorts such as those affected by rare diseases, systemic inequities persist.

These inequities not only create unacceptable disparities in health outcomes but also impose significant social and economic burdens on families, communities, and the health system as a whole.

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**Children in Australia face persistent and systemic barriers to timely, equitable access to safe and effective medicines.**

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These barriers arise from gaps in paediatric-specific drug development, unresponsive regulatory regimes, insufficient investment rationale to develop and test paediatric specific indications, market failures relating to development of therapies for rare diseases, and inequities affecting vulnerable populations.

Children have historically been described as “therapeutic orphans” due to limitations in development of clinical evidence, lack of age-appropriate formulations, and exclusion from clinical research for new medicines and therapies. This report acknowledges that generating of clinical evidence in paediatric populations is inherently complex due to ethical, methodological, recruitment, and safety considerations. However, fragmented

funding, and regulatory pathways disincentivise the development and availability of therapies for children and make it difficult to build the clinical evidence base to develop age appropriate formulations and dosing approaches.

As a result, children and their families are disproportionately required to resort to off-label prescribing and have trouble accessing innovative therapies. The costs and risks of this are real. Off-label prescribing in children can increase financial burden and cost risk, as many medicines used outside approved indications are not subsidised and may require private payment by families. It also carries clinical safety risks as paediatric dosing is often extrapolated from adult data, which can increase the likelihood of adverse drug reactions when robust paediatric efficacy and safety evidence is lacking.

Australia continues to lag behind comparable jurisdictions in embedding paediatric-specific approaches into medicines development, trials, and approvals, meaning those therapies are less likely to be developed and approved specifically with paediatric indications.

This further compounds other barriers to access viable therapies, such as geographical and socio-economic factors. These limitations disproportionately affect children with rare diseases and those from marginalised or underserved communities.



However, with modest reform, Australia is well placed to leverage its advantages to ensure equitable access to paediatric therapies and medicines.

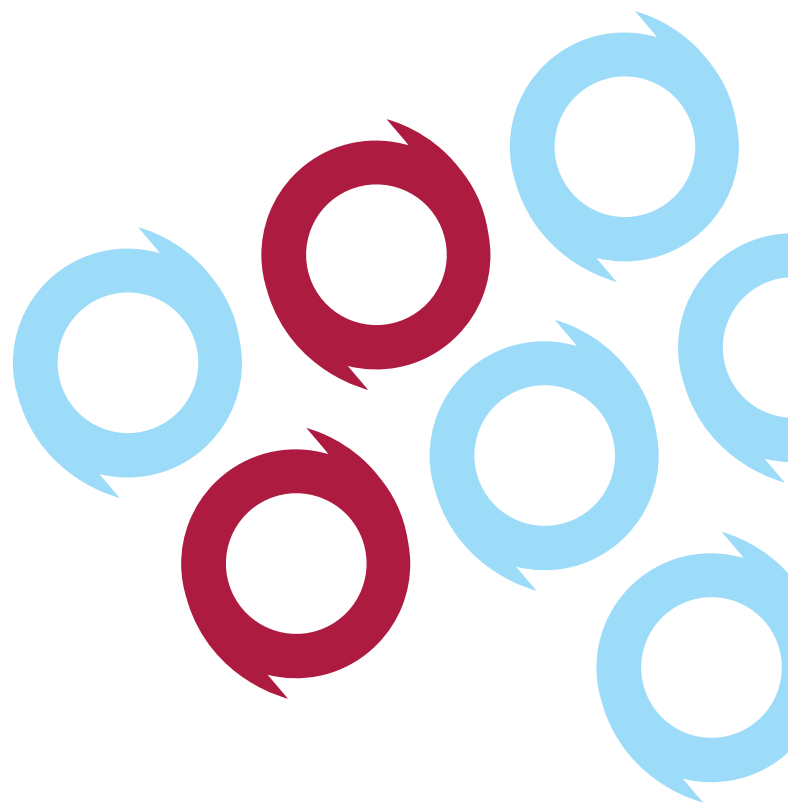
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Australia's high-quality hospital system, skilled paediatric workforce, and strong ethics and governance processes, mean it is well placed to become a leader in paediatric medicine development.

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This should be complemented by an appropriately calibrated regulatory framework that facilitates the development and listing of paediatric medicines and therapies.

This report provides an in-depth assessment of inequities in access to medicines for children in Australia. It highlights regulatory and development gaps, insufficient support for the development and evaluation of paediatric medicines, inequities affecting children with rare diseases, social and demographic disparities affecting access (including First Nations and culturally and linguistically diverse populations), and systemic and economic constraints.



# Gaps in Paediatric-Specific Approaches to Medicines Development

## REGULATORY FRAMEWORKS GOVERNING THERAPY DEVELOPMENT CONTAINS GAPS

Australia's regulatory framework for medicines development, established under the *Therapeutic Goods Act 1989*, does not incorporate paediatric-specific medicine development as a fundamental requirement within medicine approvals pathways. This means that, unlike the United States and the European Union (EU), Australia has no legislative obligations requiring pharmaceutical companies seeking Therapeutic Goods Administration (TGA) approval to study medicines in children, even if paediatric use is anticipated. The TGA provides guidance encouraging consideration of paediatric data, but this guidance is non-binding and largely reliant on sponsor discretion.<sup>2</sup>

In contrast, both the US and the EU have mandatory paediatric development legislation. In the US, the Paediatric Research Equity Act (PREA) requires that sponsors submit and fulfil a Paediatric Study Plan (PSP) for new drugs or biologics that would be used in adults, unless a waiver or deferral is granted. This obligation is statutory and linked to marketing application acceptance.<sup>3</sup> In the EU, the Paediatric Regulation (EC No 1901/2006) mandates an agreed Paediatric Investigation Plan (PIP) for all new active substances and

for new indications, formulations or routes of administration, unless waivers apply.<sup>4</sup>

In addition, a significant regulatory difference in the European Union lies in how broadly the EU legislative framework interprets paediatric needs as it relates to their mandated requirement for paediatric studies. The EU legislation uses the term "condition" broadly, such that an EU mandated Paediatric Investigation Plan for a new medicine or therapy may require studies in paediatric subpopulations even outside the specific adult indication, if a paediatric medical need exists.<sup>5</sup>

Access to medicines in Australia is via a two-tiered regulatory and approvals system. Licensing of medicines by the TGA is usually followed by a request for medicines to be listed through the Pharmaceutical Benefits Scheme (PBS), which means that the Commonwealth Government subsidises most of the cost of the medicine for Medicare card holders.

The Pharmaceutical Benefits Advisory Committee (PBAC), the Committee responsible for recommending which medicines the government should subsidise through the PBS, relies on a systematic process called the Health Technology Assessment (HTA) that evaluates safety, clinical effectiveness and cost-effectiveness of medical interventions to inform its decision making.



However, because of the lack of regulatory requirements, the HTA framework does not have a dedicated evaluation pathway for paediatric medicines. While the HTA framework applied to paediatric medicines evaluation is the same as all medicines, paediatric-specific therapies are not specifically assessed, limiting the confidence and ability for the PBAC to recommend paediatric specific medicines and therapies for listing on the PBS. Many medicines are subsequently approved only for adult populations in accordance with TGA-approved prescribing information<sup>6</sup> which focuses on adult populations. This results in medicines being registered by the TGA but remaining financially inaccessible to many families due to the lack of government subsidy through the PBS. It should be noted, however, that the HTA Review acknowledged that access to PBS-listed medicines for children and young people could be increased, including by PBAC adopting an approach of being age-agnostic with its restriction unless there are specific reasons to limit.<sup>7</sup>

This absence becomes especially visible when Australia's methods are benchmarked against comparable systems globally. A recent cross jurisdictional comparison of health technology assessment approaches in Australia, Canada, and the UK found that despite approaches being highly aligned on core technical matters, Australia diverges significantly in providing dedicated orphan drug\* pathways and related uncertainty handling mechanisms.<sup>8</sup>

International evidence suggests Australia has a longer average time from regulatory approval to reimbursement for medicines (approximately 18–24 months)<sup>9</sup> compared with the United States (approximately 6–10 months to payer coverage decisions) and European markets (approximately 7–12 months following EMA approval, varying by country).<sup>10</sup> Available evidence indicates that, even if paediatric and rare disease medicines are submitted for assessment for listing on

the PBS, they are particularly affected by reimbursement delays in Australia due to higher evidentiary uncertainty and cost-effectiveness constraints. The results of this lack of regulatory certainty is that, even in the rare instance a medicine is submitted for PBAC assessment, approval is not likely to be achieved until 2 years after TGA approval.

This gap has not gone unrecognised. The Australian Government undertook a review of the HTA, including its policy and methods, between 2022 and 2024. The Final Report contains several recommendations designed to address access equity issues for vulnerable cohorts and paediatric patients.

A central recommendation is the introduction of more flexible and proportionate assessment pathways for high-need and under-served populations, including children with rare and complex conditions. This includes streamlined and earlier consideration of therapies where evidence is necessarily limited (e.g. small paediatric trials), and reforms to better incorporate uncertainty management, real-world evidence, and broader value elements such as severity of disease and impact on carers into reimbursement decisions.

The Review also recommends strengthening equity considerations within HTA decision-making, explicitly recognising populations such as paediatric patients, First Nations peoples and those with rare diseases as priority groups where standard cost-effectiveness frameworks may not fully capture social value. Key reforms include improved patient and consumer involvement, more transparent decision pathways, and enhanced flexibility in listing medicines for ultra-rare and paediatric conditions through tailored HTA and funding pathways (including accelerated and conditional reimbursement models). Collectively, these recommendations are intended to reduce delays in access to innovative medicines for children and address structural inequities arising from reliance on traditional large-trial evidence thresholds.

In response to the Report, the PBAC has supported recommendations designed to address these access equity issues.<sup>11</sup> These recommendations should be implemented in full in order to address key access and equity barriers.

## **COMMERCIAL INCENTIVES FOR PAEDIATRIC THERAPY DEVELOPMENT ARE INSUFFICIENT**

Australia's regulatory and commercial frameworks governing therapy development also do not offer meaningful inducements such as market exclusivity extensions or expedited review pathways specifically tied to paediatric studies. In contrast, jurisdictions such as the US and the EU<sup>12</sup> link paediatric study completion to commercial benefits, such as market exclusivity extensions, in some cases up to 10 years<sup>13</sup> creating a stronger business case for investment.

A key structural difference between the US and EU systems is how incentives and regulatory requirements are combined. In the US, the incentive mechanisms are established through the Best Pharmaceuticals for Children Act (BPCA), which is distinct from PREA. Fulfilling PREA requirements doesn't automatically yield exclusivity. Instead, the BPCA requires that sponsors seeking to register new medicines or therapies must complete studies of those medicines in paediatric populations as requested by the FDA to earn a six-month patent extension. In the EU, the obligations and incentive rewards are unified under the Paediatric Regulation. Compliance with an agreed PIP can lead to additional exclusivity benefits and no separate legislative mechanism is needed.

In contrast, Australia's orphan drug and paediatric frameworks rely largely on fee waivers. Combined fees for both TGA assessment and registration and PBS application and listing could rise above \$1 million depending on the nature of

the medicine and the complexity of the application and assessment processes required, based on publicly available fee schedules published by TGA and PBS.<sup>14,15</sup> However, recent systematic literature reviews of the R&D cost required to develop a new drug demonstrates that total average pre-launch R&D costs could range from \$161 million to \$4.54 billion (2019 USD).<sup>16</sup> The disparity between the modest commercial inducements in Australia and the significant cost of therapy development clearly demonstrates that fee waivers, while substantial, do not materially offset the commercial risks of paediatric medicine development. Companies aren't avoiding paediatric programs there is just no additional benefit to have clinical sites as part of broader development programs in Australia.

## **AUSTRALIA'S REGULATORY AND SUBSIDY PATHWAYS ARE NOT ALIGNED**

Policy fragmentation between regulatory approval and subsidy pathways compounds these challenges. Even when paediatric data exists, the alignment between TGA approval and PBS listing is often weak. Paediatric indications, formulations, or age-specific dosing are frequently excluded from PBS listings, particularly where evidence is derived from small trials or extrapolated studies.<sup>17</sup> PBAC guidance explicitly discourages reliance on weak or small studies and that larger more robust studies should be prioritised. In settings for rare paediatric medicines development, large study cohorts are difficult to identify, and it is difficult to acquire appropriate data on paediatric cohorts due to a lack of paediatric arms on trials, making PBS listing more difficult. The absence of a PBS listing for paediatric may lead to prescriptions in which medicines approved on the PBS for different indications, dosages, routes of



administration, or patient groups are used in children. This transfers financial and clinical risk to families and clinicians.<sup>18</sup>

## INEQUITIES FOR CHILDREN WITH RARE DISEASES

Children with rare diseases experience some of the most profound inequities in medicine access in Australia. Small patient populations further exacerbate commercial disincentives regarding therapy development, as discussed above. Access to medicines for children living with rare diseases in Australia is constrained by systemic limitations within the PBS framework. Health Technology Assessment (HTA) processes used by the Pharmaceutical Benefits Advisory Committee (PBAC) and the PBS are designed for conditions with large evidence bases and common indications, which disadvantages rare conditions that inherently have small patient cohorts and limited trial data. Assessment models often require repeated submissions and significant resource investment, further prolonging the wait for subsidised access to the PBS and leaving families to self-fund high-cost drugs or forgo them entirely.<sup>19</sup>

As a result, children with rare diseases frequently depend on off-label use of medicines because TGA approved treatments either have not been developed or are not subsidised. In some cases, patients are forced to travel overseas for treatments not available in Australia.<sup>20</sup>

Submissions to the Australian Parliament's Standing Committee on Health, Aged Care and Sport describe the lack of streamlined and equitable pathways for repurposing existing medicines, originally approved for common diseases, as treatments for rare conditions. This means clinicians must prescribe off-label and patients pay out-of-pocket.<sup>21</sup>

Another avenue potentially available for accessing medicines and therapies for

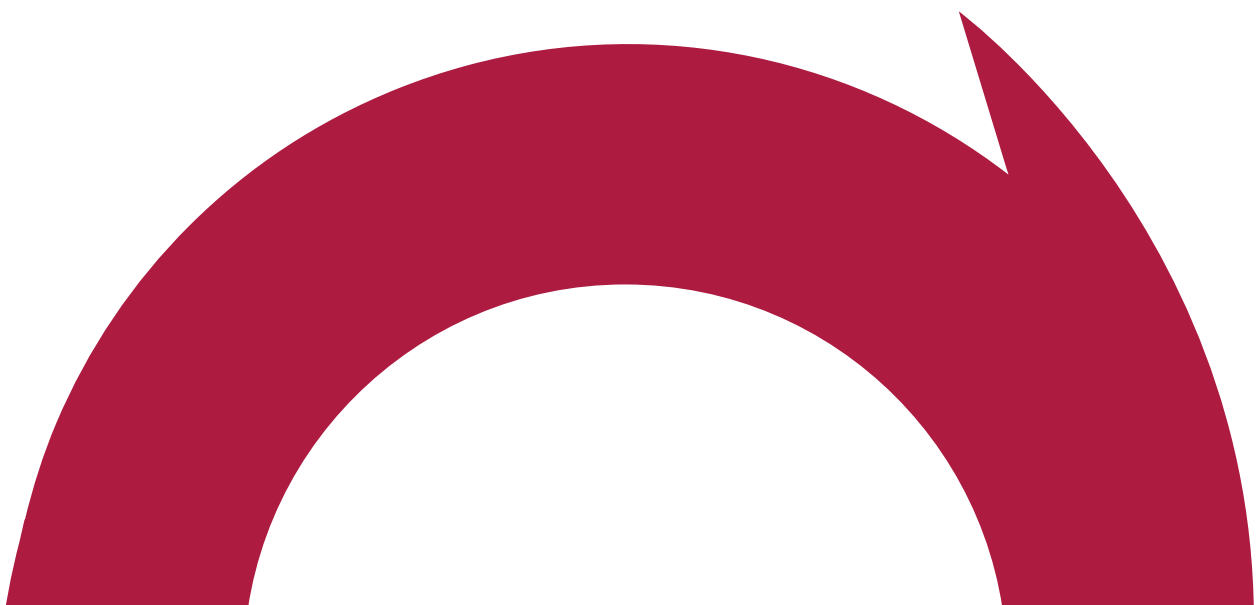
children with rare diseases is through the TGA's Special Access Scheme (SAS). This provides a pathway for paediatric clinicians to access therapies or indications that are not yet registered on the Australian Register of Therapeutic Goods (ARTG). This includes overseas medicines, advanced gene therapies, investigational products, and highly specialised rare disease treatments. This is particularly important in paediatrics because many therapies for rare childhood conditions are either still experimental or approved only in overseas jurisdictions.

However, the effectiveness of the SAS is also constrained by several significant, practical limitations. Approval through SAS does not guarantee funding, supply, or equitable access. Many experimental paediatric therapies remain extremely expensive, particularly gene and cell therapies, and hospitals or families may still need to secure separate funding arrangements even after TGA authorisation is granted. Access can depend heavily on whether a child is being treated at a major tertiary paediatric centre with specialised expertise, research infrastructure, and relationships with pharmaceutical sponsors. This creates inequities between metropolitan and regional patients and between hospitals with differing advanced therapeutics capabilities.

Clinicians and hospitals also assume significant responsibility when utilising the SAS because unapproved therapies have not undergone full TGA evaluation for safety, efficacy, or quality. The TGA guidance<sup>22</sup> emphasises that treating clinicians carry responsibility for clinical justification, informed consent, and monitoring of adverse outcomes. For highly experimental paediatric therapies, this creates substantial governance, ethical and legal complexity, often requiring additional oversight through hospital ethics committees, biosafety panels, and multidisciplinary review processes.

# Geographic, demographic, and **socio-economic barriers**

Geographic and socio-economic inequities further compound challenges for children in accessing paediatric medicines in Australia, especially children with rare diseases. National survey data show that parents of children with rare conditions can incur high out-of-pocket costs for diagnostics, medications and travel to specialist centres, with families in regional and remote areas facing significantly greater barriers than those in major cities<sup>23</sup>.





When seeking a diagnosis, average out-of-pocket expenses for families living in major cities were AU\$2,604 per family, but inner regional families spent AU\$5,390, and families in rural or remote regions spent AU\$9,250.<sup>24</sup> Although rare disease medications may be available, access can be limited by stock issues, financial cost, requirements for special authority prescriptions and the need to attend hospital facilities for administration, which disproportionately affects families with lower income or who lack proximity to tertiary treatment centres.<sup>25</sup>

## **ABORIGINAL AND TORRES STRAIT ISLANDER PEOPLES EXPERIENCE PERSISTENT BARRIERS TO ACCESSING PAEDIATRIC MEDICINES**

Aboriginal and Torres Strait Islander Australians experience persistent and multifaceted inequities in accessing most healthcare, including access to paediatric medicines and related services. Moreover, access to subsidised prescription medicines through the Closing the Gap PBS Co-payment Program, varies significantly by location, with research indicating that First Nations people in areas with high chronic disease burden often do not use available programs equitably, and access can depend heavily on postcode and the presence of local services.<sup>26</sup> This compounds other structural inequities for paediatric medicine subsidisation in Australia, including difficulties listing new therapies or existing drugs with new indications on the PBS. Further increasing the difficulty in accessing paediatric medicines for Aboriginal and Torres Strait Islander peoples in Australia.

Qualitative studies of cancer care treatment access in the Northern Territory highlight a range of clinical, logistical and cultural issues that require stronger commitments from government to overcome. These include transport difficulties, financial constraints, lack of culturally safe care, and poorly coordinated services. All of which contribute to reduced engagement with necessary treatment and worse outcomes

compared to non-Indigenous Australians.<sup>27</sup>

These inequities also exist for culturally and linguistically diverse (CALD) communities, again not limited to paediatric medicine access. Research has documented that CALD populations often have lower utilisation of health services, experience unmet healthcare needs, and face issues related to language proficiency, cultural understanding, and system navigation that affect access to both primary and specialist services.<sup>28</sup> A universal healthcare system, such as Australia's must commit to addressing these access issues.

Another important consideration for these vulnerable groups is the opportunity and availability of participation in clinical trials in Australia. Clinical trials provide patients with an opportunity to gain access to a medicine or therapy not listed on the PBS that could be lifesaving. It also provides the opportunity for trial sponsors to build a robust dataset that may enable both TGA registrations and PBS listings in the future. Pharmaceutical companies also have a role here to ensuring safe, informed, culturally appropriate participation in trials that helps to build robust datasets.

A recent multicentre cohort study of two clinical centres in Sydney found that of 114 active participants just one identified as Aboriginal or Torres Strait Islanders while 34 identified as CALD.<sup>29</sup> This is a pattern that mirrors broader health inequities affecting these demographic cohorts.

Barriers to access include geographical remoteness, which makes trial sites harder to reach; language and literacy difficulties in understanding trial information; and lack of culturally appropriate recruitment and consent processes that recognise distinct community perspectives and histories.<sup>30</sup> These factors are compounded by limited data collection on Indigenous participation, making it difficult to design targeted strategies and measure equity in trial access.

# Economic impacts of poor access to paediatric medicines and therapies

Many studies of the economic costs of paediatric conditions and rare diseases often focus on direct healthcare costs, such as impacts of accessing specialised care, direct non-healthcare costs, such as education costs; or indirect costs, such as loss of income or productivity.<sup>31,32</sup> These studies have also demonstrated that there are higher per-person costs for children with rare diseases than for adults with rare diseases.

There is a meaningful body of evidence showing that poor access to paediatric therapies and treatments creates substantial indirect costs for families and the wider economy. The mechanism is consistent; delayed or absent treatment increases care intensity, which results in caregivers reducing workforce participation and committing more time and labour to unpaid caregiving.

A recent review of paediatric rare diseases found repeated evidence of absenteeism, reduced work hours, changes in employment status and substantial informal caregiving burdens, with lost income identified as a major indirect cost.<sup>33</sup>

The productivity burden is measurable. Caregiver studies frequently use instruments such as the Work Productivity and Activity Impairment (WPAI) measure to quantify

productivity impacts and report substantial effects. One recent caregiver study found average caregiver presenteeism around 28% and activity impairment over 40%, with reduced working hours being common.<sup>34</sup> A recent Australian study quantifying caregiver burden, including work productivity, for caregivers of individuals with developmental and epileptic encephalopathies found that 83.9% indicated their employment has been affected as a consequence of being a carer.<sup>35</sup>

The limited recognition of caregiver burden in health technology assessment and reimbursement decision-making means that many of the most significant costs borne by families remain invisible. Introduction of a qualitative values framework would better recognise broader value of innovative medicines, particularly in paediatric and



rare diseases, where effective treatments may reduce caregiving demands, support workforce participation and alleviate substantial productivity losses experienced by caregivers.

Quantifying broader, economy wide productivity impacts becomes more challenging as there are variations in the degree of impact dependent on the condition and limited Australian data exists to support this.

**HOWEVER, WHEN CONSIDERING THAT:**

- around 4.5% of Australian children aged 0-14 (roughly 200,000) had a profound or severe disability, and<sup>36</sup>
- around 400,000 children in Australia live with a rare disease<sup>37</sup>

It is likely that hundreds of thousands of Australian children may experience some degree of therapy or medicine access challenges, subsequently impacting caregiver productivity and resulting in broad economic impact.

Indicative modelling suggests productivity losses associated with poor access to paediatric therapies may plausibly be in the order of several billion dollars annually in Australia, although substantial uncertainty remains due to limited national data on caregiver workforce impacts.

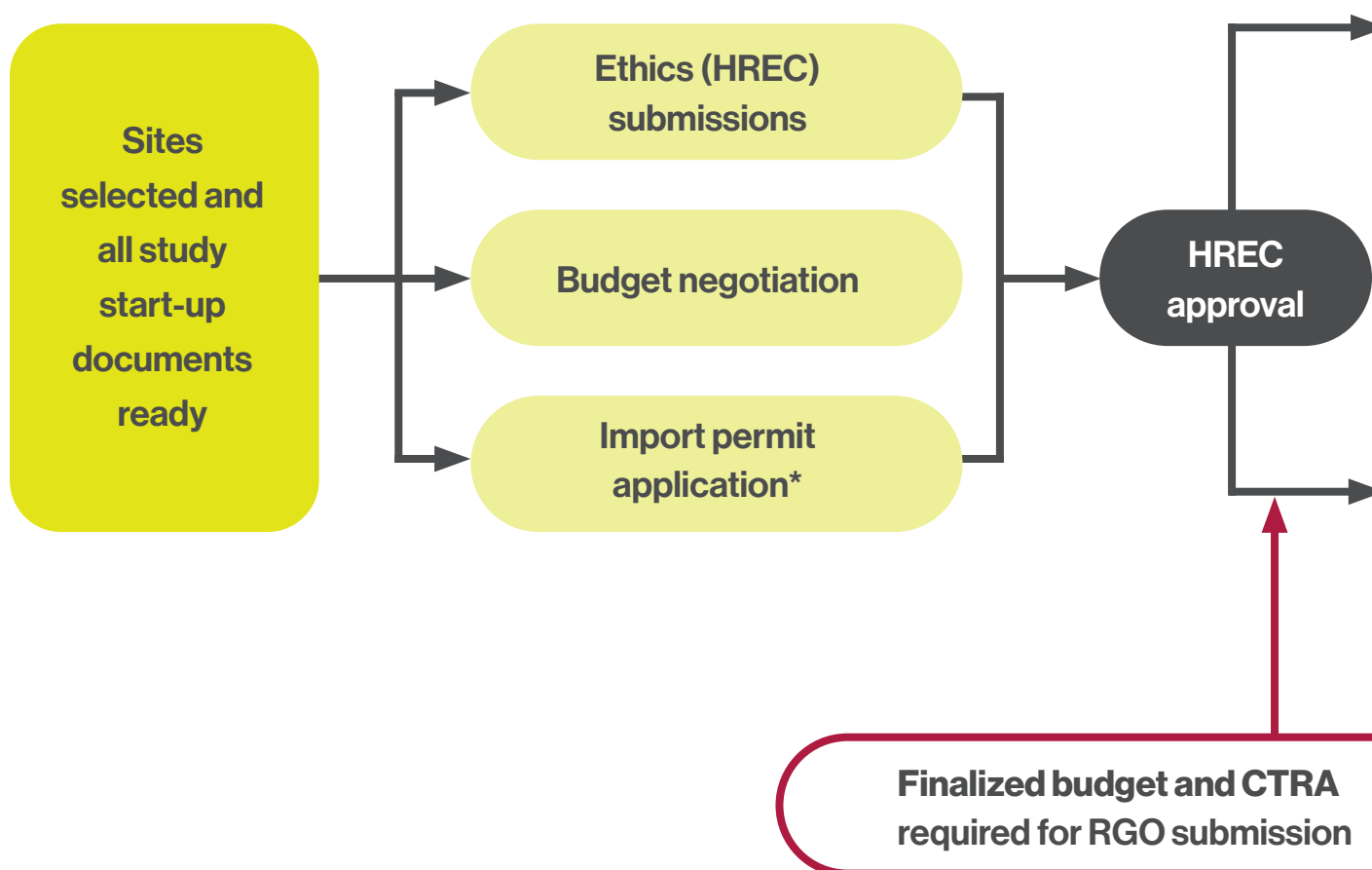
# Regulatory timelines and clinical trial barriers impacting paediatric medicines development

## AUSTRALIA'S CLINICAL TRIAL REGULATORY FRAMEWORK & TIMELINES

All clinical trials in Australia require a local sponsor, such as a research organisation that has responsibility for the initiation, management, and financing of a clinical trial and for regulatory submissions and safety reporting to the TGA.

There are two primary regulatory pathways for clinical trials—the Clinical Trial Notification (CTN) scheme and the Clinical Trial Approval (CTA) scheme.

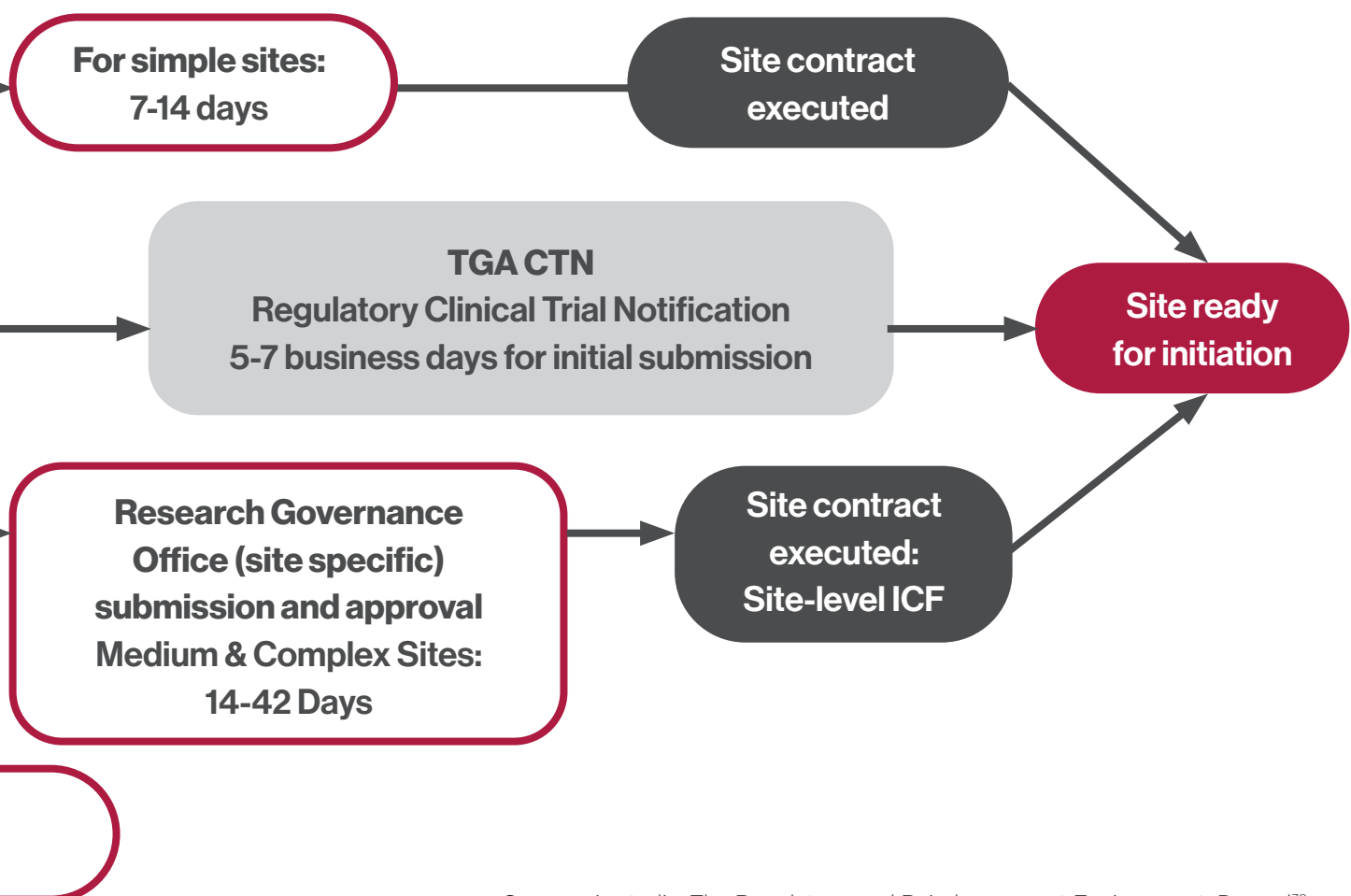
**FIGURE 1** General Timeline for CTN-based Trial Start-Up





## CTN vs CTA: Regulatory pathways and typical approval durations

CTN is a process involving notification of the TGA regarding unapproved therapeutics. The CTN channel is generally used for clinical trials of chemical drugs and biological products considered low to medium risk. The advantage of the CTN channel lies in its speed of approval. After approval by a Human Research Ethics Committee (HREC), the TGA is notified of the clinical trial at sites via the online CTN process scheme. The timeline from initial submission to TGA acknowledgement is five to ten days. The TGA is not required to perform full regulatory review of protocols, only review of a CTN form submitted by the local sponsor. No dossier, protocol or other trial-related documents are required to be submitted. However, these core documents are needed for the local sponsor to be able to complete the CTN form. Figure 1 below outlines the process for establishing a clinical trial under the CTN process.



Source: Australia: The Regulatory and Reimbursement Environment, Parexel<sup>38</sup>

The CTN pathway is the dominant route for most trials and is often completed much faster than formal regulatory review pathways. Industry sources suggest that under CTN, HREC review and subsequent TGA notification typically take between 4-8 weeks, with the TGA formally acknowledging the notification within days.

In contrast, formal CTA applications require a two-stage approval that involves a HREC approval followed by a detailed TGA review of scientific data (especially relevant for early-phase or novel therapeutics). This can extend timelines by 30–50 days or longer depending on complexity.

These approval horizons are a barrier for paediatric medicines as many novel paediatric indications arise in early-phase research and development. This creates a need for complex study designs to acquire robust data to support the CTA approvals pathway. These study designs are time and resource intensive and create further disincentives to undertake paediatric clinical trials.

The Commonwealth Government recently initiated a review of the CTA scheme and sought feedback from HRECs across Australia. The feedback report<sup>39</sup> highlights several key issues that require attention including a lack of clarity about who is responsible for recommending the CTA pathway and lack of clarity on criteria TGA uses to evaluate CTAs.

Some HRECs also note that it is likely that aspects of their review would be duplicative of the TGA evaluation, which may cause undue delays to approvals. HRECs did note that greater collaboration between the TGA and HRECs would help improve data and information sharing and joint problem solving on emerging issues and challenges.<sup>40</sup>

A further complication can present itself when seeking to complete clinical trial research. Due to the small cohort numbers for paediatric clinical research, it is common that recruitment

targets and timelines are missed at clinical trial sites. A recent review of paediatric interventional drug trials between 2015 and 2024 found that 67% of trials enrolled less than 100 participants.<sup>41</sup> This difficulty in hitting recruitment targets can cause lengthy research delays and impact on the validity of trials and trial data.<sup>42</sup>

## Ethical and governance issues create complexity for paediatric trials

A fundamental complexity in paediatric clinical research in Australia is determining who can legitimately consent to participate. Legal consent generally remains with adults until the age of 18. The concept of assent, referring to a child's agreement to participate, is recognised in national guidance, but it does not have formal legal standing under Australian law.<sup>43</sup>

Ethical guidelines such as the NHMRC National Statement on Ethical Conduct in Human Research<sup>44</sup> require researchers and HRECs to ensure that participation is in the best interests of the child and that a child's refusal to take part should be respected where they are capable of expressing it. However, determining a child's competence is complex because maturity varies by individual. Researchers must balance the need to generate paediatric evidence by ethical inclusion against the principle that research should only proceed when justified by minimal risk or direct benefit and the inability to obtain the same data from adults.

Ethical practice in paediatric trials acknowledges that consent and assent are processes rather than one-time events.<sup>45</sup> Children and their families must be provided appropriate, evolving information throughout the study so that they can understand what participation entails and feel empowered to withdraw at any point without disadvantage. Given varied comprehension levels across ages, this requires tailored communication



strategies, from simplified information sheets to ongoing dialogue between researchers and participants or their families.

Recent empirical literature highlights challenges with recording assent documentation when engaging paediatric participants, even where compliance with national ethical guidelines is high.<sup>46</sup> A document analysis of 60 paediatric clinical trial protocols approved by Human Research Ethics Committees (HRECs) from 2017 to 2023 found that while 90% of reviewed trials adhered to national ethics guidelines, only 52% actively included child assent documentation<sup>47</sup>.

Parents also expressed concerns about the complexity of consent forms, with 65% stating they needed further explanation during the consent process. Investigators cited time constraints and lack of training as barriers to improved ethical practices<sup>48</sup>.

This research demonstrates that while Australia maintains high ethical standards for paediatric research, there is a critical need for increased emphasis on child participation, clearer communication with families and ethics education for research staff.<sup>49</sup>

Complexities also arise from the necessity with small cohort groups for paediatric trials to undertake multi-centre clinical trials. Although Australia has adopted mechanisms like National Mutual Acceptance (NMA) to streamline ethical reviews for multi-centre trials, evidence suggests that site-specific and ethics approvals remain inconsistent in predictability and timelines. The National Aggregate Statistics (NAS) database for measuring clinical trials metrics in Australia shows that the aspirational target of 60 days for initiation of the regulatory process to site specific approval is met by just 46% of Australian trials.<sup>50</sup>

These complexities make it difficult for sustained research and generation of detailed

empirical paediatric data that can inform both TGA authorisation and support listings on the PBS.

## Systemic and economic constraints impact trials, therapy delivery, and research and development

Australia's paediatric clinical research environment also continues to contend with significant structural and systemic barriers that limit the pace and scope of paediatric medicine development. Unlike adult drug development, where pharmaceutical investment and trial activity are comparatively robust, paediatric studies often struggle to attract consistent funding, recruit appropriately sized cohorts, and sustain specialist infrastructure. A recent literature review of published paediatric clinical trials' participation rates found that on average roughly 10% of eligible patients according to inclusion and exclusion criteria are enrolled in clinical trials, with some trials reporting participation rates as low as 2%.<sup>51</sup> The same review also found that only half of paediatric clinical trials reached their target sample size.<sup>52</sup>

Paediatric clinical trials are inherently resource-intensive, requiring ethical safeguards, specialised consent processes, and tailored operational systems to accommodate children across a wide age range and over extended time periods to conduct studies. For example, a trial of itraconazole for the prevention of severe fungal infection in children and adults with chronic granulomatous disease, took 10 years to recruit just 39 patients.<sup>53</sup>

These challenges contribute to a stop-start model of research that relies on intermittent grants, philanthropic contributions, or project-specific state or Commonwealth funding, rather than sustained core investment. This pattern limits Australia's capacity to generate high-quality local evidence for both industry-sponsored and investigator-initiated trials.

Moreover, the limited integration of clinical research into routine paediatric care means that many tertiary paediatric centres lack dedicated staff, protected research time, and clinical trial support services, undermining the translation of basic science into paediatric therapeutic advances.<sup>54</sup> A recent study of Australia's clinical trial landscape reported that the intermittent nature of paediatric research, characterised by insecure funding models resulted in difficulties retaining clinical researchers and trial staff.<sup>55</sup> This dynamic is worsened as the workforce itself is often understaffed and fatigued, making it less likely that appropriate clinicians will prioritise work on clinical trials in addition to other tasks.

For many children with rare diseases, participating in clinical research may represent one of the only avenues to access cutting-edge treatments, yet inequities in trial availability, information, complex consent processes and medical research infrastructure and workforce gaps mean that geographically isolated and socially disadvantaged families may be effectively excluded from these opportunities. National advocacy organisations have called for policy and infrastructure reforms to improve trial access through coordinated networks and support mechanisms.<sup>56</sup>

Crucially, Australia lacks a coordinated approach to integrate regulatory requirements, research funding, clinical trials, approvals and commercial incentives. Responsibility for paediatric medicines development is dispersed across Commonwealth regulatory systems, State health services, and research funding bodies, with no single mechanism to identify and prioritise unmet paediatric needs. This policy gap, combined with weak commercial and regulatory incentives for the private sector, contributes to underinvestment in paediatric pharmacology expertise and limited clinical trial infrastructure

## **BENEFITS OF IMPROVED ACCESS TO PAEDIATRIC MEDICINES FOR CHILDREN IN AUSTRALIA**

Improved access to paediatric medicines in Australia yields multi-dimensional benefits. It has the potential to enhance children's direct health outcomes, strengthens equity, reduces long-term health system costs, lifts educational and social participation, supports family wellbeing, and promotes broader economic productivity and sustainability. The evidence underscores that prioritising accessible, affordable paediatric therapies is not only a health imperative but also a social and economic investment in Australia's future.

For example, a recent modelling study focused on reducing childhood overweight and obesity, a condition closely linked with chronic disease and higher healthcare utilisation, found that a modest 5% reduction in prevalence across today's children and adolescents could save roughly AU\$7.44 billion over their lifetimes.<sup>57</sup> These projected savings arise predominantly from lower lifetime healthcare costs and reduced premature mortality, underscoring how early, effective interventions to improve children's health translate into economic benefits decades later.

Hospital admissions among children impose high direct and indirect costs on both families and the healthcare system. A University of Sydney analysis of childhood hospitalisation found that families can incur AU\$687 per admission in productivity losses, travel and incidental expenses,<sup>58</sup> reflecting broader societal costs beyond the immediate clinical episode. By improving access to preventive and effective medicines, many of these admissions could potentially be avoided or shortened, delivering savings for families and the system.

Infectious diseases in children contribute



substantially to hospital costs: a study of paediatric hospitalisations in New South Wales reported total hospitalisation costs of around AU\$230.9 million for common infections over a cohort of 339 000 admissions,<sup>59</sup> with intensive care visits adding disproportionate costs. Enhanced access to appropriate paediatric treatments—such as evidence-based antivirals, antibiotics, or vaccines where relevant—can reduce infection severity and duration, reducing costly admissions and resource use.

One area of economic evaluation directly relevant to enhanced health access is care coordination for chronic childhood conditions. In an Australian trial, families receiving coordinated care (often involving better access to necessary treatments and follow-up) showed cost-effectiveness compared with standard care, with an incremental cost-effectiveness ratio of just AU\$548 per quality-adjusted life-year (QALY) gained. This is a benchmark widely considered good value for health systems. This suggests that enabling better continuity of care, including access to medicines, improves outcomes at relatively low additional cost across health and social sectors.<sup>60</sup>

Reducing preventable hospitalisations through better primary care and early treatment is vital for economic sustainability: in 2023–24, potentially preventable hospitalisations cost

Australia around AU\$7.7 billion in admitted patient services alone.<sup>61</sup> Many of these preventable admissions involve conditions that can be managed more effectively outside hospital with timely access to medicines, education and primary care follow-up. Improved access for children thus has clear system-level cost reduction potential.

Beyond direct treatment savings, early health improvements support long-term workforce productivity. For individuals, healthier childhoods lead to lower lifetime morbidity and higher school attendance, both of which are linked with better adult employment outcomes and reduced welfare dependency. These are outcomes that deliver broader economic returns not captured in healthcare budgets alone.







# RARE DISEASES SPOTLIGHT

**Timely access to medicines is a consistent challenge for many Australians and their families, especially those experiencing a rare disease.**

A nationally representative survey<sup>62</sup> of 462 Australian families caring for children with rare diseases covering more than 240 different conditions illustrates how access to effective medicines and therapies remains a central challenge for many families. Of survey respondents, only 45% reported that specific medications exist for their child's condition, while only 82% of those children were receiving those medicines, indicating availability gaps and some accessibility related issues. Importantly, one-quarter of all parents said that no effective medication exists for their child's condition, reflecting the reality that for many rare diseases there simply are no approved or established treatments available in Australia.

**For those families whose children do have medicines available, additional barriers compound the challenge of access.**

About 13% of parents reported specific problems obtaining medications, with the most commonly cited issues being lack of stock (41%), financial cost (21%), and requirements for special authority prescriptions (21%) to access certain drugs.<sup>63</sup>

**Some families also had to travel to hospitals to obtain specialised formulations, and a small number were relying on international sources or compassionate access programs to access ultra-rare disease medicines not routinely supplied in Australia.**

Four families in the study were even awaiting entry into clinical trials as a pathway to access novel, otherwise unaffordable therapies. These findings demonstrate that even when effective paediatric medicines exist, systemic, logistical and regulatory hurdles can significantly limit access for children with rare diseases.



## AUSTRALIANS EXPERIENCING DRAVET SYNDROME EXPERIENCED A COMPLEX PATH FOR SUPPORT

One such case where these impacts are being felt is among Australians living with Dravet syndrome, a rare, severe and life-limiting developmental and epileptic encephalopathy (DEE), with symptoms typically emerging within the first year of life. As one of the most severe forms of childhood epilepsy, it is characterised by frequent and often treatment-resistant seizures, developmental impairment, cognitive and behavioural challenges, increased risk of injury and hospitalisation, and a significantly elevated risk of premature mortality, including sudden unexpected death in epilepsy (SUDEP)<sup>64,65</sup>.

Dravet syndrome affects a very small patient population, with prevalence estimates ranging from approximately 1 in 83,000 to 1 in 15,000 people, it is estimated to affect between 335 and 1,850 Australians<sup>66</sup>.

The impact of Dravet syndrome extends well beyond seizures alone. Individuals often require lifelong multidisciplinary care involving neurologists, allied health professionals, educators and support services. Families and caregivers similarly experience significant emotional, physical, social and financial burdens associated with managing a complex and severe lifelong condition. These impacts can affect educational attainment, workforce participation, family wellbeing and overall quality of life for both people living with Dravet syndrome and those who care for them.

Providing access to medicines to treat Dravet syndrome is challenging as some of the most significant consequences of Dravet syndrome, including reduced quality of life, long-term disability, educational impacts, caregiver burden, and broader societal costs, are not always fully recognised in

the health technology process that informs reimbursement decisions in Australia.

Ensuring that decision-making processes appropriately recognise disease severity, unmet need and broader societal value is critical to supporting investment in clinical research, encouraging the development of innovative therapies, facilitating timely reimbursement decisions, and improving equitable access to treatments. This is particularly important for rare diseases and paediatric conditions, but also for other serious conditions where evidence generation is often more challenging and where delays in equitable access can significantly impact patient access to potentially life-changing treatments.

## FENFLURAMINE OFFERS A SOLUTION FOR AUSTRALIANS EXPERIENCING DRAVET SYNDROME

Fenfluramine is a prescription medicine approved by the Therapeutic Goods Administration (TGA) as add-on therapy in the treatment of seizures associated with Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) in patients 2 years of age and older<sup>67</sup>.

Australian patients and clinical investigators played an important role in the development of fenfluramine, participating in the Phase III clinical trial program that included study sites in Melbourne, Sydney and Brisbane between 2017 and 2020<sup>68</sup>. Their contribution helped generate the evidence supporting the medicine's quality, safety and effectiveness and informed regulatory decisions in major international jurisdictions.

Fenfluramine received regulatory approval from the United States Food and Drug Administration (FDA) on 25 June 2020<sup>69</sup>,



the European Commission on 18 December 2020<sup>70</sup> and the TGA on 28 November 2024<sup>71</sup>. While the TGA completed its evaluation within its standard performance timeframes (255 working days<sup>72</sup>), the medicine was not eligible for an accelerated regulatory pathway, despite prior approval by comparable overseas regulators and a substantial body of post-marketing material.

Fenfluramine received orphan drug designation in both the United States and the European Union, recognising the rarity and seriousness of Dravet syndrome and providing access to incentives designed to support the development of treatments for small patient populations. In contrast, fenfluramine was not granted orphan drug designation in Australia under the applicable criteria. Consequently, the medicine was not eligible for orphan-specific fee waivers or cost recovery exemptions, increasing the financial burden associated with seeking registration and reimbursement in Australia. For medicines treating very small patient populations, such differences in market incentives may impact commercial decision-making and the prioritisation of investment, which can create additional barriers to timely patient access.

In addition to these regulatory considerations, Dravet syndrome presents challenges for the traditional health technology assessment (HTA) process, especially with respect to capturing the full value of treatment to patients, caregivers and society. Many of the most significant impacts of the condition, including caregiver burden and broader societal costs, are not routinely reflected in the clinical and economic assessments of the HTA evaluation. For medicines developed for rare diseases or paediatric populations, uncertainty regarding how evidence of broader patient, caregiver and societal benefits will be weighted and valued in HTA decision-making can make it difficult to

demonstrate the full value of a therapy. This uncertainty may influence market prioritisation decisions, creating barriers to reimbursement and contributing to delays in patient access to innovative therapies.

While the Australian regulatory assessment was completed within standard timeframes, Australian patients received access to a TGA-registered treatment almost four years after patients in Europe and more than four years after patients in the United States. During this period, eligible patients were able to access fenfluramine through a company-managed access program and, following registration, patients could access treatment through hospital arrangements. While these mechanisms provided important interim access options, they were dependent on company support or local funding decisions and did not provide a consistent and equitable pathway for patients. Importantly, such arrangements are not a substitute for timely, equitable and sustainable access through the Pharmaceutical Benefits Scheme (PBS), which remains the primary mechanism for ensuring equitable, consistent and affordable access to medicines for Australian patients.

The significant unmet need in Dravet syndrome was further demonstrated through direct patient and caregiver advocacy. In 2023, 12,028 Australians signed a Parliamentary e-Petition EN5090<sup>73</sup> calling for improved access to fenfluramine for people living with Dravet syndrome. For a rare disease affecting a relatively small number of Australians, the scale of community support was remarkable, reflecting the far-reaching impact of the disease beyond those directly affected. The petition highlighted the significant burden experienced by patients, families and caregivers, and underscored the importance placed on timely access to innovative treatments.

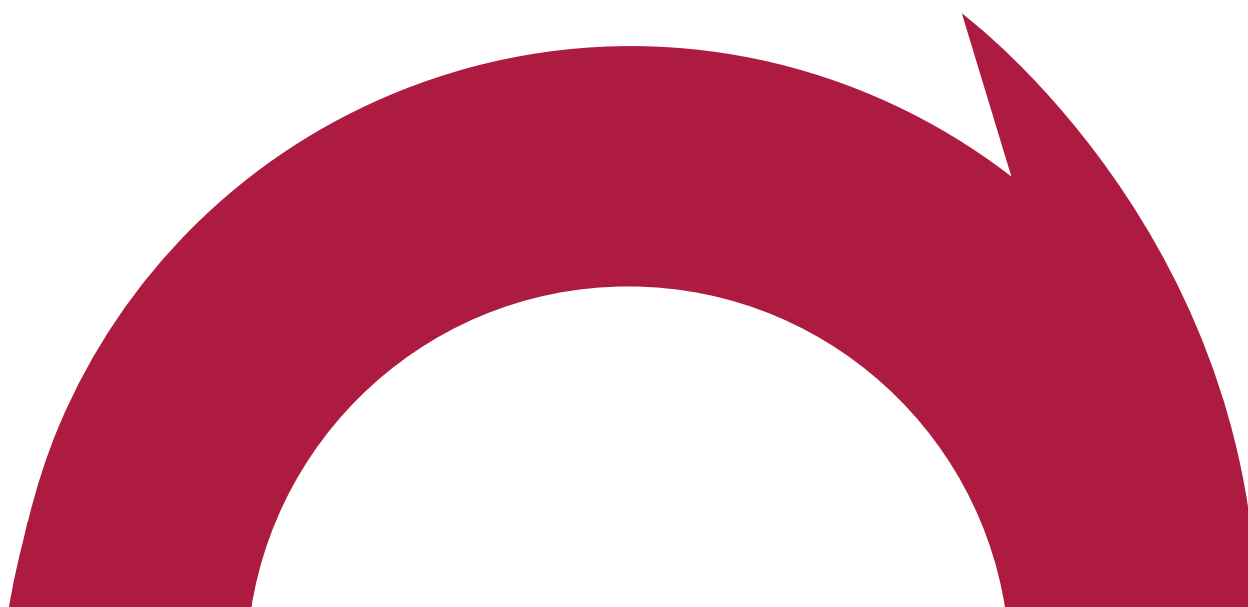
## A SIX-MONTH GAP BETWEEN PBAC SUPPORT AND PBS REIMBURSEMENT

Fenfluramine achieved PBS reimbursement on 1 May 2025<sup>74</sup>, following a positive recommendation from the Pharmaceutical Benefits Advisory Committee (PBAC) at its 6-8 November 2024 meeting. The reimbursement submission was lodged in July 2024 and progressed in parallel with the TGA evaluation to facilitate timely patient access following registration. In recommending PBS listing, the PBAC determined that fenfluramine met the criteria for Pricing Pathway A under the National Health (Pharmaceuticals and Vaccines - Cost Recovery) Regulations 2022, recognising that it addressed a high and urgent unmet clinical need and provided a substantial and clinically relevant improvement over available treatment options<sup>75</sup>.

Despite being recognised by the PBAC as addressing a high and unmet clinical need, Australian patients experienced delayed in accessing PBS-funded fenfluramine compared with patients in comparable international markets. PBS listing occurred 174 days after the PBAC's positive

recommendation in November 2024, a period that included the completion of PBS listing processes that occur between the company and the Australian Government. Overall, Australian patients waited a total of 1,595 days from European Commission approval to reimbursed access in Australia.

The fenfluramine experience highlights the challenges that can arise in translating clinical innovation into timely patient access for rare or paediatric conditions. Uncertainty regarding how the broader value of treatments for rare diseases or paediatric populations will be assessed and recognised within regulatory and HTA processes can influence global investment and market prioritisation decisions, creating barriers to reimbursement and contributing to delays in patient access. Regulatory and HTA frameworks that better recognise disease severity, unmet need, and the broader benefits of treatment to patients, caregivers and society, together with more streamlined and accelerated pathways for therapies addressing significant unmet need, could provide greater certainty for investment decisions and support more timely access to innovative therapies for Australian patients.





# Australia must build on its strengths

Despite these challenges, Australia has a significant opportunity to improve access to paediatric medicines by leveraging the strengths of its established regulatory framework, universal healthcare architecture, and internationally recognised paediatric clinical sector.

A major strength within the Australian context is the maturity and international alignment of the TGA regulatory framework. The TGA already operates sophisticated assessment and post-market surveillance systems and works closely with overseas regulators such as the European Medicines Agency and the U.S. Food and Drug Administration through international harmonisation and reliance arrangements. This creates a substantial opportunity for Australia to introduce targeted paediatric regulatory pathways.

Australia also possesses a highly sophisticated paediatric clinical and research ecosystem that provides a strong foundation for improving medicine access. Organisations such as Murdoch Children's Research Institute, Sydney Children's Hospitals Network, and Children's Hospital at Westmead have established international reputations in paediatric clinical trials, genomics, and translational medicine.

Combined with Australia's high-quality hospital system, skilled paediatric workforce, and strong ethics and governance processes, these capabilities position the country well to expand participation in multinational paediatric clinical trials and generate locally relevant evidence for regulators and reimbursement bodies.

# Conclusion

This report has shown that the paediatric medicines gap is not the product of any single failure, but of an accumulation of several deficiencies in Australia's medicines framework: a regulatory system that does not require medicines to be studied in children; commercial settings are insufficient; assessment and reimbursement processes designed for large adult populations rather than small paediatric ones; a clinical trials system constrained by fragmented approvals and insecure funding; and entrenched geographic, cultural and socio-economic inequities that fall hardest on the families least able to bear them.



The consequences are borne by hundreds of thousands of children and their families, resulting in prescribing that shifts clinical and financial risk onto households, in treatment windows that close while assessment processes run their course, and in an economic burden to the nation likely exceeding two billion dollars each year.

The case studies in this report are a reminder that behind every statistic is a child for whom the difference between timely and delayed access is not measured in days on a dashboard, but in the health outcomes of Australian children.

The encouraging truth is that Australia does not need to design the solution from scratch. The HTA Review has already done much of that work, and its paediatric access agenda — age-agnostic listings, a joint TGA–PBAC working party, a High Unmet Clinical Need pathway, bridging funding, parallel processing, real-world evidence and horizon scanning — has the support of the PBAC itself. Paired with internationally proven development requirements and incentives, a properly funded national paediatric trials network, and a deliberate commitment to equity, these reforms would transform access for Australian children within a decade.

Australia has the regulatory maturity, the clinical and research excellence, and the universal healthcare architecture to lead the world in paediatric medicine. What has been missing is not capability but priority.

The recommendations in this report offer governments a clear, practical and costed-in-ambition pathway to give Australia's sickest children what every child deserves: medicines developed for them with access to clinical trials in Australia, tested for them, and available to them when they need them.

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- <https://www.health.gov.au/sites/default/files/2025-09/hta-review-implementation-advisory-group-interim-report.pdf> \*\* An orphan drug is a pharmaceutical product developed specifically to treat a rare medical condition. Because the patient population is small, these drugs are usually not commercially viable to develop without government incentives
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